HUMAN GENE TRANSFER PROTOCOLS

Last updated: 11-21-12

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8810-001 (Closed) Gene Marking/Cancer/In Vitro/Tumor Infiltrating Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *The Treatment of Patients with Advanced Cancer Using Cyclophosphamide, Interleukin-2 and Tumor Infiltrating Lymphocytes*.

*RAC Recommends Approval: 10-3-88/NIH Approval: 3-2-89
Closed: 4-04-96

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9007-002 (Closed) Gene Therapy/Phase I/Monogenic Disease/Severe Combined Immune Deficiency due to Adenosine Deaminase Deficiency/In Vitro/Autologous Peripheral Blood Cells/CD34+ Autologous Peripheral Blood Cells/Cord Blood/Placenta Cells/Retrovirus/Adenosine Deaminase cDNA/Neomycin Phosphotransferase cDNA/Intravenous

Blaese, R. Michael; National Institutes of Health, Bethesda, Maryland; *Treatment of Severe Combined Immune Deficiency (SCID) due to Adenosine Deaminase (ADA) Deficiency with Autologous Lymphocytes Transduced with the Human ADA Gene: An Experimental Study*.

*RAC Recommends Approval: 7-31-90/NIH Approval: 9-6-90

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9007-003 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/n Vitro/Tumor Infiltrating Lymphocytes/Retrovirus/Cytokine/Tumor Necrosis Factor cDNA/Neomycin Phosphotransferase cDNA/Intravenous

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Gene Therapy of Patients with Advanced Cancer Using Tumor Infiltrating Lymphocytes Transduced with the Gene Coding for Tumor Necrosis Factor*.

*RAC Recommends Approval: 7-31-90/NIH Approval: 9-6-90
Closed: 6-26-96

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9102-004 (Closed) Gene Marking/Cancer/Acute Myelogenous Leukemia/n Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Hale, Gregory Alan; St. Jude Children's Research Hospital, Memphis, Tennessee; *Autologous Bone Marrow Transplant for Children with Acute Myelogenous Leukemia in First Complete Remission: Use of Marker Genes to Investigate the Biology of Marrow Reconstitution and the Mechanism of Relapse*.

*RAC Recommends Approval: 2-4-91/NIH Approval: 7-12-91
Closed: 1-21-93

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9105-005 (Closed) Gene Marking/Cancer/Neuroblastoma/n Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Brenner, Malcolm K.; Mirro, Joseph; Santana, Victor; and Ihle, James; St. Jude Children's Research Hospital, Memphis, Tennessee; *A Phase I/II Trial of High Dose Carboplatin and Etoposide with Autologous Marrow Support for Treatment of Stage D Neuroblastoma in First Remission: Use of Marker Genes to Investigate the Biology of Marrow Reconstitution and the Mechanism of Relapse*.

*RAC Recommends Approval: 5-31-91/NIH Approval: 7-12-91
Closed: 9-1-92

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9105-006 (Closed) Gene Marking/Cancer/Neuroblastoma/n Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Brenner, Malcolm K.; Mirro, Joseph; Santana, Victor; and Ihle, James; St. Jude Children's Research Hospital, Memphis, Tennessee; *A Phase II Trial of High-Dose Carboplatin and Etoposide with Autologous Marrow Support for Treatment of Relapse/Refractory Neuroblastoma Without Apparent Bone Marrow Involvement*.

*RAC Recommends Approval: 5-31-91/NIH Approval: 7-12-91
Closed: 4-9-93

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9105-007 (Closed) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Deisseroth, Albert B.; M.D. Anderson Cancer Research Center, Houston, Texas; *Autologous Bone Marrow Transplantation for Chronic Myelogenous Leukemia in which Retroviral Markers are Used to Discriminate between Relapse which Arises from Systemic Disease Remaining after Preparative Therapy Versus Relapse due to Residual Leukemic Cells in Autologous Marrow: A Pilot Trial*.

*RAC Recommends Approval: 5-31-91/NIH Approval: 7-12-91
Closed: 6-1-93
Closed: 4-9-93

9105-008 (Closed) Gene Marking/Acute Hepatic Failure/In Vitro/Autologous Hepatocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intrahepatic

Ledley, Fred D.; Woo, Savio; Ferry, George; and Hartwell, Whisennand; Baylor College of Medicine, Houston, Texas; *Hepatocellular Transplantation in Acute Hepatic Failure and Targeting Genetic Markers to Hepatic Cells*.

*RAC Recommends Approval: 5-30-91/NIH Approval: 7-12-91
Closed: Protocol Never Initiated

9105-009 (Closed) Gene Marking/Cancer/Melanoma/In Vitro/Tumor Infiltrating Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous

Lotze, Michael T.; University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *The Administration of Interleukin-2 and Tumor Infiltrating Lymphocytes to Patients with Melanoma*.

*RAC Recommends Approval: 5-30-91/NIH Approval: 1-17-92
Closed: 4-95

9110-010 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Renal Cell/Colon/Breast/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Tumor Necrosis Factor cDNA/Neomycin Phosphotransferase cDNA/Subcutaneous Injection

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Immunization of Cancer Patients Using Autologous Cancer Cells Modified by Insertion of the Gene for Tumor Necrosis Factor (TNF)*.

*RAC Recommends Approval: 10-7-91/NIH Approval: 10-15-91
Closed: 4-15-96

9110-011 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Renal Cell/Colon/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Immunization of Cancer Patients Using Autologous Cancer Cells Modified by Insertion of the Gene for Interleukin-2 (IL-2)*.

*RAC Recommends Approval: 10-7-91/NIH Approval: 10-15-91
Closed: 4-15-96

9110-012 (Closed) Gene Therapy/Phase I/Monogenic Disease/Familial Hypercholesterolemia/In Vitro/Low Density Lipoprotein Receptor cDNA/Intrahepatic/Portal Vein Catheter

Wilson, James M.; University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *Ex Vivo Gene Therapy of Familial Hypercholesterolemia*.

*RAC Recommends Approval: 10-8-91/NIH Approval: 11-14-91
Closed: 3-11-94

9202-013 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Adenocarcinoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DC-Chol/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral/Direct Injection/Catheter Delivery to Pulmonary Nodules

Nabel, Gary J.; University of Michigan, Ann Arbor, Michigan; *Immunotherapy of Malignancy by In Vivo Gene Transfer into Tumors*.

*RAC Recommends Approval: 2-10-92/NIH Approval: 4-17-92
Closed: 11-19-92 (Replaced by Protocol #9306-045)

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9202-014 (Closed) Gene Marking/Cancer/Acute Myelogenous Leukemia/Acute Lymphocytic Leukemia/In Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Cornetta, Kenneth; Indiana University, Indianapolis, Indiana; *Retroviral-Mediated Gene Transfer of Bone Marrow Cells during Autologous Bone Marrow Transplantation for Acute Leukemia*.

*RAC Recommends Approval: 2-11-92/NIH Approval: 4-17-92
Closed 5-1-95

9202-015 (Closed) Gene Marking/Cancer/Melanoma/Renal Cell/In Vitro/CD4+ Autologous Peripheral Blood Lymphocytes/CD8+ Autologous Peripheral Blood Lymphocytes/CD4+ Autologous Tumor Infiltrating Lymphocytes/CD8+ Autologous Tumor Infiltrating Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous

Economou, James S. and Belldegrun, Arie; University of California at Los Angeles, Los Angeles, California; *The Treatment of Patients with Metastatic Melanoma and Renal Cell Cancer Using In Vitro Expanded and Genetically-Engineered (Neomycin Phosphotransferase) Bulk, CD8 (+) and/or CD4(+) Tumor Infiltrating Lymphocytes and Bulk, CD8(+) and/or CD4(+) Peripheral Blood Leukocytes in Combination with Recombinant Interleukin-2 Alone, or with Recombinant Interleukin-2 and Recombinant Alpha Interferon*.

*RAC Recommends Approval: 2-11-92/NIH Approval: 4-17-92
Closed: 6-94

9202-016 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intraperitoneal Administration

Freeman, Scott M.; Tulane University Medical Center, New Orleans, Louisiana; *Gene Transfer for the Treatment of Cancer*.

*RAC Recommends Approval: 2-10-92/NIH Approval: 2-5-93

9202-017 (Open) Gene Therapy/Infectious Disease/Human Immunodeficiency Virus/In Vitro/CD8+ Allogeneic Cytotoxic T Lymphocytes/CD8+ Syngeneic Cytotoxic T Lymphocytes/Retrovirus/Hygromycin Phosphotransferase/Herpes Simplex Virus Thymidine Kinase cDNA/Intravenous

Greenberg, Philip D. and Riddell, Stanley; Fred Hutchinson Cancer Research Center, University of Washington, Seattle; *Phase I Study to Evaluate the Safety of Cellular Adoptive Immunotherapy Using Genetically Modified CD8+ HIV-Specific T Cells in HIV Seropositive Individuals*.

*RAC Recommends Approval: 2-11-92/NIH Approval: 4-17-92

9206-018 (Closed) Gene Therapy/Phase I/Cancer/Relapsed-Refractory Neuroblastoma/Immunotherapy/In Vitro/Autologous Neuroblastoma Cells/Allogeneic Partially HLA-Matched/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection

Brenner, Malcolm K.; Furman, Wayne; Santana, Victor; Bowman, Laura; and Meyer, William; St. Jude Children's Research Hospital, Memphis, Tennessee; *Phase I Study of Cytokine-Gene Modified Autologous Neuroblastoma Cells for Treatment of Relapsed/Refractory Neuroblastoma*.

*RAC Recommends Approval: 6-1-92/NIH Approval: 8-14-92
Closed to accrual: 1-29-02

9206-019 (Closed) Gene Therapy/Phase I/Cancer/Brain/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Stereotactic Injection

Oldfield, Edward; National Institutes of Health, Bethesda, Maryland; *Gene Therapy for the Treatment of Brain Tumors Using Intra-Tumoral Transduction with the Thymidine Kinase Gene and Intravenous Ganciclovir*. Sponsor: Genetic Therapy, Inc./Novartis

*RAC Recommends Approval: 6-1-92/NIH Approval: 8-14-92
Closed: 12-94

9206-020 (Closed) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/Autologous Bone Marrow Cells/Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Deisseroth, Albert B.; M.D. Anderson Cancer Center, Houston, Texas; *Use of Two Retroviral Markers to Test Relative Contribution of Marrow and Peripheral Blood Autologous Cells to Recovery After Preparative Therapy*.

*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92
Closed: 2-13-96

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9206-021 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Partially HLA-Matched/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection

Gansbacher, Bernd; Houghton, Alan; and Livingston, Philip; Memorial Sloan Kettering Cancer Center, New York, New York; *Immunization with HLA-A2 matched Allogeneic Melanoma Cells that Secrete Interleukin-2 in Patients with Metastatic Melanoma*.

*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92
Closed: 10-19-94

9206-022 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vitro/Allogeneic Partially HLA-Matched/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection

Gansbacher, Bernd; Motzer, Robert; Houghton, Alan; and Bander, Neil; Memorial Sloan Kettering Cancer Center, New York, New York; *Immunization with Interleukin-2 Secreting Allogeneic HLA-A2 Matched Renal Cell Carcinoma Cells in Patients with Advanced Renal Cell Carcinoma*.

*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92

9206-023 (Closed) Gene Marking/Cancer/Multiple Myeloma/In Vitro/CD34+ Autologous Peripheral Blood Cells/Intravenous/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Dunbar, Cynthia; National Institutes of Health, Bethesda, Maryland; *Retroviral-Mediated Gene Transfer of Bone Marrow and Peripheral Blood Stem Cells During Autologous Bone Marrow Transplantation for Multiple Myeloma*.

*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92
Closed to accrual: December 1996

9206-024 (Closed) Gene Marking/Cancer/Breast/In Vitro/CD34+ Autologous Peripheral Blood Cells/Intravenous/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Dunbar, Cynthia; National Institutes of Health, Bethesda, Maryland; *Retroviral-Mediated Gene Transfer of Bone Marrow and Peripheral Blood Stem Cells During Autologous Bone Marrow Transplantation for Metastatic Breast Cancer*.

*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92
Closed to accrual: October 1996

9206-025 (Closed) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/Intravenous/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Dunbar, Cynthia; National Institutes of Health, Bethesda, Maryland; *Retroviral-Mediated Gene Transfer of Bone Marrow and Peripheral Blood Stem Cells During Autologous Bone Marrow Transplantation for Chronic Myelogenous Leukemia*.

*RAC Recommends Approval: 6-2-92/NIH Approval: 8-14-92

9209-026 (Closed) Gene Marking/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Syngeneic Peripheral Blood Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous

Tavel, Jorge; National Institutes of Health, Bethesda, Maryland; *A Study of the Safety and Survival of the Adoptive Transfer of Genetically Marked Syngeneic Lymphocytes in HIV Infected Identical Twins*.

*RAC Recommends Approval: 9-14-92/NIH Approval: 9-3-93
Closed to new enrollment. Individuals will be followed, long-term, in a new protocol (that does not involve administration of recombinant DNA): 1-17-02

9209-027 (Closed) Gene Marking/Cancer/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Schuening, Friedrich G.; Miller, A. Dusty; and Kiem, Hans-Peter; Fred Hutchinson Cancer Research Center, University of Washington, Seattle, Washington; *Study on Contribution of Genetically Marked Peripheral Blood Repopulating Cells to Hematopoietic Reconstitution after Transplantation*.

*RAC Recommends Approval: 9-14-92/NIH Approval: 2-5-93
Closed: 4-29-97

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9209-028 (Closed) Gene Marking/Cancer/Lymphoid Malignancies/In Vitro/G-CSF Mobilized Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Schuening, Friedrich G.; Fred Hutchinson Cancer Research Center, University of Washington, Seattle, Washington; *Evaluation of the Use of Recombinant Human G-CSF Stimulated Peripheral Blood Progenitor Cell Supplementation in Autologous Bone Marrow Transplantation in Patients with Lymphoid Malignancies*.

*RAC Recommends Approval: 9-14-92/NIH Approval: 2-5-93
Closed: 2-25-94 (Merged with protocol # 9209-027)

9209-029 (Closed) Gene Marking/Cancer/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Schuening, Friedrich G.; Fred Hutchinson Cancer Research Center, University of Washington, Seattle, Washington; *A Trial of G-CSF Stimulated Peripheral Blood Stem Cells for Engraftment in Identical Twins*.

*RAC Recommends Approval: 9-14-92/NIH Approval: 2-5-93
Closed: Protocol Never Initiated

9209-030 (Open) Gene Marking/Cancer/Chronic Lymphocytic Leukemia/Follicular Non-Hodgkin's Lymphoma/In Vitro/Autologous Bone Marrow Cells/Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Deisseroth, Albert B.; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Use of Retroviral Markers to Identify Efficacy of Purging and Origin of Relapse Following Autologous Bone Marrow and Peripheral Blood Cell Transplantation in Indolent B Cell Neoplasms (Follicular Non-Hodgkin's Lymphoma or Chronic Lymphocytic Leukemia) Patients*.

*RAC Recommends Approval: 9-14-92/NIH Approval: 12-2-93

9403-031 (Open) Gene Therapy/Phase I/Cancer/Non-small Cell Lung Cancer/Antisense/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Retrovirus/p53 cDNA/kras Antisense/Intratumoral/Bronchoscope

Roth, Jack A.; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; and Garver, Robert I., Jr.; University of Alabama at Birmingham, Birmingham, AL; *Clinical Protocol for Modification of Oncogene and Tumor Suppressor Gene Expression in Non-Small Cell Lung Cancer (NSCLC)*.

*RAC Recommends Approval: 3-4-94/NIH Approval: 1-4-95

9209-032 (Closed) Gene Marking/Cancer/Neuroblastoma/In Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Brenner, Malcolm K.; St. Jude Children's Research Hospital, Memphis, Tennessee; *A Phase II Trial of the Baxter Neuroblastoma Bone Marrow Purging System Using Gene Marking to Assess Efficacy*.

*RAC Recommends Approval: 9-15-92/NIH Approval: 2-5-93

9209-033 (Open) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/In Combination with Untransduced Autologous Tumor Cells/Retrovirus/Cytokine/Interleukin-4 cDNA/Subcutaneous Injection

Lotze, Michael T. and Rubin, Joshua T.; University of Pittsburgh, Pittsburgh, Pennsylvania; *Gene Therapy of Cancer: A Pilot Study of IL-4 Gene Modified Antitumor Vaccines*.

*RAC Recommends Approval: 9-15-92/NIH Approval: 2-5-93

9212-034 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)

Crystal, Ronald G.; Rockefeller University Hospital, New York, New York; *A Phase I Study, in Cystic Fibrosis Patients, of the Safety, Toxicity, and Biological Efficacy of a Single Administration of a Replication Deficient, Recombinant Adenovirus Carrying the cDNA of the Normal Human Cystic Fibrosis Transmembrane Conductance Regulator Gene in the Lung*.

*RAC Recommends Approval: 12-3-92/NIH Approval: 4-16-93
Protocol closed, IND inactive: 5-30-00

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9212-035 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adenovirus/Serotype 5/E2a Temperature Sensitive Mutant/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)

Wilson, James M., University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; Simon, Richard H., University of Michigan Medical Center, Ann Arbor, Michigan; McCoy, Karen, Cystic Fibrosis Center at Ohio State University; *Gene Therapy of Cystic Fibrosis Lung Diseases Using E1 Deleted Adenoviruses: A Phase I Trial*.

*RAC Recommends Approval: 12-3-92/NIH Approval: 8-26-93
No additional enrollment planned: 8-05-99

9212-036 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Adenovirus/Serotype 2/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal

Welsh, Michael J.; Howard Hughes Medical Institute, Iowa City, Iowa; and Smith, Alan E.; Genzyme Corporation, Framingham, Massachusetts; *Cystic Fibrosis Gene Therapy Using an Adenovirus Vector: In Vivo Safety and Efficacy in Nasal Epithelium.* Sponsor: Genzyme Corporation

*RAC Recommends Approval: 12-4-92/NIH Approval: 4-16-93
Protocol ended in November 1993

9303-037 (Closed) Gene Therapy/Phase I/Cancer/Glioblastoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection

Van Gilder, John C.; University of Iowa, Iowa City, Iowa; Berger, Mitchell; University of California, San Francisco, California; Prados, Michael; University of Washington, Seattle, Washington; Warnick, Ronald; University of Cincinnati Medical Center, Cincinnati, Ohio; Schold, Clifford; University of Texas Southwestern Medical Center, Dallas, Texas; Fetell, Michael; Columbia Presbyterian Medical Center, New York, New York; Schramm, Johannes; Neurochirurgische Universitatsklinik, Bonn, Germany; Westphal, Manfred; University Clinic Eppendorf, Hamburg, Germany; Tonn, Jorg-Christian; University Kliniken, Wurzburg, Germany; Moumdjian, Robert; Notre-Dame Hospital, Montreal, Quebec, Canada; Shaffrey, Mark; University of Virginia, Charlottesville, Virginia; Asher, Anthony; Charlotte Neurological Associates and Presbyterian Hospital, Charlotte, North Carolina; Epstein, Mel; Brown University, Providence, Rhode Island; Schmitz-Schackert, Gabriele Anna Maria; University Klinikum Karl-Gustav-Carus, Dresden, Germany; Mendez, Ivar; Victoria General Hospital, Nova Scotia, Canada; Bernstein, Mark; The Toronto Hospital, Toronto, Ontario, Canada; *Gene Therapy for the Treatment of Recurrent Glioblastoma Multiforme with In Vivo Tumor Transduction with the Herpes Simplex Thymidine Kinase Gene/Ganciclovir System*. Sponsor: Genetic Therapy, Inc./Novartis

*RAC Recommends Approval: 3-1-93/NIH Approval: 4-16-93

9303-038 (Closed) Gene Marking/Cancer/Leukemia/Non-malignant Disorders/In Vitro/Epstein-Barr Virus Specific Allogeneic Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Heslop, Helen E.; Brenner, Malcolm K.; and Rooney, Cliona; St. Jude Children's Research Hospital, Memphis, Tennessee; *Administration of Neomycin Resistance Gene Marked EBV Specific Cytotoxic T Lymphocytes to Recipients of Mismatched-Related or Phenotypically Similar Unrelated Donor Marrow Grafts*.

*RAC Recommends Approval: 3-2-93/NIH Approval: 4-16-93
Gene Marking portion discontinued: February 1996

9303-039 (Closed) Gene Marking/Cancer/Acute Myelogenous Leukemia/In Vitro/Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Hale, Gregory; St. Jude Children's Research Hospital, Memphis, Tennessee; *Assessment of the Efficacy of Purging by Using Gene-Marked Autologous Marrow Transplantation for Children with Acute Myelogenous Leukemia in First Complete Remission*.

*RAC Recommends Approval: 3-2-93/NIH Approval: 4-16-93

9303-040 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Subcutaneous Injection

Simons, Jonathan; Johns Hopkins Oncology Center, Baltimore, Maryland; *Phase I Study of Non-Replicating Autologous Tumor Cell Injections Using Cells Prepared With or Without Granulocyte-Macrophage Colony Stimulating Factor Gene Transduction in Patients with Metastatic Renal Cell Carcinoma*.

*RAC Recommends Approval: 3-1-93/NIH Approval: 12-2-93
Study closed, long-term follow-up continues: 7-16-01

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9303-041 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)

Wilmott, Robert W. and Whitsett, Jeffrey; Children's Hospital Medical Center, Cincinnati, Ohio; and Trapnell, Bruce; Genetic Therapy, Inc., Gaithersburg, Maryland; *A Phase I Study of Gene Therapy of Cystic Fibrosis Utilizing a Replication Deficient Recombinant Adenovirus Vector to Deliver the Human Cystic Fibrosis Transmembrane Conductance Regulator cDNA to the Airways*. Sponsor: Genetic Therapy, Inc./Novartis

*RAC Recommends Approval: 3-2-93/NIH Approval: 4-16-93
Closed: 4-28-97 (IND Withdrawn)

9303-042 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator CDNA/Intranasal

Boucher, Richard C. and Knowles, Michael R.; University of North Carolina, Chapel Hill, North Carolina; *Gene Therapy for Cystic Fibrosis Using E1 Deleted Adenovirus: A Phase I Trial in the Nasal Cavity*.

*RAC Recommends Approval: 3-2-93/NIH Approval: 10-7-93
Closed: 10-94

9306-043 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Gamma Interferon cDNA/Subcutaneous Injection

Seigler, Hilliard F.; Duke University Medical Center, Durham, North Carolina; and Merritt, James A.; Viagene, Inc., San Diego, California; *A Phase I Trial of Human Gamma Interferon-Transduced Autologous Tumor Cells in Patients With Disseminated Malignant Melanoma*.

*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93
Closed to further enrollment: September 1997

9306-044 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Chemoprotection/In Vitro/CD34+ Autologous Bone Marrow Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Bone Marrow Transplant

Deisseroth, Albert B.; Kavanagh, John; and Champlin, Richard; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Use of Safety-Modified Retroviruses to Introduce Chemotherapy Resistance Sequences into Normal Hematopoietic Cells for Chemoprotection During the Therapy of Ovarian Cancer: A Pilot Trial*.

*RAC Recommends Approval: 6-7-93/NIH Approval: 12-2-93

9306-045 (Closed) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral/Direct Injection/Catheter Delivery to Pulmonary Nodules

Nabel, Gary J.; University of Michigan Medical Center, Ann Arbor, Michigan; *Immunotherapy for Cancer by Direct Gene Transfer into Tumors*.

*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93

9306-046 (Closed) Gene Therapy/Phase I/Monogenic Disease/Gaucher Disease/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Glucocerebrosidase cDNA/Bone Marrow Transplant

Barranger, John A.; University of Pittsburgh, Pittsburgh, Pennsylvania; *Gene Therapy for Gaucher Disease: Ex Vivo Gene Transfer and Autologous Transplantation of CD34(+) Cells*.

*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93

9306-047 (Closed) Gene Therapy/Phase I/Monogenic Disease/Gaucher Disease/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Glucocerebrosidase cDNA/Bone Marrow Transplant

Karlsson, Stefan and Dunbar, Cynthia; National Institutes of Health, Bethesda, Maryland; and Kohn, Donald B.; Childrens Hospital Los Angeles, Los Angeles, California; *Retroviral Mediated Transfer of the cDNA for Human Glucocerebrosidase into Hematopoietic Stem Cells of Patients with Gaucher Disease*. Sponsor: Genetic Therapy, Inc./Novartis

*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93
Closed: 4-30-97 (IND Withdrawn)

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9306-048 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1IIIB Envelope Protein/Intramuscular Injection

Galpin, Jeffrey E.; University of Southern California; Casciato, Dennis A.; Shared Medical Research Foundation, Tarzana, California; and Merritt, James A.; Viagene, Inc., San Diego, California; *A Preliminary Study to Evaluate the Safety and Biologic Effects of Murine Retroviral Vector Encoding HIV-1 Genes [HIV-IT(V)] in Asymptomatic Subjects Infected with HIV-1*. Sponsor: Chiron Corporation

*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93
Closed: 9-8-94

9306-049 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Retrovirus/Particle Mediated Gene Transfer (Accell®)/RSV-tar/Rev M10/Intravenous

Nabel, Gary J.; University of Michigan Medical Center, Ann Arbor, Michigan; *A Molecular Genetic Intervention for AIDS - Effects of a Transdominant Negative Form of Rev*.

*RAC Recommends Approval: 6-7-93/NIH Approval: 9-3-93

IND terminated: 3-13-00

9306-050 (Open) Gene Therapy/Phase I/Cancer/Astrocytoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Ommaya Injection

Raffel, Corey; Mayo Clinic, Rochester, Minnesota; Villablanca, Judith; Childrens Hospital Los Angeles, Los Angeles, California; Packer, Roger, Childrens National Medical Center, Washington, DC; Tonn, Jorg-Christian, Neurochirurgische Klinik und Poliklinik, Universitats-Klinikin, Wurzburg, Germany; and Burdach, Stefan; University Center for Paediatrics, Heinrich-Heine Universitat, Dusseldorf, Germany; *Gene Therapy for the Treatment of Recurrent Pediatric Malignant Astrocytomas with In Vivo Tumor Transduction with the Herpes Simplex Thymidine Kinase Gene*. Sponsor: Genetic Therapy, Inc./Novartis

*RAC Recommends Approval: 6-8-93/NIH Approval: 9-3-93

9306-051 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Brain/Chemoprotection/In Vitro/CD34+ Autologous Bone Marrow Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Bone Marrow Transplant

Hesdorffer, Charles and Antman, Karen; Columbia University College of Physicians and Surgeons, New York, New York; *Human MDR Gene Transfer in Patients with Advanced Cancer*.

*RAC Recommends Approval: 6-8-93/NIH Approval: 9-3-93

9306-052 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma/Antisense/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Lipofectin (Gibco BRL)/Insulin-like Growth Factor Antisense/Subcutaneous Injection

Ilan, Joseph; Case Western Reserve University School of Medicine and University Hospitals of Cleveland, Cleveland, Ohio; *Gene Therapy for Human Brain Tumors Using Episome-Based Antisense cDNA Transcription of Insulin-Like Growth Factor I*.

*RAC Recommends Approval: 6-8-93/NIH Approval: 12-2-93

9309-053 (Open) Gene Therapy/Phase I/Cancer/Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Lipofectin (Gibco BRL)/Cytokine/Interleukin-2 cDNA/Neomycin Phosphotransferase cDNA/Subcutaneous Injection

Podack, Eckhard R.; Sridhar, Kasi; University of Miami; and Savaraj, Niramol; Miami Veterans Administration Hospital, Miami, Florida; *Phase I Study of Transfected Cancer Cells Expressing the Interleukin-2 Gene Product in Limited Stage Small Cell Lung Cancer*.

*RAC Recommends Approval: 9-9-93/NIH Approval: 12-2-93

9309-054 (Closed) Gene Therapy/Phase I/Cancer/Breast/Chemoprotection/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Intravenous

O'Shaughnessy, Joyce; Kentuckiana Medical Oncology Association, Louisville, Kentucky; *Retroviral Mediated Transfer of the Human Multi-Drug Resistance Gene (MDR-1) into Hematopoietic Stem Cells During Autologous Transplantation after Intensive Chemotherapy for Breast Cancer*.

*RAC Recommends Approval: 9-9-93/NIH Approval: 10-7-93
Study completed, follow-up continues: 5-31-00

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9309-055 (Closed) Gene Therapy/Phase I/Cancer/Brain Tumors/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection

Kun, Larry E.; Sanford, R. A.; Brenner, Malcolm K.; and Heideman, Richard L.; St. Jude Childrens Research Hospital, Memphis, Tennessee; and Oldfield, Edward H.; National Institutes of Health, Bethesda, Maryland*; Gene Therapy for Recurrent Pediatric Brain Tumors*. Sponsor: Genetic Therapy, Inc./Novartis

*RAC Recommends Approval: 9-9-93/NIH Approval: 10-7-93
Closed to accrual: July 1997

9309-056 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Interleukin-2 cDNA/Neomycin Phosphotransferase cDNA/Subcutaneous Injection

Das Gupta, Tapas K. and Cohen, Edward P.; University of Illinois at Chicago, Chicago, Illinois; *Immunization of Malignant Melanoma Patients with Interleukin 2-Secreting Melanoma Cells Expressing Defined Allogeneic Histocompatibility Antigens*.

*RAC Recommends Approval: 9-10-93/NIH Approval: 4-19-94

9309-057 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus-1/Replication Inhibition/Hairpin Ribozyme/In Vitro/CD4+ Peripheral Blood Cells/Retrovirus/Hairpin Ribozyme/Intravenous

Wong-Staal, Flossie; Poeschla, Eric; and Looney, David; University of California, San Diego, California; *A Phase I Clinical Trial to Evaluate the Safety and Effects in HIV-1 Infected Humans of Autologous Lymphocytes Transduced with a Ribozyme that Cleaves HIV-1 RNA*.

*RAC Recommends Approval: 9-10-93/NIH Approval: 10-25-94

9309-058 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/In Combination with Untransduced Autologous Tumor Cells/Retrovirus/Interleukin-2 cDNA/Subcutaneous Injection

Economou, James S. and Glaspy, John A.; University of California Medical Center, Los Angeles, California; *Genetically Engineered Autologous Tumor Vaccines Producing Interleukin-2 for the Treatment of Metastatic Melanoma*.

*RAC Recommends Approval: 9-10-93/NIH Approval: 12-2-93

9312-059 (Closed) Gene Therapy/Phase I/Cancer/Leptomeningeal Carcinomatosis/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intraventricular Injection/Subarachnoid Injection

Oldfield, Edward H. and Ram, Zvi; National Institutes of Health, Bethesda, Maryland; *Intrathecal Gene Therapy for the Treatment of Leptomeningeal Carcinomatosis*. Sponsor: Genetic Therapy, Inc./Novartis

*RAC Recommends Approval: 12-2-93/NIH Approval: 1-20-94
Closed: 1/95

9312-060 (Open) Gene Therapy/Phase I/Cancer/Colon/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/In Combination with Untransduced Autologous Tumor Cells/Retrovirus/Interleukin-2 cDNA/Subcutaneous Injection

Sobol, Robert E. and Royston, Ivor; San Diego Regional Cancer Center, San Diego, California; *Injection of Colon Carcinoma Patients with Autologous Irradiated Tumor Cells and Fibroblasts Genetically Modified to Secrete Interleukin-2*.

*RAC Recommends Approval: 12-2-93/NIH Approval: 1-4-95

9312-061 (Closed) Gene Therapy/Phase I/Monogenic Disease/Gaucher Disease/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/Glucocerebrosidase cDNA/Intravenous

Schuening, Friedrich; Fred Hutchinson Cancer Research Center, Seattle, Washington; *Retrovirus-Mediated Transfer of the cDNA for Human Glucocerebrosidase into Peripheral Blood Repopulating Cells of Patients with Gaucher's Disease*.

*RAC Recommends Approval: 12-2-93/NIH Approval: 11-15-94
Closed: 4-29-97

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9312-062 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1IIIB Envelope Protein/Intramuscular Injection

Haubrich, Richard; University of California at San Diego Treatment Center, San Diego, California; and Merritt, James A.; Viagene, Inc., San Diego, California; *An Open Label, Phase I/II Clinical Trial to Evaluate the Safety and Biological Activity of HIV-IT(V) (HIV-1 IIIBenv/rev Retroviral Vector) in HIV-1 Infected Subjects*.

*RAC Recommends Approval: 12-3-93/NIH Approval: 4-19-94
Closed: 10-13-94

9312-063 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Lipofectin (Gibco BRL)/B7 (CD80) cDNA/Neomycin Phosphotransferase cDNA/Subcutaneous Injection

Sznol, Mario; National Institutes of Health, Frederick, Maryland; *A Phase I Trial of B7-Transfected Lethally Irradiated Allogeneic Melanoma Cell Lines to Induce Cell Mediated Immunity Against Tumor-Associated Antigens Presented by HLA-A2 or HLA-A1 in Patients with Stage IV Melanoma*.

*RAC Recommends Approval: 12-3-93/NIH Approval: 4-19-94

9312-064 (Closed) Gene Therapy/Phase I/Cancer/Colon/Hepatic Metastases/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral/Hepatic Injection

Rubin, Joseph; Mayo Clinic, Rochester, Minnesota; *Phase I Study of Immunotherapy of Advanced Colorectal Carcinoma by Direct Gene Transfer into Hepatic Metastases*. Sponsor: Vical, Incorporated

*RAC Recommends Approval: 12-3-93/NIH Approval: 4-19-94
Closed: 3-16-95 (Closed to accrual - maximum number of subjects entered)

9312-065 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Used in Combination with Anti-CD3 and Interleukin-2 Primed Autologous Lymph Node Cells to Prime Autologous Peripheral Blood Cells In Vitro/Retrovirus/GM-CSF cDNA/Intravenous

Chang, Alfred E.; University of Michigan, Ann Arbor, Michigan; *Adoptive Immunotherapy of Cancer with Activated Lymph Node Cells Primed In Vivo with Autologous Tumor Cells Transduced with the GM-CSF Gene*.

*RAC Recommends Approval: 12-3-93/NIH Approval: 8-23-94

9312-066 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cationic Liposome Complex/DMRIE-DOPE/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal

Sorscher, Eric J. and Logan, James L.; University of Alabama, Birmingham, Alabama; *Gene Therapy for Cystic Fibrosis Using Cationic Liposome Mediated Gene Transfer: A Phase I Trial of Safety and Efficacy in the Nasal Airway*.

*RAC Recommends Approval: 12-3-93/NIH Approval: 1-4-95
Closed: May 2000

9312-067 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Maxillary Sinus Epithelial Cells/Adenovirus/Serotype 2/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Maxillary Sinus Administration

Welsh, Michael J.; Howard Hughes Medical Institute, Iowa City, Iowa; *Adenovirus-Mediated Gene Transfer of CFTR to the Nasal Epithelium and Maxillary Sinus of Patients with Cystic Fibrosis*. Sponsor: Genzyme Corporation

*RAC Recommends Approval: 12-3-93/NIH Approval: 2-10-94
Protocol ended in May 1995

9403-068 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Gamma Interferon cDNA/Subcutaneous Injection

Rosenblatt, Joseph; University of California, Los Angeles, California; Seeger, Robert; Childrens Hospital, Los Angeles, California; and Merritt, James A.; Viagene, Inc., San Diego, California; *A Phase I Study of Immunization with Gamma Interferon Transduced Neuroblastoma Cells*.

*RAC Recommends Approval: 3-3-94/NIH Approval: 10-25-94

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9403-069 (Closed) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vitro/CD8+ Syngeneic Peripheral Blood Cells/Retrovirus/CD4-zeta Chimeric Receptor/Intravenous/Concurrent Interleukin-2 Therapy

Walker, Robert; National Institutes of Health, Bethesda, Maryland; *A Phase I/II Pilot Study of the Safety of the Adoptive Transfer of Syngeneic Gene-Modified Cytotoxic T-Lymphocytes in HIV-Infected Identical Twins*. Sponsor: NIH/Cell Genesys, Inc.

*RAC Recommends Approval: 3-3-94/NIH Approval: 8-23-94
Closed: 2-97

9403-070 (Open) Gene Therapy/Phase I/Monogenic Disease/Alpha-1-Antitrypsin Deficiency/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Cationic Liposome Complex/DC-Chol-DOPE/Alpha-1 Antitrypsin cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)

Brigham, Kenneth; Clinical Research Center at Vanderbilt University Medical Center, Nashville, Tennessee; *Expression of an Exogenously Administered Human Alpha-1-Antitrypsin Gene in the Respiratory Tract of Humans*. Sponsor: Gene Medicine, Inc.

*RAC Recommends Approval: 3-3-94/NIH Approval: 10-25-94

9403-071 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral/Direct Injection

Vogelzang, Nicholas; the University of Chicago, Chicago, Illinois; *Phase I Study of Immunotherapy for Metastatic Renal Cell Carcinoma by Direct Gene Transfer into Metastatic Lesions*. Sponsor: Vical, Incorporated

*RAC Recommends Approval: 3-4-94/NIH Approval: 4-19-94
Closed: 4-5-95 (Closed to accrual - maximum number of subjects entered)

9403-072 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin/cDNA/Intratumoral/Direct Injection

Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; and Akporiaye; Harris; Stopeck; Unger; and Warneke; University of Arizona, Tucson, Arizona; *Phase I Study of Immunotherapy of Malignant Melanoma by Direct Gene Transfer*. Sponsor: Vical, Incorporated

*RAC Recommends Approval: 3-4-94/NIH Approval: 4-19-94
Closed: 3-27-95 (Closed to accrual - maximum number of subjects entered)

9406-073 (Open) Gene Therapy/Phase I/Colon/Immunotherapy/In Vivo/Autologous Tumor Cells/Plasmid DNA/Carcinoembryonic Antigen Plasmid Expression Vector/Kanamycin Resistance cDNA/Intratumoral/Direct Injection

Curiel, David; University of Alabama, Birmingham, Alabama; *Phase I Trial of a Polynucleotide Augmented Anti-Tumor Immunization to Human Carcinoembryonic Antigen in Patients with Metastatic Colorectal Cancer*.

*RAC Recommends Approval: 6-10-95/NIH Approval: 7-27-95

9406-074 (Closed) Gene Therapy/Phase I/Other/Rheumatoid Arthritis/In Vivo/Autologous Synovial Cells/Retrovirus/Interleukin-1 Receptor Antagonist Protein cDNA/Intrajoint/Metacarpal Phalangeal Joints

Evans, C. H. and Robbins, Paul; University of Pittsburgh, Pittsburgh, Pennsylvania; *Clinical Trial to Assess the Safety, Feasibility, and Efficacy of Transferring a Potentially Anti-arthritic Cytokine Gene to Human Joints with Rheumatoid Arthritis*.

*RAC Recommends Approval: 6-9-94/NIH Approval: 7-27-95

9406-075 (Closed) Gene Marking/Cancer/Ovarian/In Vitro/Autologous Peripheral Blood Cells/Autologous Tumor Infiltrating Lymphocytes/Retrovirus/Neomycin Phosphotransferase/cDNA/Intraperitoneal

Freedman, Ralph; M.D. Anderson Cancer Center, Houston, Texas; *Use of a Retroviral Vector to Study the Trafficking Patterns of Purified Ovarian TIL Populations Used in Intraperitoneal Adoptive Immunotherapy of Ovarian Cancer Patients: A Pilot Study*.

*RAC Recommends Approval: 6-9-94/NIH Approval: 7-12-94
No subjects entered, protocol withdrawn

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9406-076 (Closed) Gene Marking/Cancer/Pediatric Malignancies/In Vitro/CD34+ Autologous Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Heslop, Helen; Brenner, Malcolm, K.; and Krance, Robert; St. Jude Childrens Research Hospital, Memphis, Tennessee; *Use of Double Marking with Retroviral Vectors to Determine the Rate of Reconstitution of Untreated and Cytokine Expanded CD34(+) Selected Marrow Cells in Patients Undergoing Autologous Bone Marrow Transplantation*.

*RAC Recommends Approval: 6-9-94/NIH Approval: 7-12-94
Closed: November 1997

9406-077 (Closed) Gene Therapy/Phase I/Cancer/Breast/Chemoprotection/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Intravenous

Deisseroth, Albert; Hortobagyi, Gabriel; Champlin, Richard; and Holmes, Frankie; M.D. Anderson Cancer Center, Houston, Texas; *Use of Safety-Modified Retroviruses to Introduce Chemotherapy Resistance Sequences into Normal Hematopoietic Cells for Chemoprotection During the Therapy of Breast Cancer: A Pilot Trial*.

*RAC Recommends Approval: 6-9-94/NIH Approval: 7-12-94

9406-078 (Closed) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Fanconi Anemia Complementation Group C cDNA/Intravenous

Liu, Johnson, M. and Young, Neal S.; National Institutes of Health, Bethesda, Maryland; and Wagner, John E., University of Minnesota, Minneapolis, Minnesota; *Retroviral Mediated Gene Transfer of the Fanconi Anemia Complementation Group C Gene to Hematopoietic Progenitors of Group C Patients*.

*RAC Recommends Approval: 6-9-94/NIH Approval: 2-12-95
Closed: 1997, follow-up continuing

9406-079 (Closed) Gene Therapy/Phase I/Cancer/Non-small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral/Bronchoscope

Roth, Jack A.; M.D. Anderson Cancer Center, Houston, Texas; *Clinical Protocol for Modification of Tumor Suppressor Gene Expression and Induction of Apoptosis in Non-Small Cell Lung Cancer (NSCLC) with an Adenovirus Vector Expressing Wildtype p53 and Cisplatin*.

*RAC Recommends Approval: 6-10-94 and 9-11-95/NIH Approval: 9-21-95
Closed: December 1997

9406-080 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/In Combination with Untransduced Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection

Sobol, Robert and Royston, Ivor; San Diego Regional Cancer Center; San Diego, California; *Injection of Glioblastoma Patients with Tumor Cells Genetically Modified to Secrete Interleukin-2 (IL-2): A Phase I Study*.

*RAC Recommends Approval: 6-10-94/NIH Approval: 7-12-94

9406-081 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Lymphoma/Breast/Head and Neck Cancer/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-12 cDNA/Neomycin Phosphotransferase cDNA/Intratumoral/Direct Injection

Lotze, Michael T; University of Pittsburgh, Pittsburgh, Pennsylvania; *IL-12 Gene Therapy Using Direct Injection of Tumor with Genetically Engineered Autologous Fibroblasts*.

*RAC Recommends Approval: 6-10-94/NIH Approval: 2-10-95
Complete: January 2000

9408-082 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Subcutaneous Injection

Simons, Jonathan; Johns Hopkins Oncology Center, Baltimore, Maryland; *Phase I/II Study of Autologous Human GM-CSF Gene Transduced Prostate Cancer Vaccines in Patients with Metastatic Prostate Carcinoma*.

NIH/ORDA Approval: 8-3-94 (Accelerated Review)
Study closed, long-term follow-up continues: 7-16-01

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9409-083 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)

Zeitlin, Pamela L.; Johns Hopkins Childrens Center, Baltimore, Maryland and Flotte, Terence R., University of Florida, Gainesville, Florida; *A Phase I Study of an Adeno-associated Virus-CFTR Gene Vector in Adult CF Patients with Mild Lung Disease*. Sponsor: Targeted Genetics Corporation

*RAC Recommends Approval: 9-12-94/NIH Approval: 11-15-94

9409-084 (Open) Gene Therapy/Phase I/Cancer/Breast/Antisense /In Vivo/Autologous Tumor Cells/Retrovirus/c-fos Antisense RNA/c-myc Antisense/Intrapleural/Intraperitoneal

Holt, Jeffrey, and Arteaga, Carlos B.; Clinical Research Center at Vanderbilt University Medical Center, Nashville, Tennessee; *Gene Therapy for the Treatment of Metastatic Breast Cancer by In Vivo Infection with Breast-Targeted Retroviral Vectors Expressing Antisense c-fos or Antisense c-myc RNA*.

*RAC Recommends Approval: 9-12-94/NIH Approval: 1-4-95

9409-085 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Respiratory Epithelial Cells/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intranasal/Respiratory Tract Administration (Bronchoscope)/Multiple Dose

Crystal, Ronald G.; New York Hospital-Cornell Medical Center, New York, New York; *Evaluation of Repeat Administration of a Replication Deficient, Recombinant Adenovirus Containing the Normal Cystic Fibrosis Transmembrane Conductance Regulator cDNA to the Airways of Individuals with Cystic Fibrosis*.

*RAC Recommends Approval: 9-12-94/NIH Approval: 11-30-94

9409-086 (Closed) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Avectin™/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection

Lyerly, H. Kim; Duke University Medical Center, Durham, North Carolina; *A Pilot Study of Autologous Human Interleukin-2 Gene Modified Tumor Cells in Patients with Refractory or Recurrent Metastatic Breast Cancer*.

*RAC Recommends Approval: 9-12-94/NIH Approval: 10-25-94

9409-087 (Closed) Gene Therapy/Phase I/Monogenic Disease/Hunter Syndrome/In Vitro/Autologous Peripheral Blood Cells/Retrovirus/Iduronate-2-Sulfatase cDNA/Intravenous

Whitley, Chester B.; University of Minnesota, Minneapolis, Minnesota; *Retroviral-Mediated Transfer of the Iduronate-2-Sulfatase Gene into Lymphocytes for Treatment of Mild Hunter Syndrome (Mucopolysaccharidosis Type II)*.

*RAC Recommends Approval: 9-13-94/NIH Approval: 8-20-95

9409-088 (Closed) Gene Therapy/Phase I/Other/Peripheral Artery Disease/In Vivo/Vascular Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor cDNA/Intraarterial/Angioplasty Catheter/Hydrogel Coated Balloon

Isner, Jeffrey M. and Walsh, Kenneth; St. Elizabeth's Medical Center, Tufts University, Boston, Massachusetts; *Arterial Gene Transfer for Therapeutic Angiogenesis in Patients with Peripheral Artery Disease*.

*RAC Recommends Approval: 9-13-94/NIH Approval: 11-15-94
Follow-up has been completed: 11-29-01

9409-089 (Closed) Gene Therapy/Phase I/Cancer/Central Nervous System/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Stereotactic Injection

Eck, Stephen L. and Alavi, Jane B.; University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *Treatment of Advanced CNS Malignancy with the Recombinant Adenovirus H5.020RSVTK: A Phase I Trial*.

*RAC Recommends Approval: 9-13-94/NIH Approval: 2-2-96

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9409-090 (Closed) Gene Therapy/Phase I/Cancer/n/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intrapleural

Albelda, Steven M.; University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *Treatment of Advanced Mesothelioma with the Recombinant Adenovirus H5.010RSVTK: A Phase I Trial.*

*RAC Recommends Approval: 9-13-94/NIH Approval: 1-4-95

9409-091 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Respiratory Epithelial Cells/Adenovirus/Serotype 2/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Respiratory Epithelial Cells/Bronchoscope

Dorkin, Henry L.; New England Medical Center, Tufts University, Boston, Massachusetts; and Lapey, Allen; Massachusetts General Hospital, Harvard Medical School, Boston, Massachusetts; *Adenovirus Mediated Gene Transfer for Cystic Fibrosis: Safety of Single Administration in the Lung (lobar instillation)*. Sponsor: Genzyme Corporation

NIH/ORDA Approval: 10-5-94 (Accelerated Review)
Protocol ended in December 1997

9411-092 (Closed) Gene Marking/Cancer/Lymphoma/Breast/In Vitro/CD34+ Autologous Bone Marrow Cells/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Douer, Dan; University of Southern California; Kenneth Norris Comprehensive Cancer Center and Hospital, Los Angeles, California; *High Dose Chemotherapy and Autologous Bone Marrow plus Peripheral Blood Stem Cell Transplantation for Patients with Lymphoma or Metastatic Breast Cancer: Use of Marker Genes to Investigate the Biology of Hematopoietic Reconstitution in Adults*.

NIH/ORDA Approval: 11-18-94 (Accelerated Review)
Notification that trial has been closed: 6-13-01

9411-093 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Subcutaneous Injection

Dranoff, Glen; Dana Farber Cancer Institute, Boston, Massachusetts; *A Phase I Study of Vaccination with Autologous, Irradiated Melanoma Cells Engineered to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor*.

NIH/ORDA Approval: 11-23-94 (Accelerated Review)
Study closed, long-term follow-up continues: 7-16-01

9412-094 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Respiratory Epithelial Cells/Adenovirus/Serotype 2/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Respiratory Epithelial Cells/Aerosol Administration

Dorkin, Henry L.; New England Medical Center, Tufts University, Boston, Massachusetts; and Lapey, Allen; Massachusetts General Hospital, Harvard Medical School, Boston, Massachusetts; *Adenovirus Mediated Gene Transfer for Cystic Fibrosis: Safety of a Single Administration in the Lung (aerosol administration)*. Sponsor: Genzyme Corporation

*RAC Recommends Approval: 12-1-94/NIH Approval: 7-24-95
Protocol ended in December 1997

9412-095 (Closed) Gene Therapy/Phase I/Solid Tumors/Lymphoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1102/Cytokine/Interleukin-2 cDNA/Intratumoral/Direct Injection

Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; and Rinehart, John; Scott and White Clinic; Temple Texas. *Phase I Trial of Interleukin-2 Plasmid DNA/DMRIE/DOPE Lipid Complex as an Immunotherapeutic Agent in Solid Malignant Tumors or Lymphomas by Direct Gene Transfer*. Sponsor: Vical, Incorporated

*RAC Recommends Approval: 12-1-94/NIH Approval: 3-2-95

9412-096 (Closed) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral/Bronchoscope

Clayman, Gary; M.D. Anderson Cancer Center, Houston, Texas; *Clinical Protocol for Modification of Tumor Suppressor Gene Expression in Head and Neck Squamous Cell Carcinoma (HNSCC) with an Adenovirus Vector Expressing Wild-type p53*.

*RAC Recommends Approval: 12-2-94 and 9-11-95/NIH Approval: 9-21-95
Closed: July 1997

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9412-097 (Open) Gene Therapy/Phase I/Cancer/Colon/Hepatic Metastases/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intrahepatic/Hepatic Artery/Bolus Infusion

Venook, Alan and Warren, Robert; Moffitt-Long Hospital of the University of California, San Francisco Medical Center; *Gene Therapy of Primary and Metastatic Malignant Tumors of the Liver Using ACN53 Via Hepatic Artery Infusion: A Phase I Study*. Sponsor: Schering Plough Corporation (formerly Canji)

RAC Recommends Approval Contingent Upon Meeting Stipulations: 12-2-94

9412-098 (Open) Gene Therapy/Phase I/Cancer/Central Nervous System Malignancies/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intra- tumoral/Stereotactic Injection

Grossman, Robert and Woo, Savio; The Methodist Hospital, Houston, Texas; *Phase I Study of Adenoviral Vector Delivery of the HSV-TK Gene and the Intravenous Administration of Ganciclovir in Adults with Malignant Tumors of the Central Nervous System*.

*RAC Recommends Approval: 12-2-94/NIH Approval: 2-2-96

9502-099 (Closed) Gene Therapy/Phase I/Cancer/Astrocytoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Stereotactic Injection

Fetell, Michael; Columbia Presbyterian Medical Center, New York, New York; Warnick, Ronald; University of Cincinnati, Cincinnati, OH; Yung, W.K. Alfred; M.D. Anderson Cancer Center, Houston, Texas; Maria, Bernard L.; University of Florida, Gainesville, Florida; Shaffrey, Mark; University of Virginia Health Sciences Center, Charlottesville, Virginia; Ram, Zvi; Chaim Sheba Medical Center, Tel Aviv University Sackler School of Medicine, Tel Hashomer, Israel; Prados, Michael; University of California, San Francisco, California; and Grossman, Stuart; Johns Hopkins University Hospital Oncology Center; Baltimore, Maryland; *Stereotactic Injection of Herpes Simplex Thymidine Kinase Vector Producer Cells (PA317/G1TkSvNa.7) and Intravenous Ganciclovir for the Treatment of Recurrent Malignant Glioma*. Sponsor: Genetic Therapy, Inc./Novartis

NIH/ORDA Approval: 2-10-95 (Accelerated Review)
Closed: April 1997

9503-100 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intraperitoneal/Catheter

Link, Charles; Human Gene Therapy Research Institute; and Moorman, Donald; Iowa Methodist Medical Center, Des Moines, Iowa; *A Phase I Trial of In Vivo Gene Therapy with Herpes Simplex Thymidine Kinase/Ganciclovir System for the Treatment of Refractory or Recurrent Ovarian Cancer*.
*RAC Recommends Approval: 3-6-95/NIH Approval: 7-27-95

9503-101 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-7 cDNA/Hygromycin Phosphotransferase/Herpes Simplex Virus Thymidine Kinase cDNA/Subcutaneous Injection

Economou, James; Glaspy, John; and McBride, William; University of California, Los Angeles, California; *A Phase I Testing of Genetically Engineered Interleukin-7 Melanoma Vaccines*.

*RAC Recommends Approval: 3-6-95/NIH Approval: 8-20-95
Closed: 3-97

9503-102 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/HLA-Matched Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-2 cDNA/Gamma Interferon cDNA/Subcutaneous Injection

Gansbacher, Bernd; Memorial Sloan Kettering Cancer Center, New York, New York; *Phase I/II Study of Immunization with MHC Class I Matched Allogeneic Human Prostatic Carcinoma Cells Engineered to Secrete Interleukin-2 and Interferon-γ*.

RAC Recommends Approval Contingent Upon Meeting Stipulations: 3-6-95

9503-103 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/Antisense TAR/Transdominant Rev/Intravenous

Tavel, Jorge; National Institutes of Health, Bethesda, Maryland; *Gene Therapy for AIDS Using Retroviral Mediated Gene Transfer to Deliver HIV-1 Antisense TAR and Transdominant Rev Protein Genes to Syngeneic Lymphocytes in HIV Infected Identical Twins*.

*RAC Recommends Approval: 3-7-95/NIH Approval: 4-1-95
Closed to new enrollment. Individuals will be followed, long-term, in a new protocol (that does not involve administration of recombinant DNA): 1-17-02

9503-104 (Closed) Gene Therapy/Phase I/Monogenic Disease/Chronic Granulomatous Disease In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/p47phox/Intravenous

Malech, Harry; National Institutes of Health, Bethesda, Maryland; *Gene Therapy Approach for Chronic Granulomatous Disease*.

*RAC Recommends Approval: 3-7-95/NIH Approval: 4-15-95
Closed to enrollment: 4-12-04

9503-105 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1IIIB Envelope Protein/Intramuscular Injection

Parenti, David; George Washington University Medical Center, Washington, D.C.; Haubrich, Richard; University of California San Diego Treatment Center, San Diego, California; Frame, Peter; University of Cincinnati AIDS Treatment Center, Cincinnati, Ohio; Powderly, William; Washington University AIDS Clinical Trials Unit; St. Louis, Missouri; and Loveless, Mark; Oregon Health Sciences University, Portland, Oregon; *A Repeat Dose Safety and Efficacy Study of HIV-IT(V) in HIV-1 Infected Subjects with Greater Than or Equal to 100 CD4+ T Cells and No AIDS Defining Symptoms*.

NIH/ORDA Approval: 3-11-95 (Accelerated Review)
Notification that trial has been completed, IND is inactive: 5-22-00

9506-106 (Open) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/Autologous G-CSF and ATA-C Mobilized Bone Marrow Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Verfaillie, Catherine; University of Minnesota, Minneapolis, Minnesota; *Autologous Marrow Transplantation for Chronic Myelogenous Leukemia Using Stem Cells Obtained After In Vivo Chemotherapy Cytokine Priming*.

NIH/ORDA Approval: 5-5-95

9506-107 (Closed) Gene Therapy/Phase I/Cancer/Multiple Myeloma/Pro-Drug/In Vitro/Allogeneic T Lymphocytes/Retrovirus/Herpes Simplex Thymidine Kinase/Ganciclovir/Intravenous

van Rhee, Frits and Barlogie, Bart; University of Arkansas for Medical Sciences, Little Rock, Arkansas; *Thymidine Kinase (TK) Transduced Donor Leukocyte Infusions as a Treatment for Patients with Relapsed or Persistent Multiple Myeloma after T-cell Depleted Allogeneic Bone Marrow Transplant*.
Sponsor: Genetic Therapy, Inc./Novartis

*RAC Recommends Approval: 6-9-95/NIH Approval: 7-27-95
Closed: 5-30-03

9506-108 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Subcutaneous Injection

Fox, Bernard A. and Urba, Walter J.; Earle A. Chiles Research Institute, Providence Medical Center, Portland, Oregon; *Adoptive Cellular Therapy of Cancer Combining Direct HA-B7/β-2 Microglobulin Gene Transfer with Autologous Tumor Vaccination for the Generation of Vaccine-Primed Anti-CD3 Activated Lymphocytes*.

*RAC Recommends Approval: 6-9-95/NIH Approval: 9-30-95

9506-109 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vitro/Anti-CD3 Stimulated Autologous Peripheral Blood Lymphocytes/Retrovirus/Antibody/MOv-gamma (Reactive with Folate Binding Protein)/Intravenous/Intraperitoneal

Hwu, Patrick; National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Advanced Epithelial Ovarian Cancer Using Anti-CD3 Stimulated Peripheral Blood Lymphocytes Transduced with a Gene Encoding a Chimeric T-cell Receptor Reactive with Folate Binding Protein*.

RAC Recommends Approval Contingent Upon Meeting Stipulations: 6-9-95

9506-110 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/DDAB-DOPE/Cytokine/Interleukin-2 cDNA/Intradermal Injection

Berchuck, Andres and Lyerly, H. Kim; Duke University Medical Center, Durham, North Carolina; *A Phase I Study of Autologous Human Interleukin-2 (IL-2) Gene Modified Tumor Cells in Patients with Refractory Metastatic Ovarian Cancer*.

*RAC Recommends Approval: 6-10-95/NIH Approval: 9-30-95

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9506-111 (Closed) Gene Therapy/Phase I/Monogenic Disease/Purine Nucleoside Phosphorylase Deficiency/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/Purine Nucleoside Phosphorylase cDNA/Intravenous

McIvor, R. Scott; Institute of Human Genetics, University of Minnesota, Minneapolis, Minnesota; *Gene Therapy for Purine Nucleoside Phosphorylase Deficiency*.

*RAC Recommends Approval: 6-9-95/NIH Approval: 7-27-95
Closed: 9-24-00 No individuals enrolled, IND not submitted

9506-112 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Single Chain Antibody Gene/In Vitro/CD4+ Autologous Peripheral Blood Lymphocytes/Retrovirus/sFv105 Anti-HIV-1 Envelope Protein(gp160)Gene/Intravenous

Marasco, Wayne A.; Dana Farber Cancer Institute, Boston, Massachusetts; *Intracellular Antibodies Against HIV-1 Envelope Protein for AIDS Gene Therapy*.
*RAC Recommends Approval: 6-9-95/NIH Approval: 7-27-95
Never activated: 4-21-04

9504-113 (Closed) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus-1/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1IIIB Envelope Protein/Intramuscular Injection

Conant, Marcus, Conant Medical Group; Lang, William, ViRx, Inc.; and Merritt, James, Viagene, Inc., San Francisco, California; *A Randomized, Double Blinded, Phase I/II Dosing Study to Evaluate the Safety and Optimal CTL Inducing Dose of HIV-IT(V) in Pre-Selected HIV-1 Infected Subjects*.

*RAC Recommends Approval: NA/NIH Approval: NA (Non-NIH funded institution)
FDA Approval: 5-6-94

9507-114 (Closed) Gene Therapy/Phase I-II/Monogenic Disease/Cystic Fibrosis/In Vivo/Maxillary Sinus Epithelial Cells/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Maxillary Sinus Administration

Gardner, Phyllis; Stanford University School of Medicine, Stanford, California; *A Phase I/II Study of tg-CF for the Treatment of Chronic Sinusitis in Patients with Cystic Fibrosis*. Sponsor: Targeted Genetics Corporation

Sole FDA Review Recommended by NIH/ORDA: 7-11-95

9508-115 (Closed) Gene Therapy/Phase II/Cancer/Metastatic Malignancies(Breast Adenocarcinoma, Renal Cell Carcinoma, Melanoma, Colorectal Adenocarcinoma, non-Hodgkin's Lymphoma)/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL 1005/HLA-B7/Beta-2 Microglobulin cDNA/Direct Intratumoral Injection

Chang, Alfred E.; University of Michigan Medical Center, Ann Arbor, Michigan; Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; Vogelzang, Nicholas; University of Chicago Medical Center, Chicago, Illinois; Levy, Ronald; Stanford University Medical Center, Palo Alto, California; Redman, Bruce; Wayne State University School of Medicine; Detroit, Michigan; Figlin, Robert; University of California Medical Center, Los Angeles, California; Rubin, Joseph; Mayo Foundation for Medical Evaluation and Research, Rochester, Minnesota; Rinehart, John J.; Scott and White Hospital, Texas A & M University, Temple Texas; Doroshow, James H.; City of Hope National Medical Center, Duarte, California; Klasa, Richard; British Columbia Cancer Agency, Vancouver, British Columbia; Sobol, Robert; Sidney Kimmel Cancer Center, San Diego, California; *Phase II Study of Immunotherapy of Metastatic Cancer by Direct Gene Transfer*. Sponsor: Vical, Incorporated

Sole FDA Review Recommended by NIH/ORDA: 8-2-95

9508-116 (Closed) Gene Therapy/Phase I/Cancer/Glioma/Immunotherapy/In Vitro/Autologous Tumor (Glioma) Cells/Non-Irradiated/Retrovirus/Cytokine/Interleukin-4 cDNA/Subcutaneous Injection

Pollack, Ian; Okada, Hideho; and Lotze, Michael T.; University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; *Gene Therapy of Malignant Gliomas: A Phase I Study of IL-4 Gene -Modified Autologous Tumor to Elicit an Immune Response*.

Sole FDA Review Recommended by NIH/ORDA: 8-7-95
Closed: 3-15-05

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9508-117 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus-1/Replication Inhibition/In Vitro/Autologous CD34+ Peripheral Blood Cells/Retrovirus/Hammerhead Ribozyme/Intravenous

Mitsuyasu, Ronald; University of California Los Angeles, California; *A Phase I Trial of Autologous CD34+ Hematopoietic Progenitor Cells Transduced with an Anti-HIV-1 Ribozyme*.

Sole FDA Review Recommended by NIH/ORDA: 8-7-95
Closed to accrual: 1-24-00

9508-118 (Open) Gene Therapy/Phase I/Other/Restenosis/In Vivo/Vascular Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor cDNA/Intraarterial/Angioplasty Catheter/Hydrogel Coated Balloon

Losordo, Douglas W.; St. Elizabeth's Medical Center, Tufts University School of Medicine, Boston, Massachusetts; *Accelerated Re-endothelialization and Reduced Neointimal Thickening Following Catheter Transfer of phVEGF165*.

Sole FDA Review Recommended by NIH/ORDA: 8-7-95

9508-119 (Open) Gene Therapy/Phase I/Human Immunodeficiency Virus-1/In Vitro/CD8+ Allogeneic Cytotoxic T Lymphocytes/CD8+ Syngeneic Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase/Herpes Simplex Virus Thymidine Kinase cDNA/Retrovirus/Intravenous

Riddell, Stanley R.; Fred Hutchinson Cancer Research Center, Seattle, Washington; *Phase I Study to Evaluate the Safety of Cellular Adoptive Immunotherapy Using Autologous Unmodified and Genetically Modified CD8+ HIV-Specific T Cells in HIV Seropositive Individuals*. Sponsor: Targeted Genetics Corporation

Sole FDA Review Recommended by NIH/ORDA: 8-7-95

9508-120 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Used to Derive Tumor Infiltrating Lymphocytes/HLA-B7 cDNA/Intravenous

Chang, Alfred E. and Nabel, Gary J.; University of Michigan Medical Center, Ann Arbor, Michigan; *Phase I Study of Tumor-Infiltrating Lymphocytes Derived from In Vivo HLA-B7 Gene Modified Tumors in the Adoptive Immunotherapy of Melanoma*.

Sole FDA Review Recommended by NIH/ORDA: 8-14-95

9508-121 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell/Immunotherapy/In Vivo/Autologous Tumor Cells/HLA B7 cDNA/Intratumoral/Concurrent Interleukin-2 Therapy

Figlin, Robert A.; University of California Los Angeles Medical Center, Los Angeles, California; *Phase I Study of HLA-B7 Plasmid DNA/DMRIE/DOPE Lipid Complex as an Immunotherapeutic Agent in Renal Cell Carcinoma by Direct Gene Transfer with Concurrent Low Dose Bolus IL-2 Protein Therapy*. Sponsor: Vical, Incorporated

Sole FDA Review Recommended by NIH/ORDA: 8-14-95

9508-122 (Closed) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies (type of cancer not specified)/Immunotherapy/In Vivo/Autologous Muscle Cells/Canarypox Virus/Carcinoembryonic Antigen cDNA/Intramuscular Injection

Hawkins, Michael J. and Marshall, John L.; Georgetown University Medical Center, Washington, D.C.; *A Study of Recombinant ALVAC Virus that Expresses Carcinoembryonic Antigen in Patients with Advanced Cancers*.

Sole FDA Review Recommended by NIH/ORDA 8-14-95
Closed: 2-97

9509-123 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Antisense/In Vivo/Autologous Tumor Cells/Retrovirus/Antisense c-myc RNA/Intraprostate Injection

Steiner, Mitchell S., Clinical Research Center at Vanderbilt University Medical Center, Nashville, Tennessee; and Holt, Jeffrey T., Vanderbilt University School of Medicine, Nashville, Tennessee; *Gene Therapy for the Treatment of Advanced Prostate Cancer by In Vivo Transduction with Prostate-Targeted Retroviral Vectors Expressing Antisense c-myc RNA*.

*RAC Recommends Approval: 9-11-95/NIH Approval: 9-30-95

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9509-124 (Closed) Gene Therapy/Phase I/Cancer/Ovarian and Extraovarian/Anti-erbB-2 Single Chain Antibody Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Anti-erbB-2 (oncoprotein/extracellular domain) Single-chain Antibody Gene/Intraperitoneal Injection

Curiel, David T. and Alvarez, Ronald D.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Study of Recombinant Adenovirus Vector-Mediated Delivery of an Anti-erbB-2 Single Chain (sFv) Antibody Gene for Previously Treated Ovarian and Extraovarian Cancer Patients*.

RAC Recommends Approval Contingent Upon Meeting Stipulations: 9-11-95
Completed: 4-13-99

9509-125 (Closed) Gene Therapy/Phase I/Cancer/Colon Carcinoma (Hepatic Metastases)/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/E. coli Cytosine Deaminase cDNA/Intratumoral (Hepatic) Injection/Combined with Oral 5-Fluorocytosine

Crystal, Ronald, G.; Hershowitz, Edward; and Lieberman, Michael; New York Hospital-Cornell Medical Center, New York, New York; *A Phase I Study of Direct Administration of a Replication-Deficient Adenovirus Vector Containing the E. coli Cytosine Deaminase Gene to Metastatic Colon Carcinoma of the Liver in Association with the Oral Administration of the Pro-Drug 5-Fluorocytosine*.

*RAC Recommends Approval: 9-11-95/NIH Approval: 9-30-95
Notification that IND has been withdrawn: 2-2-00

9509-126 (Open) Gene Therapy/Phase I/Cancer/Prostate Adenocarcinoma/Immunotherapy/In Vivo/Vaccination/Vaccinia Virus/Prostate Specific Antigen/Intradermal Injection

Chen, A.P.; National Naval Medical Center, Bethesda, Maryland; *A Phase I Study of Recombinant Vaccinia that Expresses Prostate Specific Antigen in Adult Patients with Adenocarcinoma of the Prostate.*

Sole FDA Review Recommended by NIH/ORDA: 9-22-95

9509-127 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cationic Liposome Complex/DOPE/Cystic Fibrosis Transmembrane Conductance Regulator cDNA; Intranasal Administration

Welsh, Michael J. and Zabner, Joseph; Howard Hughes Medical Institute, University of Iowa College of Medicine, Iowa City, Iowa; *Cationic Lipid Mediated Gene Transfer of CFTR: Safety of a Single Administration to the Nasal Epithelia.* Sponsor: Genzyme Corporation

Sole FDA Review Recommended by NIH/ORDA: 9-26-95
Completed: 12-97

9510-128 (Closed) Gene Therapy/Phase I/Cancer/Gastrointestinal Tract, Breast, or Lung Adenocarcinoma (CEA-Expressing Malignancies)/Immunotherapy/In Vivo/Vaccination/Vaccinia Virus/Carcinoembryonic Antigen/Intradermal Injection in Combination with Subcutaneous Peptide Challenge

Cole, David J.; Medical University of South Carolina, Charleston, South Carolina; *Phase I Study of Recombinant CEA Vaccinia Virus Vaccine with Post Vaccination CEA Peptide Challenge.*

Sole FDA Review Recommended by NIH/ORDA: 10-16-95
Closed: 9-99

9510-129 (Open) Gene Marking/Cancer/EBV-Positive Hodgkin Disease/In Vitro/EBV-Specific Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Roskrow, Marie; Hudson, Melissa; Rooney, Cliona; Heslop, Helen; and Brenner, Malcolm; St. Jude Children's Research Hospital, Memphis, Tennessee; *Administration of Neomycin Resistance Gene Marked EBV Specific Cytotoxic T Lymphocytes as Therapy for Patients Receiving a Bone Marrow Transplant for Relapsed EBV-Positive Hodgkin Disease.*

Sole FDA Review Recommended by NIH/ORDA: 10-17-95

9510-130 (Open) Gene Marking/Cancer/EBV-Positive Hodgkin Disease/In Vitro/EBV-Specific Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Administration

Roskrow, Marie; Hudson, Melissa; Rooney, Cliona; Heslop, Helen; and Brenner, Malcolm; St. Jude Children's Research Hospital, Memphis, Tennessee; *Administration of Neomycin Resistance Gene Marked EBV Specific Cytotoxic T Lymphocytes to Patients with Relapsed EBV-Positive Hodgkin Disease.*

Sole FDA Review Recommended by NIH/ORDA: 10-17-95

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9510-131 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD8+ T Cells/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous

Connick, Elizabeth; University of Colorado Health Sciences Center, Denver, Colorado; and Deeks, Steven G.; University of California, San Francisco General Hospital, San Francisco, California; *A Randomized, Controlled, Phase II Study of the Activity and Safety of Autologous CD4-Zeta Gene-Modified T Cells in HIV-Infected Patients.* Sponsor: Cell Genesys, Inc.

Sole FDA Review Recommended by NIH/ORDA: 10-17-95
Closed 8-6-97 (No longer enrolling patients)

9510-132 (Open) Gene Therapy/Phase I/Cancer/Locally Advanced or Metastatic Prostate/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Cytokine/Interleukin-2 cDNA/Intradermal Injection

Paulson, David; and Lyerly, H. Kim; Duke University Medical Center, Durham, North Carolina; *A Phase I Study of Autologous Human Interleukin-2 (IL-2) Gene Modified Tumor Cells in Patients with locally Advanced or Metastatic Prostate Cancer.*

Sole FDA Review Recommended by NIH/ORDA: 10-19-95

9511-133 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous Tumor Cells (Non-irradiated)/Type 5 Adenovirus/Cytokine/Interleukin-2 cDNA/Subcutaneous Injection

Brenner, Malcolm K.; Dilloo, Dagmar; and Bowman, Laura; St. Jude Children's Research Hospital, Memphis, Tennessee; *Phase I Study of Cytokine Gene Modified Autologous Neuroblastoma Cells for Treatment of Relapsed/Refractory Neuroblastoma Using an Adenoviral Vector.*

Sole FDA Review Recommended by NIH/ORDA: 11-1-95

9511-134 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/Autologous CD4+ T Cells/Retrovirus/Neomycin Phosphotransferase Gene/PolyTAR Decoy Gene/RRE-polyTAR Decoy Gene

Greenberg, Philip D.; Fred Hutchinson Cancer Research Center, University of Washington Medical Center, Seattle, Washington; *Phase I Study to Evaluate the Safety and In Vivo Persistence of Adoptively Transferred Autologous CD4+ T Cells Genetically Modified to Resist HIV Replication.*

Sole FDA Review Recommended by NIH/ORDA: 11-1-95

Trial is closed to new accrual; follow-up will continue: 03-19-01

9511-135 (Closed) Gene Therapy/Phase I/Cancer/Ovarian and Extraovarian Cancer/Single Chain Antibody/In Vivo/Autologous Tumor Cells/Adenovirus/Herpes Simplex Thymidine Kinase Gene/Intraperitoneal Injection/Combined with Intravenous Ganciclovir Administration

Alvarez, Ronald D. and Curiel, David T.; University of Alabama Comprehensive Cancer Center, Birmingham, Alabama; *A Phase I Study of Recombinant Adenovirus Vector-Mediated Intraperitoneal Delivery of Herpes Simplex Virus Thymidine Kinase (HSV-TK) Gene and Intravenous Ganciclovir for Previously Treated Ovarian and Extraovarian Cancer Patients.*

Sole FDA Review Recommended by NIH/ORDA: 11-1-95
Completed: 5-04-00

9511-136 (Open) Gene Therapy/Phase I/Cancer/Metastatic Melanoma/Immunotherapy In Vitro/Autologous CD8+ Tyrosinase-Specific TCells/Retrovirus/Hygromycin Phosphotransferase/Intravenous Administration

Yee, Cassian and Greenberg, Philip D.; Fred Hutchinson Cancer Research Center, University of Washington Medical Center, Seattle, Washington; *Phase I Study to Evaluate the Safety of Cellular Adoptive Immunotherapy Using Autologous Unmodified and Genetically Modified CD8+ Tyrosinase-Specific T Cells in Patients with Metastatic Melanoma.*

Sole FDA Review Recommended by NIH/ORDA: 11-1-95

9512-137 (Closed) Gene Therapy/Phase I/Cancer/Ovarian,Breast/Oncogene Regulation/HER-2/*neu*/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal, Intrapleural Administration

Hortobagyi, Gabriel N.; Lopez-Berstein, Gabriel; and Hung, Mien-Chien; M.D. Anderson Cancer Center, Houston, Texas; Kilbourn, Robert, Rush-Presbyterian/St. Luke's Medical Center, Chicago, Illinois; Weiden, Paul; Virginia Mason Medical Center, Seattle, Washington; *Phase I Study of E1A Gene Therapy for Patients with Metastatic Breast or Ovarian Cancer that Overexpresses Her-2/neu*. Sponsor: Targeted Genetics Corporation

*RAC Recommends Approval: 12-4-95/NIH Approval: 2-2-96
No longer active: 11-10-03

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9512-138 (Closed) Gene Therapy/Phase I/Cancer/Malignant Glioma/Antisense/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Plasmid DNA--Electroporation/TGF-β2/Subcutaneous Injection

Black, Keith L.; and Fakhrai, Habib; University of California, Los Angeles, School of Medicine, Los Angeles, California; *A Phase I Study of the Safety of Injecting Malignant Glioma Patients with Irradiated TGF-β2 Antisense Gene Modified Autologous Tumor Cells*.

*RAC Recommends Approval: 12-4-95/NIH Approval: 4-2-96
Closed: 5-22-03

9512-139 (Open) Gene Therapy/Phase I/Monogenic Disease/Partial Ornithine Transcarbamylase (OTC) Deficiency/In Vivo/Autologous Peripheral Blood Cells/Adenovirus/Type 5 (E2a Temperature-Sensitive Mutant)/Ornithine Transcarbamylase cDNA/Intravenous

Batshaw, Mark; Institute for Human Gene Therapy, University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *A Phase I Study of Adenoviral Vector Mediated Gene Transfer to Liver in Adults with Partial Ornithine Transcarbamylase Deficiency*.

RAC Recommends Approval Contingent Upon Meeting Stipulations: 12-4-95

9512-140 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Type 2/MART-1 Melanoma Antigen/Subcutaneous Injection/Immunization

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Phase I Trial in Patients with Metastatic Melanoma of Immunization with a Recombinant Adenovirus Encoding the MART-1 Melanoma Antigen.*

Sole FDA Review Recommended by NIH/ORDA: 12-1-95
Closed: 9-17-99, follow-up continuing

9512-141 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus-1/Replication Inhibition/In Vitro/Autologous CD4+ Peripheral Blood Lymphocytes/Retrovirus/Anti-Rev SFv/Intravenous

Pomerantz, Roger J; Jefferson Medical College, Thomas Jefferson University, Philadelphia, Pennsylvania; *Intracellular Immunization Against HIV-1 Infection Using an Anti-Rev Single Chain Variable Fragment (SFv).*

Sole FDA Review Recommended by NIH/ORDA: 12-13-95

9512-142 (Closed) Gene Therapy/Phase I/Gene Therapy/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL 1005/HLA-B7/Beta-2 Microglobulin cDNA/Direct Intratumoral Injection

Gluckman, Jack L.; University of Cincinnati Medical Center, Cincinnati, Ohio; *Allovectin-7 in the Treatment of Squamous Cell Carcinoma of the Head and Neck*.

Sole FDA Review Recommended by NIH/ORDA: 12-15-95

9601-143 (Closed) Gene Therapy/Phase I/Cancer/Breast/Chemoprotection/In Vitro/Autologous CD34+ Peripheral Blood Lymphocytes//Retrovirus/Multi-Drug Resistance-1 cDNA/Neomycin Phosphotransferase cDNA/Intravenous

Cowan, Kenneth H.; National Institutes of Health, Bethesda, Maryland; *Antimetabolite Induction, High-Dose Alkylating Agent Consolidation, and Retroviral Transduction of the MDR1 Gene Into Peripheral Blood Progenitor Cells Followed by Intensification Therapy with Sequential Paclitaxel and Doxorubicin for Stage 4 Breast Cancer*.

Sole FDA Review Recommended by NIH/ORDA: 1-26-96
Closed: 6-14-00

9601-144 (Open) Gene Therapy/Phase I/Cancer/Prostate/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Intra-prostatic Tumor Injection

Scardino, Peter T.; Thompson, TImothy C.; and Woo, Savio L.C.; Baylor College of Medicine, Houston, Texas; *Phase I Study of Adenoviral Vector Delivery of the HSV-tk Gene and the Intravenous Administration of Ganciclovir in Men with Local Recurrence of Prostate Cancer after Radiation Therapy*.

Sole FDA Review Recommended by NIH/ORDA: 1-29-96

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9601-145 (Closed) Gene Therapy/Phase I/Cancer/Bladder/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Retinoblastoma cDNA/Intravesical Catheter Administration

Small, Eric J. and Carroll, Peter R.; University of California, San Francisco, California; *Gene Therapy of Bladder Cancer Using Recombinant Adenovirus Containing the Retinoblastoma Gene (ACNRB): A Phase IA Study*. Sponsor: Schering Plough Corporation (formerly Canji)

Sole FDA Review Recommended by NIH/ORDA: 1-30-96
Canceled: 4-4-97

9602-146 (Open) Gene Therapy/Phase I/Cancer/Hematologic Malignancies Following Allogeneic Bone Marrow Transplant/ProDrug/Elimination of Graft Versus Host Disease/In Vitro/Allogeneic Peripheral Blood Lymphocytes/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous

Link, Charles J.; Human Gene Therapy Research Institute, Des Moines, Iowa; Burt, Richard K. and Traynor, Ann; Northwestern University School of Medicine, Chicago, Illinois; *Adoptive Immunotherapy for Leukemia: Donor Lymphocytes Transduced with the Herpes Simplex Thymidine Kinase Gene for Remission Induction*.

Sole FDA Review Recommended by NIH/ORDA: 2-8-96

9602-147 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD34+ Autologous Bone Marrow Cells/Retrovirus/RRE Decoy Gene, and Retrovirus/Neomycin Phosphotransferase Gene/Intravenous

Kohn, Donald B.; Childrens Hospital Los Angeles, Los Angeles, California; *Transduction of CD34+ Cells from the Bone Marrow of HIV-1 Infected Children: Comparative Marking by an RRE Decoy and a Neutral Gene.*

Sole FDA Review Recommended by NIH/ORDA: 2-8-96
Study is in follow-up: 6-22-00

9602-148 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection

O'Malley, Bert W.; Johns Hopkins University, Baltimore, Maryland; *Phase I Study of Adenoviral Vector Delivery of the HSV-tk Gene and the Intravenous Administration of Ganciclovir in Adults with Recurrent or Persistent Head and Neck Cancer.*

Sole FDA Review Recommended by NIH/ORDA: 2-13-96

9603-149 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Retrovirus/BRCA-1 Gene/Intraperitoneal Administration (Ultrasound Guided)

Holt, Jeffrey T.; Clinical Research Center at Vanderbilt University Medical Center, Nashville, Tennessee; *Ovarian Cancer Gene Therapy with BRCA-1.*

Sole FDA Review Recommended by NIH/ORDA: 3-6-96
Closed: 07-99

9603-150 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy In Vivo/Autologous Tumor Cells/HLA B7/cDNA/Intratumoral/Concurrent Interleukin-2 Therapy

Hersh, Evan M.; Arizona Cancer Center, Tucson, Arizona; and Sondak, Vernon K.; University of Michigan Medical Center, Ann Arbor, Michigan; *Evaluation of Intratumoral Gene Therapy with HLA-B7/DMRIE/DOPE plus Subcutaneous Low Dose II-2*.

Sole FDA Review Recommended by NIH/ORDA; 3-26-96
Closed: 3-11-97. Protocol Never Initiated

9604-151 (Closed) Gene Therapy/Phase I/ Cancer/Melanoma/Immunotherapy In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 2/GP100 Melanoma Antigen/Subcutaneous or Intramuscular Injection/Concurrent Interleukin-2 Therapy

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Phase I Trial in Patients with Metastatic Melanoma of Immunization with a Recombinant Adenovirus Encoding the GP100 Melanoma Antigen.*

Sole FDA Review Recommended by NIH/ORDA: 4-19-96
Terminated: 9-17-99

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9604-152 (Closed) Gene Therapy/Phase I/Inherited Genetic Disorder/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/Correction In Vitro/CD34+ Autologous Umbilical Cord Blood or Bone Marrow/Retrovirus/cDNA for Common γ Chain of Multiple Cytokine Receptors/Intravenous

Weinberg, Kenneth I.; Childrens Hospital Los Angeles (CHLA) ; Los Angeles, California; *Gene Therapy for X-linked Severe Combined Immune Deficiency Using Retroviral Mediated Transduction of the γc cDNA into CD34+ Cells.*

Sole FDA Review Recommended by NIH/ORDA: 4-24-96
Study replaced by 0108-494; never initiated: 5-28-03

9604-153 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Hammerhead Ribozyme/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Tat and Rev Hammerhead Ribozyme/Intravenous

Kohn, Donald B.; Childrens Hospital of Los Angeles (CHLA), Los Angeles, California; and Zaia, John A.; City of Hope National Medical Center, Duarte, California; *Transduction of CD34+ Autologous Peripheral Blood Progenitor Cells from HIV-1 Infected Persons: a Phase I Study of Comparative Marking Using a Ribozyme Gene and a Neutral Gene.*

Sole FDA Review Recommended by NIH/ORDA: 4-24-96

9605-154 (Closed) Gene Therapy/Phase I/Cancer/Brain Tumors/Pro-Drug/In Vivo/Autologous Tumor Cells/psiCRIP-MFG-S-TK1-67 Cells/Retrovirus/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection

Harsh IV, Griffith R.; Chiocca, E. Antonio; and Hochberg, Fred H.; Harvard Medical School, Boston, Massachusetts; *Phase I Study of Retroviral-Mediated Incorporation of the HSV Thymidine Kinase Gene and Ganciclovir in Malignant Gliomas.*

Sole FDA Review Recommended by NIH/ORDA: 5-1-96
Terminated: 6-11-98

9605-155 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Cationic Liposome Complex/B7(CD80) cDNA/Retrovirus/Herpes Simplex Thymidine Kinase/Ganciclovir/Intraperitoneal

Freeman, Scott M.; and Robinson III, William R.; Tulane University School of Medicine, New Orleans, Louisiana; *Tumor Vaccination With HER-2/Neu Using a B7 Expressing Tumor Cell Line Prior To Treatment With HSV-TK Gene-Modified Cells.*

Sole FDA Review Recommended by NIH/ORDA: 5-2-96

9608-156 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/B7(CD80) cDNA/Subcutaneous Injection

Urba, Walter J.; Providence Portland Medical Center, Portland, Oregon; *Phase I Trial Using a CD80-Modified Allogeneic Breast Cancer Line to Vaccinate HLA-A2-Positive Women with Breast Cancer .*

Sole FDA Review Recommended by NIH/ORDA: 8-6-96

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9608-157 (Closed) Gene Therapy/Phase III of #9303-037/Cancer/Glioblastoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection

Maria, Bernard; University of Florida, Gainesville, Florida; Gutheil, John; Sharp Healthcare, Sidney Kimmel Cancer Center, San Diego, California; Bucholz, Richard; St. Louis University, St. Louis, Missouri; Olson, Jeffrey; Emory School of Medicine, Winship Cancer Center, Atlanta, Georgia; Lillehei, Kevin; University of Colorado, Denver, Colorado; Van Gilder, John; University of Iowa College of Medicine, Iowa City, Iowa; Nemunaitis, John; Texas Oncology P.A., Baylor University Medical Center, Dallas, Texas; Origitano, Thomas; Loyola University Medical Center, Maywood, Illinois; Warnick, Ronald; University of Cincinnati Medical Center, The Christ Hospital, Good Samaritan Hospital, Jewish Hospital of Cincinnati, Veterans Affairs Medical Center, Cincinnati, Ohio; Weber, Friederich Dr. med.; Heinrich Heine Universtat, Dusseldorf, Germany; Rainov, Nikolai, PD Dr. med.; Martin Luther Universtat, Halle, Germany; Cloughesy, Timothy; UCLA Department of Neurology, Reed Neurological Research Center, Boywer Oncology Clinic, Los Angeles, California; Markert, James; University of Alabama at Birmingham, Birmingham, Alabama; Matti Vapalahti, Kuopio Universiy Hopsital, Kuopio, Finland; Yasuhiro Yonekawa, University Hospital, Zurich, Switzerland; Nanno Harrie Mulder, Academic Hospital Groningen, Groningen, The Netherlands; Susanne Osante, Academic Hospital Leiden, Leiden, The Netherlands; Fetell, Michael; Columbia-Presbyterian Medical Center Neurological Institute, New York, New York; Schramm, Johannes; Prof. Dr. med., Univ. Klinikum Neurochirurgische Klinik, Bonn, Germany; Westphal, Manfred, PD Dr. med.; Klinikum Eppendorf Neurochirurgie/Univ. Martinstr. 52, Hamburg, Germany; Tonn, Jorg-Christian, PD Dr. med.; u. Poliklinik/Univ. Kliniken, Wurzberg, Germany; Moumdjian, Robert, Dr.; Hospital Notre-Dame, Montreal, Quebec, Canada; Shaffrey, Mark; University of Virginia, Charlottesville, Virginia; Asher, Anthony; Presbyterian Hospital, Cancer Center, Charlotte, North Carolina; Epstein, Mel; Brown University, Providence, Rhode Island; Schmidt-Schackert, Frau.Prof. Dr. med.; Gabriele, Univ.-Klin. Kar-G. Carus, Klinik f. Neurochirurgie, Dresden, Germany; Mendez, Ivar; Victoria General Hospital, Halifax, Nova Scotia, Canada; Bernstein, Mark, The Toronto Hospital, Toronto, Ontario, Canada; Quigley, Mathew, Alleghemy University of Health Sciences, Pittsburgh, Pennsylvania; Payner, Troy; Indianapolis Surgical Group, Indianapolis, Indiana; Kulvik, Martti; Helsinki University Central Hospital, Helsinki, Finland; Seiler, Rolf W.; University Hospital, Bern, Switzerland; Weiss, Martin Harvey; University of Southern California, Department of Neurosurgery, Los Angeles, California; Fick, James R.; Medical College of Georgia, Department of Surgery, Augusta, Georgia; Leblanc, Richand; Montreal Neurological Institute, Montreal, Quebec, Canada; Buchfelder, Michael; Neurochirurgische Klinik mit Poliklinik der Universtat Erlangen-Nurnberg, Erlangen, Germany; Brotchi, Jacques; Hopital Erasme, Neruosergery, Cliniques Universiaites de Bruxelles, Bruxelles, Belgium; Astrup, Jens; Arhus Kommunehospital, Arhus C, Denmark; Henriksson, Roger; University Hospital, Umea, Sweden; Maciunas, Robert J.; Vanderbilt University Medical Center, Nashville, Tennessee; Ram, Zvi; The Chaim Sheba Medical Center; Tel-Hashomer, Israel; Andrews, David; Thomas Jefferson University Hospita, Philadelphia, Pennsylvania; Verlooy, Jan; University Hospital Antwerp; Antwerp, Belgium; Stockhammer, Gunther; Universitatsklinik fur Neurologie, Innsbruck, Austria; Favrot, Marie; Centre Leon Berard, Lyon, France; and Finocchiaro, Gaetano; Unita Neuroncologia Molecolare e Terapia Genica, Istituto Nazionale Neurologico Carlo Besta, Milano Mi Italy; *Prospective, Open-Label, Parallel-Group, Randomized Multicenter Trial Comparing the Efficacy of Surgery, Radiation, and Injection of Murine Cells Producing Herpes Simplex Thymidine Kinase Vector Followed by Intravenous Ganciclovir Against the Efficacy of Surgery and Radiation in the Treatment of Newly Diagnosed, Previously Untreated Glioblastoma.* Sponsor: Genetic Therapy, Inc./Novartis

Sole FDA Review Recommended by NIH/ORDA: 8-22-96

9608-158 (Open) Gene Therapy/Phase I-IB/Cancer/Melanoma or Sarcoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Plasmid DNA/Particle Mediated Gene Transfer (Accell®)/Cytokine/GM-CSF cDNA/Subcutaneous Injection

Mahvi, David M.; University of Wisconsin Hospital and Clinics Comprehensive Cancer Center, Madison, Wisconsin; *Phase I/IB Study of Immunization with Autologous Tumor Cells Transfected with the GM-CSF Gene by Particle-Mediated Transfer in Patients with Melanoma or Sarcoma.*

Sole FDA Review Recommended by NIH/ORDA: 8-26-96

9605-159 (Closed) Gene Marking/Cancer/Pediatric Malignancies/In Vitro/CD34+ Autologous Bone Marrow and Peripheral Blood/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplant

Heslop, Helen E.; Brenner, Malcolm K.; Krance, Robert A.; Baylor College of Medicine, Houston, Texas; *A Comparative Evaluation of the Utility of Hemopoietic Progenitor Cells Derived from Peripheral Blood vs Bone Marrow.*

Sole FDA Review Recommended by NIH/ORDA: 5-15-96
Closed, no participants entered into this study: 1-11-05

9609-160 (Closed) Gene Therapy/Phase I/Cancer/Prostate Adenocarcinoma/Immunotherapy/In Vivo/Vaccination/Vaccinia Virus/Prostate Specific Antigen/Intradermal Injection

Kufe, Donald W.; and Eder, Joseph Paul; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Trial Of Recombinant Vaccinia Virus That Expresses PSA In Patients With Adenocarcinoma Of The Prostate.*

Sole FDA Review Recommended by NIH/ORDA: 9-18-96
Closed to subject entry: 1-31-00

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9609-161 (Closed) Gene Therapy/Phase I/Cancer/Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/Lipofectin(Gibco BRL)/B7-1(CD80) cDNA/Subcutaneous Injection

Antonia, Scott J.; H. Lee Moffitt Cancer Center, Tampa, Florida; *Treatment of Small Cell Lung Cancer Patients In Partial Remission Or At Relapse With B7-1 Gene-Modified Autologous Tumor Cells As A Vaccine With Systemic Interferon Gamma.*

Sole FDA Review Recommended by NIH/ORDA: 10-10-96
Closed: 1-23-98. Protocol Never Initiated

9610-162 (Closed) Gene Therapy/Phase I/Cancer/Solid Tumors/Oncogene Regulation/HER-2/neu/ In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intratumoral Injection

LaFollette, Suzanne; Rush/Presbyterian/St. Luke's Medical Center, Chicago, Illinois; Murray, James L.; M.D. Anderson Cancer Center, Houston, Texas; Yoo, George; Wayne State University, Detroit, Michigan; *A Phase I Multicenter Study of Intratumoral E1A Gene Therapy for Patients with Unresectable or Metastatic Solid Tumors that Overexpress HER-2/neu.* Sponsor: Targeted Genetics Corporation

Sole FDA Review Recommended by NIH/ORDA: 10-29-96
No longer active: 11-10-03

9610-163 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/MART-1 Melanoma Antigen/Intramuscular Injection

Rosenberg, Steven A.; NIH, Bethesda, Maryland; *Phase I Trial In Patients With Metastatic Melanoma Of Immunization With A Recombinant Fowlpox Virus Encoding The MART-1 Melanoma Antigen.*

Sole FDA Review Recommended by NIH/ORDA: 5-23-96

9610-164 (Closed) Gene Therapy/Phase I/Cancer/Liver(Hepatic)Metastases/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection

Sung, Max W.; and Woo, Savio L.C.; Mount Sinai Medical Center, New York, New York; *Phase I Trial of Adenoviral Vector Delivery of the Herpes Simplex Thymidine Kinase Gene by Intratumoral Injection Followed by Intravenous Ganciclovir in Patients with Hepatic Metastases.*

Sole FDA Review Recommended by NIH/ORDA: 11-12-96

9611-165 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/gp100 Melanoma Antigen/Intramuscular Injection

Rosenberg, Steven A.; NIH, Bethesda, Maryland; *Phase I Trial In Patients With Metastatic Melanoma Of Immunization With A Recombinant Fowlpox Virus Encoding the GP100 Melanoma Antigen.*

NIH/ORDA Receipt Date: 11-13-96. Sole FDA Review Recommended: 1-17-96

9611-166 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Vaccinia Virus/MART-1 Melanoma Antigen/Intramuscular Injection

Rosenberg, Steven A.; NIH, Bethesda, Maryland; *Phase I Trial In Patients With Metastatic Melanoma Of Immunization With A Recombinant Vaccinia Virus Encoding the MART-1 Melanoma Antigen.*

NIH/ORDA Receipt Date: 11-13-96. Sole FDA Review Recommended: 1-17-96

9611-167 (Closed) Gene Therapy/Phase II/Cancer/Glioblastoma/Pro-Drug/In Vivo/Autologous Tumor Cells/PA317/Retrovirus/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Direct Injection

Maria, Bernard, *et al.* (All #9608-157 sites are eligible to participate in this study.) *Prospective, Open-Label, Multicenter, Extension Trial for the Treatment of Recurrent Glioblastoma Multiforme with Surgery and Injection of Murine Cells Producing Herpes Simplex Thymidine Kinase Vector Followed by Intravenous Ganciclovir for Patients with Disease Progression Following Standard Treatment on Protocol GTI-0115.* Sponsor: Genetic Therapy, Inc./Novartis
This protocol is an extension of #9608-157.

NIH/ORDA Receipt Date: 11-13-96. Sole FDA Review Recommended by NIH/ORDA: 1-6-97

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9611-168 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL 1005/HLA-B7/Beta-2 Microglobulin cDNA/Direct Intratumoral Injection

Hersh, Evan M.; Arizona Cancer Center, Tucson, Arizona; Klasa, Richard; British Columbia Cancer Agency, Vancouver, B.C., Canada; Gonzales, Rene; University of Colorado Cancer Center, Denver, Colorado; Silver, Gary; Northern California Melanoma Clinic, San Francisco, California; Thompson, John A.; U. of Washington Medical Center, Seattle, Washington; *Phase II Study of Immunotherapy of Metastatic Melanoma by Direct Gene Transfer.* Sponsor: Vical, Incorporated

NIH/ORDA Receipt Date: 11-26-96. Sole FDA Review Recommended by NIH/ORDA: 1-6-97

9611-169 (Closed) Gene Therapy/Phase I/II/Cancer/Solid Tumors/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL 1102/Cytokine/Interleukin-2 cDNA/Direct Intratumoral Injection

Hersh, Evan, M.; Arizona Cancer Center, Tucson, Arizona; Rinehart, John; Scott and White Clinic, Temple, Texas; Rubin, Joseph; Mayo Clinic, Rochester, Minnesota; Sondak, Vernon K.; University of Michigan Medical Center, Ann Arbor, Michigan; Gonzales, Rene; University of Colorado Cancer Center, Denver, Colorado; Sobol, Robert E.; Sharp HealthCare, San Diego, California; and Forscher, Charles A.; Cedars-Sinai Comprehensive Cancer Center, Los Angeles, California; *Phase I/II Trial of Interleukin-2 DNA/DMRIE/DOPE Lipid Complex as an Immunotherapeutic Agent in Cancer by Direct Gene Transfer.* Sponsor: Vical, Incorporated

NIH/ORDA Receipt Date: 11-26-96. Sole FDA Review Recommended by NIH/ORDA: 1-17-97

9612-170 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Lung and Nasal Epithelial Cells/Cationic Liposome Complex/DOPE/CFTR cDNA/Aerosol Administration

Sorscher, Eric; University of Alabama, Birmingham, Medical Center; *Safety and Efficiency of Gene Transfer of Aerosol Administration of a Single Dose of a Cationic Lipid/DNA Formulation to the Lungs and Nose of Patients with Cystic Fibrosis.* Sponsor: Genzyme Corporation

NIH/ORDA Receipt Date: 12-17-96**.** Sole FDA Review Recommended by NIH/ORDA: 1-6-97
Completed: 12-97

9701-171 (Open) Non-Therapeutic/In Vivo/Intradermal Cells/Adenovirus/Serotype 5/E. coli Cytosine Deaminase/Intradermal Injection

Harvey, Ben-Gary; and Crystal, Ronald G.; Rockefeller University Hospital, New York, New York; *Immune Response to Intradermal Administration of an Adenovirus Type 5 Gene Transfer Vector (Ad_{GV}CD.10) in Normal Individuals.*

NIH/ORDA Receipt Date: 1-9-97. *RAC Recommends Approval: 3-6-97/NIH Approval: 4-21-97

9701-172 (Closed) Gene Therapy/Phase I/Cancer(Testicular Cancer)/Chemoprotection/In Vitro/G-CSF Mobilized Autologous CD34+ Peripheral Blood Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Bone Marrow Transplant

Cornetta, Kenneth; and Abonour, Rafat; Indiana University Department of Medicine, Indianapolis, Indiana; *High Dose Carboplatin and Etoposide Followed by Transplantation with Peripheral Blood Stem Cells Transduced with the Multiple Drug Resistance Gene in the Treatment of Germ Cell Tumors - A Pilot Study.*

NIH/ORDA Receipt Date: 1-9-97**.** Sole FDA Review Recommended by NIH/ORDA: 2-26-97
Closed to patient accrual: 2-15-00

9701-173 (Closed) Gene Therapy/Phase I/Cancer/Brain Tumors/Chemoprotection/In Vitro/Peripheral Blood CD34+ Cells/Retrovirus/O^6-Methylguanine DNA Methyltransferase cDNA/Intravenous Infusion

Croop, James; Indiana University School of Medicine, Indianapolis, Indiana; and Kieran, Mark, Dana-Farber Cancer Institute, Boston, Massachusetts; *A Pilot Study of Dose Intensified Procarbazine, CCNU, Vincristine(PCV) for Poor Prognosis Pediatric and Adult Brain Tumors Utilizing Fibronectin-Assisted, Retroviral-Mediated Modification of CD34+ Peripheral Blood Cells with O^6-Methylguanine DNA Methyltransferase.*

NIH/ORDA Receipt Date: 1-13-97. Sole FDA Review Recommended by NIH/ORDA: 2-4-97
Notification that trial is closed to new research participant enrollment: 2-20-01

9701-174 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Interleukin-2 cDNA/Neomycin Phosphotransferase cDNA/Immunoisolation Device/Subcutaneous Implantation

Das Gupta, Tapas K.; University of Illinois at Chicago, Chicago, Illinois; *A Pilot Study Using Interleukin-2 Transfected Irradiated Allogeneic Melanoma Cells Encapsulated in an Immunoisolation Device In Patients with Metastatic Malignant Melanoma.*

NIH/ORDA Receipt Date: 1-13-97. Sole FDA Review Recommended by NIH/ORDA: 2-21-97

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9701-175 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral/Stereotactic Injection

Lieberman, Frank; Germano, Isabelle; and Woo, Savio; Mount Sinai Medical Center, New York, New York; *Gene Therapy for Recurrent Glioblastoma Multiforme: Phase I Trial of Intraparenchymal Adenoviral Vector Delivery of the HSV-TK Gene and Intravenous Administration of Ganciclovir*.

NIH/ORDA Receipt Date: 1-22-97. Sole FDA Review Recommended by NIH/ORDA: 2-12-97

9702-176 (Open) Gene Therapy/Phase I/II/Cancer/Prostate Adenocarcinoma/Immunotherapy/In Vivo/Vaccination/Vaccinia Virus/Prostate Specific Antigen/Intradermal Injection

Sanda, Martin G.; University of Michigan Urology Clinics, Ann Arbor, Michigan; *A Phase I/II Clinical Trial Evaluating the Safety and Biological Activity of Recombinant Vaccinia-PSA Vaccine in Patients with Serological Recurrence of Prostate Cancer Following Radical Prostatectomy.*

NIH/ORDA Receipt Date: 2-19-97. Sole FDA Review Recommended by NIH/ORDA: 5-13-97

9702-177 (Open) Gene Marking/Cancer/Chronic Myelogenous Leukemia/In Vitro/Autologous Peripheral Blood Cells Mobilized by Cyclophosphamide and G-CSF/Retrovirus/Neomycin Phosphotransferase cDNA/Autologous Bone Marrow Transplant

Verfaillie, Catherine; McIvor, Scott; McCullough, Jeff; and McGlave, Philip; University of Minnesota, Minneapolis, Minnesota; *Autologous Marrow Transplantation for Chronic Myelogenous Leukemia Using Retrovirally Marked Peripheral Blood Progenitor Cells Obtained after In Vivo Cyclophosphamide/G-CSF Priming.*

NIH/ORDA Receipt Date: 2-21-97. Sole FDA Review Recommended by NIH/ORDA: 3-14-97

9703-178 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD34+ Autologous Cord Blood Cells/Retrovirus/Transdominant Trev/Intravenous

Belmont, John W.; Texas Children's Hospital, Houston, Texas; *Phase I Clinical Trial of TREV Gene Therapy for Pediatric AIDS.*

NIH/ORDA Receipt Date: 3-10-97. Sole FDA Review Recommended by NIH/ORDA: 3-31-97

9703-179 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Carcinoembryonic Antigen/Intravenous

Lyerly, Kim H.; Duke University Medical Center, Durham, North Carolina; *A Phase I Study of Active Immunotherapy With Carcinoembryonic Antigen RNA-Pulsed Autologous Human Cultured Dendritic Cells In Patients with Metastatic Malignancies Expressing Carcinoembryonic Antigen.*

NIH/ORDA Receipt Date: 3-14-97. Publicly Reviewed at the June 1997 RAC meeting.

Sole FDA Review Recommended by NIH/ORDA: 6-24-97

9703-180 (Closed) Gene Therapy/Phase I/Other/Cubital Tunnel Syndrome/In Vivo/Autologous Muscle Cells/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Human Insulin-Like Growth Factor-1(hIGF-1)/Intramuscular Injection

Netscher, David; Hand Clinic at the Veteran's Affairs (VA) Medical Center, Houston, Texas; *Phase I Single Dose-Ranging Study Of Formulated hIGF-1 Plasmid In Subjects With Cubital Tunnel Syndrome.* Sponsor: Gene Medicine, Inc.

NIH/ORDA Receipt Date: 3-17-97. Sole FDA Review Recommended: 4-7-97
Closed: 8-14-02

9703-181 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD8 + and CD4+ T Lymphocytes/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous/Concurrent Interleukin-2 Therapy

Connick, Elizabeth; University of Colorado Health Sciences Center, Denver, Colorado; Deeks, Steven G.; University of California, San Francisco General Hospital, San Francisco, California; Scadden, David; Massachusetts General Hospital (East), Charlestown, Massachusetts; Mitsuyasu, Ronald; University of California, Los Angeles Medical Center, Los Angeles, California; *A Phase II Study of the Activity and Safety of Autologous CD4-Zeta Gene-Modified T Cells With or Without Exogenous Interleukin-2 in HIV Infected Patients.* Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 3-19-97. Sole FDA Review Recommended: 4-18-97
Notification from sponsor that trial is closed: 4-09-01

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9703-182 (Closed) Gene Therapy/Phase II/Monogenic Inherited Disorder/Cystic Fibrosis/Sinusitis/Correction/In Vivo/Maxillary Sinus Epithelial Cells/ Adeno-associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Maxillary Sinus Administration

Gardner, Phyllis; Stanford University's General Clinical Research Center (GCRC), Palo Alto, California; *A Phase I/II Study of tgAAVCF for the Treatment of Chronic Sinusitis With Cystic Fibrosis.* Sponsor: Targeted Genetics Corporation

NIH/ORDA Receipt Date: 3-13-97. Sole FDA Review Recommended: 4-1-97
No longer active: 6-15-07

9703-183 (Closed) Gene Marking/Cancer/EBV-Positive Hodgkin Disease/In Vitro/EBV-Specific Hodgkin Disease/In Vitro/EBV-Specific Cytotoxic Lymphocytes/Retrovirus/Neomycin Phosphotransferase/Bone Marrow Transplant

Straus, Stephan E.; National Institutes of Health, Bethesda, Maryland; *Administration of Neomycin Resistance Gene Marked EBV Specific Cytotoxic T-Lymphocytes To Patients With Relapsed EBV-Positive Hodgkin Disease.*

Compassionate Case

NIH/ORDA Receipt Date: 3-19-97. Sole FDA Review Recommended by NIH/ORDA: 3-25-97
Patient never treated (closed as of 11-18-97)

9703-184 (Closed) Gene Therapy/Phase I/Cancer/Prostate Cancer/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1102/Cytokine/Interleukin-2 cDNA/Intratumoral Injection

Belldegrun, Arie; University of California, Los Angeles, School of Medicine, Los Angeles, California; *A Phase I Study Evaluating the Safety and Efficacy of Interleukin-2 Gene Therapy Delivered by Lipid Mediated Gene Transfer (Leuvectin) in Prostate Cancer Patients.* Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 3-24-97. Sole FDA Review Recommended by NIH/ORDA: 5-21-97

9704-185 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Melanoma Cell/Canarypox Virus/Cytokine/Interleukin-12 cDNA/Intratumoral Injection

Conry, Robert M.; University of Alabama at Birmingham, Birmingham, Alabama; *Phase Ib Trial of Intratumoral Injection of a Recombinant Canarypox Virus Encoding the Human Interleukin-12 Gene (ALVAC-hIL-12) in Patients with Surgically Incurable Melanoma* Sponsor: NCI- Cancer Therapy Evaluation Program

NIH/ORDA Receipt Date: 4-1-97. Sole FDA Review Recommended by NIH/ORDA: 7-2-97
Completed: 11-98

9704-186 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Cationic Liposome Complex/EDMPC/Intranasal Administration

Noone, Peadar G.; Knowles, Michael R.; University of North Carolina at Chapel Hill, North Carolina; *A Double-Blind, Placebo Controlled, Dose Ranging Study to Evaluate the Safety and Biological Efficacy of the Lipid-DNA Complex GR213487B in the Nasal Epithelium of Adult Patients with Cystic Fibrosis.* Sponsor: Glaxo Wellcome Inc.

NIH/ORDA Receipt Date: 4-23-97. Sole FDA Review Recommended by NIH/ORDA: 5-13-97

9705-187 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Pro-Drug/In Vivo/AutologousTumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection

Hall, Simon J.; Woo, Savio L.C.; Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenoviral-Mediated Herpes Simplex Thymidine Kinase Gene Transduction in Conjunction with Ganciclovir Therapy as Neo-adjuvant Treatment for Patients with Clinically Localized (Stage T1c and T2b&c) Prostate Cancer Prior to Radical Prostatectomy.*

NIH/ORDA Receipt Date: 5-7-97. Sole FDA Review Recommended by NIH/ORDA: 5-28-97
Closed to accrual: 11-12-01

9705-188 (Open) Gene Therapy/Phase I/Cancer/Chronic Myelogenous Leukemia/Chemoprotection/Tyr-22 Murine Dihydrofolate Reductase Gene/Antisense/Anti-b3a2BCR/ABL Gene/In Vitro/Autologous Peripheral Blood CD34+ Cells Mobilized by Cyclophosphamide and G-CSF/Retrovirus/Autologous Bone Marrow Transplant

Verfaillie, Catherine; McIvor, Scott; McCullough, Jeff; McGlave, Philip; University of Minnesota, Minneapolis, Minnesota; *Autologous Transplantation for Chronic Myelogenous Leukemia with Stem Cells Transduced with a Methotrexate Resistant DHFR and Anti-BCR/ABL Containing Vector and Post Transplant Methotrexate Administration.*

NIH/ORDA Receipt Date: 5-16-97. Sole FDA Review Recommended by NIH/ORDA: 6-6-97

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9705-189 (Closed) Gene Therapy/Phase I/Cancer/Hepatocellular Carcinoma/Tumor Suppressor Gene/In Vivo/Autologous nTumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection

Belani, Chandra P.; University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *Phase I Study of Percutaneous Injections of Adenovirus p53 Construct (Adeno-p53) for Hepatocellular Carcinoma.*

NIH/ORDA Receipt Date: 5-27-97. Sole FDA Review Recommended by NIH/ORDA: 9-19-97
Closed: 3-7-00

9705-190 (Open) Gene Therapy/Phase I/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DOTMA-Cholesterol/Cytokine/Interleukin-2 cDNA/Intratumoral Injection

O'Malley, Bert W.; Johns Hopkins Medical Institutions, Baltimore, Maryland; *A Double-Blind, Placebo-Controlled, Single Rising-Dose Study of the Safety and Tolerability of Formulated hIL-2 Plasmid in Patients with Squamous Cell Carcinoma of the Head and Neck (SCCHN*). Sponsor: Gene Medicine, Inc.

NIH/ORDA Receipt Date: 5-27-97. Sole FDA Review Recommended by NIH/ORDA: 6-16-97

9706-191 (Closed) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Direct Intratumoral Injection

Gluckman, Jack L..; Gleich, Lyon L., University of Cincinnati Medical Center, Cincinnati, Ohio; Swinehart, James M.; Colorado Medical Research Center, Denver, Colorado; Hanna, Ehab; University of Arkansas for Medical Sciences/Arkansas Cancer Research Center (UAMS), Little Rock, Arkansas; Castro, Dan J.; University of California, Los Angeles, Los Angeles, California; Gapany, Markus; Veterans Affairs Medical Center, Minneapolis, Minnesota; Carroll, William R.; University of Alabama at Birmingham, Birmingham, Alabama; Coltrera, Marc D.; University of Washington Medical Center, Seattle, Washington; Wolf, Gregory T.; University of Michigan Medical Center, Ann Arbor, Michigan; and Okuno, Scott; Mayo Clinic, Rochester, Minnesota; *Phase II Study of Immunotherapy by Direct Gene Transfer with Allovectin-7 for the Treatment of Recurrent or Metastatic Squamous Cell Carcinoma of the Head and Neck*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 6-6-97. Sole FDA Review Recommended by NIH/ORDA: 7-7-97

9706-192 (Open) Gene Therapy/Phase I/Cancer/Prostate/Tumor suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection

Belldegrun, Arie; and Figlin, Robert.; UCLA School of Medicine, Los Angeles, California; *A Phase I Study in Patients with Locally Advanced or Recurrent Adenocarcinoma of the Prostate Using SCH58500 (rAd/p53) Administered by Intratumoral Injection.* Sponsor: Schering-Plough Corporation

NIH/ORDA Receipt Date: 6-9-97. Sole FDA Review Recommended by NIH/ORDA: 9-17-97

9706-193 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/CEA-Expressing Malignancies/In Vivo/Autologous Muscle Cells/Canarypox Virus/Vaccinia Virus/Carcinoembryonic Antigen cDNA/Intramuscular and Percutaneous Injection

Marshall, John L.; Vincent T. Lombardi Cancer Research Center, Georgetown University Medical Center, Washington, D.C.; *A Pilot Study of Sequential Vaccinations with ALVAC-CEA and Vaccinia-CEA with the Addition of IL-2 and GM-CSF in Patients with CEA Expressing Tumors.* Sponsor: National Cancer Institute-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 6-18-97. Sole FDA Review Recommended by NIH/ORDA: 9-18-97

9706-194 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Muscle Cells/Retrovirus/HIV-1 IIIB Envelope Protein/Intramuscular Injection

Aboulafia, David; Virginia Mason Clinic, Seattle, Washington; Campbell, Thomas; University of Colorado Health Sciences Center, Denver, Colorado; Kumar, Princy; Georgetown University Medical Center, Washington, D.C.; Murphy, Robert; Northwestern University Medical School, Chicago, Illinois; Skolnik, Paul; New England Medical Center, Boston, Massachusetts; and Wheat, Joseph; Indiana University Hospital, Indianapolis, Indiana; *A Phase II, Randomized, Double Blind Placebo Controlled Study of Combination Drug Anti-Retroviral Therapy to Include a Reverse Transcriptase Inhibitor and a Protease Inhibitor Plus HIV-IT(V) or Placebo in HIV Patients with CD4+ Counts $\geq$ 100, and HIV RNA $\geq$ 1K, and $\leq$ 10K*. Sponsor: Chiron Corporation

NIH/ORDA Receipt Date: 6-23-97. Sole FDA Review Recommended by NIH/ORDA: 8-15-97
5-10-00: IND no longer active

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9706-195 (Closed) Gene Therapy/Phase I/Cancer/Immunotherapy/CEA-Expressing Malignancies/In Vivo/Vaccinia Virus/Carcinoembryonic Antigen cDNA/Intradermal and Subcutaneous Injections

Conry, Robert M.; The University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Trial of a Recombinant Vaccinia-CEA (180 Kd) Vaccine Delivered by Intradermal Needle Injection Versus Subcutaneous Jet Injection in Patients with Metastatic CEA-Expressing Adenocarcinoma*. Sponsor: Drug Regulatory Affairs Branch, Cancer Therapy Evaluation Program (CTEP), Division of Cancer Treatment, Diagnosis and Centers, NCI, NIH

NIH/ORDA Receipt Date: 6-26-97. Sole FDA Review Recommended by NIH/ORDA: 9-5-97
Completed: 7-98

9706-196 (Closed) Gene Therapy/Phase I/Monogenic Disease/Chronic Granulomatous Disease/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/gp91phox/Intravenous Infusion

Croop, James; Indiana University School of Medicine, Indianapolis, Indiana; *Fibronectin-Assisted, Retroviral-Mediated Transduction of CD34+ Peripheral Blood Cells with gp91 phox in Patients with X-Linked Chronic Granulomatous Disease: A Phase I Study.*

NIH/ORDA Receipt Date: 6-30-97. Sole FDA Review Recommended by NIH/ORDA: 7-21-97
Closed to enrollment: 4-06-04

9706-197 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Melanoma Cell/Canarypox Virus/B7(CD80)/Interleukin-12/Cytokine/Intratumoral Injection

Conry, Robert M.; University of Alabama at Birmingham, Birmingham, Alabama; *Phase Ib Trial of Intratumoral Injection of a Recombinant Canarypox Virus Encoding Human B7.1 (ALVAC-hB7.1) or a Combination of ALVAC-hB7.1 and a Recombinant Canarypox Virus Encoding Human Interleukin-12 (ALVAC-hIL-12) in Patients with Surgically Incurable Melanoma*. Sponsor: National Cancer Institute-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 6-30-97. Sole FDA Review Recommended by NIH/ORDA: 9-5-97

9707-198 (Closed) Gene Therapy/Phase I/II/Cancer/Colorectal Carcinoma Expressing TAG-72/In Vitro/Autologous CD8+ and CD4+ T Lymphocytes/Retrovirus/CC49-Zeta T Cell Receptor/Intravenous Infusion

Venook, Alan and Warren, Robert S.; University of California, San Francisco, California and Fisher, George; Stanford University, Palo Alto, California; *A Phase I/II Study of Autologous CC49-Zeta Gene-Modified T Cells and α-Interferon in Patients with Advanced Colorectal Carcinomas Expressing the Tumor-Associated Antigen, TAG-72.* Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 7-7-97. Sole FDA Review Recommended by NIH/ORDA: 8-28-97
Notification from sponsor that trial is closed: 4-09-01

9707-199 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Breast/Head and Neck Cancer/Cutaneous T-Cell Lymphoma/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-12/Intratumoral Injection

Park, Chan H.; Samsung Medical Center, Seoul, Korea; Kim, Sunyoung; Seoul National University, Seoul, Korea; Lotze, Michael; Tahara, Hideaki; and Robbins, Paul; University of Pittsburgh, Pittsburgh, Pennsylvania; *IL-12 Gene Therapy Using Direct Injection of Tumors with Genetically Engineered Autologous Fibroblasts.*

NIH/ORDA Receipt Date: 7-22-97. Sole FDA Review Recommended by NIH/ORDA: 10-30-97

9707-200 (Open) Gene Therapy/Phase I/II/Cancer/Non-Hodgkin's B-Cell Lymphoma/Mantle Cell Lymphoma/Immunotherapy/In Vivo/Naked Plasmid DNA/Tumor Idiotype/Intramuscular Injection

Levy, Ronald; Stanford University School of Medicine, Stanford, California; *A Phase I/II Study of Vaccine Therapy for B-Cell Lymphoma Utilizing Plasmid DNA Coding for Tumor Idiotype*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 7-24-97. Sole FDA Review Recommended by NIH/ORDA: 8-13-97

9707-201 (Open) Gene Therapy/Phase I/ Cancer/Ovarian/Immunotherapy/In Vitro/Autologous Tumor Cells/Canarypox Virus/B7.1 (CD80)/Intraperitoneal Injection

Freedman, Ralph; The University of Texas, M.D. Anderson Cancer Center, Houston, Texas; *Intraperitoneal (IP) Autologous Therapeutic Tumor Vaccine (AUT-OV-ALVAC-hB7.1) plus IP rIFN-γ for Patients with Ovarian Cancer. A Pilot Study*. Sponsor: NCI Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 7-28-97. Sole FDA Review Recommended by NIH/ORDA: 8-15-97

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9707-202 (Closed) Gene Therapy/Phase I/Immunotherapy/Cancer/Melanoma/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Subcutaneous Injection

Dranoff, Glenn and Soiffer, Robert; Dana-Farber Cancer Institute, Harvard Medical School, Boston, Massachusetts; *A Phase I Study of Vaccination with Autologous, Lethally Irradiated Melanoma Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor*.

NIH/ORDA Receipt Date: 7-28-97. Sole FDA Review Recommended by NIH/ORDA: 8-15-97
Enrollment complete: 12-27-01

9707-203 (Closed) Gene Therapy/Phase I/Immunotherapy/Cancer/Non-Small Cell Lung Carcinoma (NSCLC)/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Subcutaneous Injection

Dranoff, Glenn and Salgia, Ravi; Dana-Farber Cancer Institute, Harvard Medical School, Boston, Massachusetts; *A Phase I Study of Vaccination with Autologous, Lethally Irradiated Non-Small Cell Lung Carcinoma Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor*.

NIH/ORDA Receipt Date: 7-28-97. Sole FDA Review Recommended by NIH/ORDA: 8-15-97
Enrollment complete: 12-27-01

9707-204 (Closed) Gene Therapy/Phase I/Monogenic Disease/Leukocyte Adherence Deficiency (LAD)/In Vitro/G-CSF Mobilized CD34+ Autologous Peripheral Blood Cells/Retrovirus/CD18/Intravenous Infusion

Hickstein, Dennis and Bauer, Thomas R. National Institutes of Health, Bethesda, Maryland; *Retrovirus-Mediated Transfer of the cDNA for Human CD18 into Peripheral Blood Repopulating Cells of Patients with Leukocyte Adherence Deficiency*.

NIH/ORDA Receipt Date: 7-31-97. Sole FDA Review Recommended by NIH/ORDA: 9-17-97
Closed: 9-17-00

9708-205 (Closed) Gene Therapy/Phase I/II/Cancer/Prostate/Immunotherapy/ In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection

Simons, Jonathan W.; Johns Hopkins Oncology Center, Baltimore, Maryland; *Phase I/II Study of Allogeneic Human GM-CSF Gene Transduced Irradiated Prostate Cancer Cell Vaccines in Patients with Prostate Cancer*.

NIH/ORDA Receipt Date: 8-19-97. Sole FDA Review Recommended by NIH/ORDA: 9-9-97
Closed: 7-23-01

9708-206 (Closed) Gene Therapy/Phase I/II/Cancer/Chronic Myelogenous Leukemia/Adoptive Immunotherapy/In Vitro/Donor CD8+ and CD4+ Lymphocytes/Retrovirus/Hygromycin Phosphotransferase-Herpes Simplex Thymidine Kinase Fusion Gene/Intravenous Infusion

Flowers, Mary E. D. and Riddell, Stanley; Fred Hutchinson Cancer Research Center, Seattle, Washington; *Infusion of Polyclonal HyTK (hygromycin phosphotransferase and HSV thymidine kinase gene)-transduced Donor T Cells for Adoptive Immunotherapy in Patients with Relapsed CML after Allogeneic Stem Cell Transplant: Phase I-II Clinical Trial*.

NIH/ORDA Receipt Date: 8-19-97. Sole FDA Review Recommended by NIH/ORDA: 9-26-97
Closed to new accrual: 4-24-00.

9708-207 (Closed) Gene Therapy/Phase I/Cancer/Colorectal/Immunotherapy/In Vivo/Autologous Tumor Cells/Canarypox Virus/Carcinoembryonic Antigen/B7.1 (CD80)/Intradermal Scarification

Kaufman, Howard L.; Albert Einstein Cancer Center, Bronx, New York; *Phase I Clinical Trial of a Recombinant ALVAC-CEA-B7 Vaccine in the Treatment of Advanced Colorectal Carcinoma.* Sponsor: National Cancer Institute-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 8-21-97. Sole FDA Review Recommended by NIH/ORDA: 11-25-97
Closed: 2-99.

9708-208 (Open) Gene Therapy/Phase I/Cancer/Mesothelioma/Pro-Drug/In Vivo/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Herpes Simplex Virus Thymidine Kinase/Ganciclovir/Intrapleural Administration

Schwarzenberger, Paul; Louisiana State University Medical Center, New Orleans, Louisiana; *The Treatment of Malignant Pleural Mesothelioma with a Gene-Modified Cancer Vaccine: A Phase I Study.*

NIH/ORDA Receipt Date: 8-25-97. Sole FDA Review Recommended by NIH/ORDA: 9-16-97

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9708-209 (Closed) Non-Therapeutic/In Vivo/Bronchial Epithelial Cells/Adenovirus/Serotype 5/*E. coli* Cytosine Deaminase/Intrabronchial Administration

Harvey, Ben-Gary and Crystal, Ronald G.; Rockefeller University Hospital, New York, New York; *Systemic and Respiratory Immune Response to Administration of an Adenovirus Type 5 Gene Transfer Vector ($Ad_{GV}CD.10$)*.

NIH/ORDA Receipt Date: 8-26-97. Publicly Reviewed at the December 16, 1997 RAC meeting
Closed: 09-19-00

9709-210 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1005/HLA-B7/β2-Macroglobulin cDNA/Direct Intratumoral Injection

Gonzales, Rene; University of Colorado Cancer Center, Denver, Colorado; Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; Deisseroth, Albert, Yale University, New Haven, Connecticut; Paciucci, Paolo A., Mt. Sinai Medical Center, New York, New York; Hutchins, Laura F., University of Arkansas for Medical Sciences, Little Rock, Arkansas; Galanis, Evanthia, Mayo Clinic, Rochester, Minnesota; Schaefer, Paul L., Toledo Clinic, Toledo, Ohio; Amatruda, Thomas, Virginia Piper Cancer Institute Abbott Northwestern Hospital, Minneapolis, Minnesota; Kuzel, Timothy, Northwestern Medical Faculty Foundation Northwestern Memorial Hospital, Chicago, Illinois; Blum, Ronald H., Beth Israel Medical Center, Phillips Ambulatory Care Center, New York, New York; Whitman, Eric D., The Melanoma Center of St. Louis, Saint Louis, Missouri; Cobb, Patrick, Billings Interhospital Oncology Project, Billings, Montana; Amin, Bipinkumar, Mid Dakota Clinic, Bismarck, North Dakota; Chowhan, Naveed, Cancer Care Center Incorporated, New Albany, Indiana; Lutzky, Jose, Mount Sinai Medical Center, Miami, Florida; Amatruda, Thomas, North Memorial Healthcare, Hubert H. Humphrey Cancer Center, Robbinsdale, Minnesota ;Patel, Ravi, Comprehensive Blood and Cancer Center, Bakersfield, California; Dobbs, Tracy W., Baptist Hospital of East Tennessee, Knoxville, Tennessee; Ahmed, Fakhiuddin, HemOnCare, P.C., Brooklyn, New York; Thant, Myo, Maryland Hematology/Oncology Associates, Baltimore, Maryland; Stark, James J., Maryview Medical Center, Portsmouth, Virginia; Arena, Francis, Arena Oncology Associates, Great Neck, New York; Soori, Gamini, Alegent Health, Bergan Mercy Medical Center, Omaha, Nebraska; Samlowski, Wolfram, University of Utah Health Sciences Center; Huntsman Cancer Institute, Salt Lake City, Utah; Polikoff, Jonathan A., Kaiser Permanente Medical Group, San Diego, California; Hawkins, Michael, Washington Hospital Center, Washington Cancer Institute, Washington, D.C.; Richart, John, Saint Louis University Health Sciences Center, St. Louis, Missouri; Patel, Taral, Columbus, Community Clinical Oncology Program, Columbus, Ohio; Levine, Edward, Wake Forest University School of Medicine, Winston Salem, North Carolina; Richards, Jon, Lutheran General Hospital, Park Ridge, Illinois; Thompson, John A., University of Washington Medical Center, Seattle, Washington; and Schwarzenberger, Paul, Louisiana State University Health Sciences Center, New Orleans, Louisiana; *Compassionate Use Protocol for Retreatment with Allovectin-7 Immunotherapy for Metastatic Cancer by Direct Gene Transfer*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 9-8-97. Sole FDA Review Recommended by NIH/ORDA: 9-26-97

9708-211 (Closed) Gene Therapy/Phase I/Monogenetic Disease/Canavan Disease/In Vivo/Autologous Brain Cells/Plasmid DNA/Adeno-associated Virus/Poly-L-Lysine/Cationic Liposome Complex/DC-Chol/DOPE/Aspartoacylase cDNA/Intracranial (Ommaya Reservoir) Administration

Seashore, Margretta R.; Yale University, New Haven, Connecticut; *Gene Therapy of Canavan Disease: Retreatment of Previously Treated Children*.

NIH/ORDA Receipt Date: 8-28-97. Publicly Reviewed at the December 16, 1997 RAC meeting
Closed: 5-22-00

9709-212 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE Vical VCL-1005/HLA-B7/Beta-2 Microglobulin cDNA/Vical-1102/Interleukin-2 cDNA/Intratumoral Injection

Gonzalez, Rene; University of Colorado Health Sciences Center, Denver, Colorado; Hersh, Evan M.; Arizona Cancer Center, Tucson, Arizona; Rubin, Joseph; Mayo Clinic, Rochester, Minnesota; and Thompson, John A.; University of Washington Medical Center, Seattle, Washington; *Phase I Study of Direct Gene Transfer of HLA-B7 Plasmid DNA/DMRIE/DOPE Lipid Complex (Allovectin-7) with IL-2 Plasmid DNA/DMRIE/DOPE Lipid Complex (Leuvectin) as an Immunotherapeutic Regimen in Patients with Metastatic Melanoma.* Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 9-18-97. Sole FDA Review Recommended by NIH/ORDA: 10-8-97

9709-213 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD8+ T Cells/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous

Deeks, Steven G.; University of California, San Francisco General Hospital, San Francisco, California; *A Phase II Study of Autologous CD4-Zeta Gene-Modified T Cells in HIV-Infected Patients with Undetectable Plasma Viremia on Combination Antiretroviral Drug Therapy*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 9-22-97. Sole FDA Review Recommended by NIH/ORDA: 10-10-97
Study closed to new accrual, follow-up is continuing: 7-13-01

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9709-214 (Open) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53

Breau, Randall L.; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Clayman, Gary L.; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; Yoo, George H.; Wayne State University/Barbara Ann Karmanos Cancer Institute, Detroit, Michigan; Medina, Jesus E.; University of Oklahoma Health Sciences Center, Oklahoma City, Oklahoma; Murphy, Barbara S.; Vanderbilt University Medical Center, Nashville, Tennessee; Goodwin, W. Jarrard; University of Miami Hospitals and Clinics, Miami, Florida; Weber, Jeffery S.; University of Southern California, Los Angeles, California; Schuller, David E.; Ohio State University Medical Center, Columbus, Ohio; Bukowski, Ronald M.; The Cleveland Clinic Foundation, Cleveland, Ohio; Hamm, John; University of Louisville Health Sciences Center, Louisville, Kentucky; Agarwala, Sanjiv; University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; Hochster, Howard S.; New York University Medical Center, New York, New York; Dietz, Andreas; University of Heidelberg, Heidelberg, Germany; Eβer, Dirk; Ear, Nose and Throat Clinic, Erfurt, Germany; and Flood, William A.; Milton S. Hershey Medical Center, Hershey, Pennsylvania; *A Phase II Multi-Center, Open Label, Randomized Study to Evaluate Effectiveness and Safety of Two Treatment Regimens of Ad5CMV-p53 Administered by Intra-Tumoral Injections in 78 Patients with Recurrent Squamous Cell Carcinoma of the Head and Neck (SCCHN)*. Sponsor: Aventis (formerly Gencell)

NIH/ORDA Receipt Date: 9-22-97. Sole FDA Review Recommended by NIH/ORDA: 10-21-97

9709-215 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Autologous Tumor Cells/Canarypox Virus/Carcinoembryonic Antigen/B7.1 (CD80)/Intramuscular and Intradermal Injections

von Mehren, Margaret; Fox Chase Cancer Center, Philadelphia, Pennsylvania; *Phase I/Pilot Study of ALVAC-CEA-B7.1 Immunization in Patients with Advanced Adenocarcinoma Expressing CEA*. Sponsor: National Cancer Institute - Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 9-24-97. Sole FDA Review Recommended by NIH/ORDA: 10-28-97

9709-216 (Open) Gene Therapy/Phase I/Cancer/Breast/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Cutaneous or Subcutaneous

von Mehren, Margaret; Fox Chase Cancer Center, Philadelphia, Pennsylvania; *Phase I/Pilot Study of p53 Intralesional Gene Therapy with Chemotherapy in Breast Cancer* . Sponsor: National Cancer Institute - Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 9-24-97. Sole FDA Review Recommended by NIH/ORDA: 10-28-97

9710-217 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection

Logothetis, Christopher J.; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Tolerance and Efficacy Study of Intraprostatic INGN 201 Followed by Pathological Staging and Possible Radical Prostatectomy in Patients with Locally Advanced Prostate Cancer*. Sponsor: Introgen Therapeutics, Inc.

NIH/ORDA Receipt Date: 10-3-97. Sole FDA Review Recommended by NIH/ORDA: 11-6-97

9710-218 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Hammerhead Ribozyme/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Tat and Rev Hammerhead Ribozyme/Intravenous

Krishnan, Amrita and Zaia, John, A.; City of Hope Medical Center, Duarte, California; *High Dose Chemotherapy and Autologous Peripheral Stem Cell Transplantation for HIV Lymphomas: A Phase IIa Study of Comparative Marking Using a Ribozyme Gene and a Neutral Gene*. Sponsor: Ribozyme Pharmaceuticals, Inc.

NIH/ORDA Receipt Date: 10-6-97. Sole FDA Review Recommended by NIH/ORDA: 10-27-97
Closed to new enrollment: 12-17-99

9710-219 (Open) Gene Therapy/Phase I/Cancer/Bladder/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intravesical Administration

Pagliaro, Lance C.; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Trial of Intravesical Ad-p53 Treatment in Locally Advanced and Metastatic Bladder Cancer.*

NIH/ORDA Receipt Date: 10-21-97. Sole FDA Review Recommended by NIH/ORDA: 11-10-97

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9710-220 (Open) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Bronchoscopy or Percutaneous Intratumoral Injection

Dobbs, Tracy W.; East Tennessee Oncology/Hematology, P.C., Knoxville, Tennessee; *A Phase II Gene Therapy Study in Patients with Non-Small Cell Lung Cancer Using SCH 58500 (rAd/p53) in Combination with Chemotherapy for Multiple Cycles.* Sponsor: Schering Plough Research Institute

NIH/ORDA Receipt Date: 10-31-97. Not Selected for RAC Public Review: 12-15-97

9711-221 (Closed) Gene Therapy/Phase I/Other/ Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Administration

Crystal, Ronald G.; The New York Hospital-Cornell Medical Center, New York, New York; *Phase I Study of Direct Administration of a Replication-Deficient Adenovirus Vector (Ad_{GV}VEGF121.10) Containing the VEGF121 cDNA to the Ischemic Myocardium of Individuals with Life Threatening Diffuse Coronary Artery Disease.* Sponsor: Parke-Davis Pharmaceutical Research.

NIH/ORDA Receipt Date: 11-4-97. Publicly Reviewed at the December 16, 1997 RAC meeting
Completed, long-term follow-up continued: October 1999

9711-222 (Closed) Gene Therapy/Phase I/Monogenetic Disease/Canavan Disease/In Vivo/Autologous Brain Cells/Plasmid DNA/Adeno-Associated Virus/Protamine/Cationic Liposome Complex/DC-Cholesterol-DOPE/Aspartoacylase cDNA/Intracranial (Ommaya Reservoir)

Freese, Andrew; Thomas Jefferson University, Philadelphia, Pennsylvania; *Gene Therapy of Canavan Disease*.

NIH/ORDA Receipt Date: 11-12-97. Not Selected for RAC Public Review: 1-26-98
Closed: 5-22-00

9712-223 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Allogeneic Neuroblastoma Cell Lines/Retrovirus/Cytokine/Interleukin-2 (IL-2)/Plasmid/Electroporation/Chemokine/Lymphotactin/Subcutaneous Injection

Hale, Gregory; St. Jude Children's Research Hospital, Memphis, Tennessee; *Phase I Study of Chemokine and Cytokine Gene Modified Allogeneic Neuroblastoma Cells for Treatment of Relapsed/Refractory Neuroblastoma Using a Retroviral Vector*.

NIH/ORDA Receipt Date: 12-3-97. Not Selected for RAC Public Review: 12-29-97

9712-224 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous Tumor Cells (Non-Irradiated)/Type 5 Adenovirus/Cytokine/Interleukin-2 (IL-2)/Chemokine/Lymphotactin/Subcutaneous Injection

Hale, Gregory; St. Jude Children's Research Hospital, Memphis, Tennessee; *Phase I Study of Chemokine and Cytokine Gene Modified Autologous Neuroblastoma Cells for Treatment of Relapsed/Refractory Neuroblastoma Using an Adenoviral Vector*.

NIH/ORDA Receipt Date: 12-3-97. Not Selected for RAC Public Review: 12-29-97
Closed to new enrollment: 3-17-04

9712-225 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/Antisense TAR/Transdominant Rev/Intravenous

Isola, Luis M.; Mount Sinai Medical Center, New York, New York; *A Phase I Trial of Autologous and Allogeneic Bone Marrow Transplantation with Genetically Marked Cells for the Treatment of HIV Associated Lymphoid Malignancies.*

NIH/ORDA Receipt Date: 12-15-97. Not Selected for RAC Public Review: 1-7-98
IND withdrawn: 4-4-00

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9712-226 (Open) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Tumor Suppressor Gene/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injections

Dreicer, Robert; University of Iowa College of Medicine, Iowa City, Iowa; Simon, George R.; University of Colorado Health Sciences Center, Denver, Colorado; Williamson, Stephen; University of Kansas Medical Center, Kansas City, Kansas; VanEcho, David A.; University of Maryland School of Medicine, Baltimore, Maryland; Rosen, Fred; University of Illinois at Chicago Hospitals & Clinics; Endicott, James N.; University of South Florida, Tampa, Florida; Bier-Laning, Carol M.; University of Texas Southwestern Medical Center at Dallas, Dallas, Texas; Minn, Heikki; Turku University Central Hospital, Turku Finland; Guertin, Louis; CHUM - Pavilion Notre-Dame, Montreal, Quebec; Liu, Fei-Fei; Princess Margaret Hospital, Toronto, Ontario; Wadler, Scott; Montefiore Medical Center, Albert Einstein College of Medicine; Bronx, New York; Goss, Glenwood D.; Ottawa Regional Cancer Centre, Ottawa, Ontario; Saarilanti, Kauko; Helsinki University Central Hospital, Helsinki Finland; Mudad, Raja; Tulane University Medical Center, New Orleans, Louisiana; Spiro, Jeffrey; University of Connecticut Health Center, Farmington, Connecticut; Zielinski, Christoph; University of Vienna; Link, Brian; University of Iowa Hospital and Clinics, Iowa City, Iowa; and Truelson, John; University of Texas Southwestern Medical School, Dallas, Texas; *A Phase II, Multi-Center, Open Label, Study to Evaluate Effectiveness and Safety of Ad5CMV-p53 Administered by Intra-Tumoral Injections in 39 Patients with Recurrent Squamous Cell Carcinoma of the Head and Neck (SCCHN)*. Sponsor: Aventis (formerly Gencell)

NIH/ORDA Receipt Date: 12-17-97. Not Selected for RAC Public Review: 1-9-98

9801-227 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Head and Neck Cancer/Immunotherapy/In Vitro/Autologous Fibroblasts/Lethally Irradiated/Retrovirus/Cytokine/Interleukin-12 cDNA/Neomycin Phosphotransferase cDNA/Intratumoral Injection

Lotze, Michael T.; University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; *IL-12 Gene Therapy Using Direct Injection of Tumors with Genetically Engineered Autologous Fibroblasts (A Phase II Study)*.

NIH/ORDA Receipt Date: 1-2-98. Not Selected for RAC Public Review: 2-18-98
Protocol is terminated: 11-1-01

9801-228 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/ Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Acyclovir/Intraperitoneal Injection

Kieback, Dirk G.; Baylor College of Medicine, Houston, Texas; *Phase I Study of Concomitant Adenovirus-Mediated Transduction of Ovarian Cancer with HSV-tk Gene Followed by Intravenous Administration of Acyclovir and Chemotherapy with Topotecan in Patients after Optimal Debulking Surgery for Recurrent Ovarian Cancer*.

NIH/ORDA Receipt Date: 1-14-98. Not Selected for RAC Public Review: 2-5-98

9801-229 (Open) Gene Therapy/Phase I/Cancer/Prostate/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection

Kadmon, Dov; Baylor College of Medicine, Houston, Texas; *Neoadjuvant Pre-radical Prostatectomy Gene Therapy (HSV-tk Gene Transduction Followed by Ganciclovir) in Patients with Poor Prognostic Indicators*.

NIH/ORDA Receipt Date: 1-16-98. Not Selected for RAC Public Review: 2-13-98

9801-230 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/Antisense TAR/Antisense tat | rev/In Vitro/CD34+ Cells/Intravenous

Cowan, Morton J. and Conant, Marcus A.; University of California, San Francisco, San Francisco, California; *Evaluation of the Safety and Effects of Ex Vivo Modification and Re-infusion of CD34+ Cells by an Antisense Construct Against HIV-1 in a Retroviral Vector.* Sponsor: Enzo Therapeutics, Inc.

NIH/ORDA Receipt Date: 1-20-98. Not Selected for RAC Public Review: 3-26-98
Closed to enrollment, follow-up is continuing: 06-24-03

9802-231 (Open) Gene Therapy/Phase I/II/Monogenic Disease/Chronic Granulomatous Disease/In Vitro/CD 34+ Autologous Peripheral Blood Cells/Retrovirus/p47phox/gp91phox/Intravenous

Malech, Harry L.; National Institutes of Health, Bethesda, Maryland; *Gene Therapy Approach for Chronic Granulomatous Disease*.

NIH/ORDA Receipt Date: 2-2-98. Not Selected for RAC Public Review: 2-20-98
Closed to enrollment: 4-12-04; Re-opened: 10-24-06

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9802-232 (Closed) Gene Therapy/Phase I/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Administration

Isner, Jeffrey M.; Tufts University School of Medicine, Boston, Massachusetts; *Gene Therapy for Myocardial Angiogenesis*.

NIH/ORDA Receipt Date: 2-3-98. Publicly Reviewed at the June 18, 1998 RAC meeting
Follow-up has been completed: 11-29-01

9802-233 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection

Dreicer, Robert; the University of Iowa Hospitals and Clinics, Iowa City, Iowa; Seigler, Hilliard; Duke University Medical Center, Durham, North Carolina; Rubin, Joseph; Mayo Clinic, Rochester, Minnesota; DeConti, Robert; H. Lee Moffitt Cancer Center, Tampa, Florida; Gonzalez, Rene; the University of Colorado Cancer Center, Denver Colorado; Macdonald, John S.; Saint Vincent's Hospital and Medical Center, New York, NY; Hutchins, Laura; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Samlowski, Wolfram E.; the University of Utah Health Sciences Center, Salt Lake City, Utah; Bearden, James D.; Spartanburg Regional Medical Center, Spartanburg, South Carolina; Atkins, Michael B.; Beth Israel Medical Center, Boston, Massachusetts; Schwarzenberger, Paul O.; Louisiana State University Medical Center, New Orleans, Louisiana; Deisseroth, Albert; Yale University School of Medicine, New Haven, Connecticut; Blum, Ronald H.; Beth Israel Medical, New York, New York; Lutzky, Jose; Mount Sinai Medical Center, Miami, Florida; and Wallach, Sabina R.; Scripps Memorial Hospital, San Diego, La Jolla, and Encinitas, California; *Phase II Study of Direct Gene Transfer of HLA-B7 Plasmid DNA/DMRIE/DOPE Lipid Complex (Allovectin-7) as an Immunotherapeutic Agent in Patients with Stage III or IV Melanoma with No Treatment Alternatives*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 2-9-98. Not Selected for RAC Public Review: 8-28-98

9802-234 (Closed) Gene Therapy/Phase III/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection

Thompson, John A.; University of Washington, Seattle, Washington; Dreicer, Robert; the University of Iowa Hospitals and Clinics, Iowa City, Iowa; Seigler, Hilliard; Duke University Medical Center, Durham, North Carolina; Galanis, Evanthia; Mayo Clinic, Rochester, Minnesota; DeConti, Robert; H. Lee Moffitt Cancer Center, Tampa, Florida; Macdonald John S.; Saint Vincent's Hospital and Medical Center, New York, NY; Hutchins, Laura; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Samlowski, Wolfram E.; the University of Utah Health Sciences Center, Salt Lake City, Utah; Bearden, James D.; Spartanburg Regional Medical Center, Spartanburg, South Carolina; Atkins, Michael B.; Beth Israel Medical Center, Boston, Massachusetts; Gibbs, John and Oleksowicz, Leslie; Roswell Park Cancer Institute, Buffalo, New York; Schwarzenberger, Paul O.; Louisiana State University Medical Center, New Orleans, Louisiana; Ernstoff, Marc, Dartmouth Hitchcock Medical Center, Lebanon, New Hampshire; Campbell, Laura, Louisiana State University Medical Center, Shreveport, Louisiana; Levine, Edward; Wake Forest University Medical Center, Winston-Salem, North Carolina; Schuchter, Lynn M.; University of Pennsylvania Cancer Center, Philadelphia, Pennsylvania; Deisseroth, Albert; Yale University School of Medicine, New Haven, Connecticut; Paciucci, Paolo A.; Mount Sinai Medical Center, New York, New York; Richart, John; Saint Louis University Health Sciences Center, St. Louis, Missouri; Meyskens Jr., Frank L.; University of California, Irvine, Orange, California; Blum, Ronald H.; Beth Israel Medical, New York, New York; Amatruda, Thomas; Virginia Piper Cancer Institute Abbott Northwestern Hospital, Minneapolis, Minnesota; Kuzel, Timothy; Northwestern Medical Faculty Foundation and Northwestern Memorial Hospital, Chicago, Illinois; Hawkins, Michael; Washington Cancer Institute, Washington, DC; Whitman, Eric D.; The Melanoma Center of St. Louis, Saint Louis, Missouri; Cobb, Patrick; Billings Interhospital Oncology Project, Billings, Montana; Amin, Bipinkumar; Mid Dakota Clinic, Bismarck, North Dakota; Chowhan, Naveed; Cancer Care Center Incorporated, New Albany, Indiana; Lutzky, Jose; Mount Sinai Medical Center, Miami, Florida; Amatruda, Thomas; North Memorial Healthcare, Hubert H. Humphrey Cancer Center, Robbinsdale, Minnesota; Patel, Ravi; Comprehensive Blood and Cancer Center, Bakersfield, California; Dobbs, Tracy W.; Baptist Hospital of East Tennessee, Knoxville, Tennessee; Ahmed, Fakhiuddin; HemOnCare, P.C., Brooklyn, New York; Thant, Myo; Maryland Hematology/Oncology Associates, Baltimore, Maryland; Stark, James J.; Maryview Medical Center, Portsmouth, Virginia; Arena, Francis; Arena Oncology Associates, Great Neck, New York; Brotherton, Timothy; Danville Hematology and Oncology, Inc. Danville Diagnostic Imaging Center, Danville, Virginia; Brouillard, Robert P.; Scripps Memorial Hospital, La Jolla, Encinitas, and El Cajon, California; Polikoff, Jonathan A.; Kaiser Permanente Medical Group, San Diego, California; Ritch, Paul S.; Medical College of Wisconsin and Froedtert Memorial Lutheran Hospital, Milwaukee, Wisconsin; Bernstein, Joel I.; Scripps Memorial Hospital, La Jolla, Encinitas, El Cajon, California; Richards, Jon; Lutheran General Hospital, Park Ridge, Illinois; and Giguere, Jeffrey; Hematology and Oncology Associates, Greenville, South Carolina; *A Controlled, Randomized Phase III Trial Comparing the Response to Dacarbazine with and without Allovectin-7 in Patients with Metastatic Melanoma.* Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 2-9-98. Not Selected for RAC Public Review: 7-20-98

9802-235 (Open) Gene Therapy/Phase I/Cancer/Brain Tumors/Glioblastoma/Vector-Directed Cell Lysis/In Vivo/Autologous Tumor Cells/Herpes Simplex Virus Type I/Tumor Lysis/Intratumoral Injection

Markert, James; University of Alabama, Birmingham, Alabama; and Medlock, Michael; Georgetown University Medical Center, Washington, D.C.; *A Dose Escalating Phase I Study of the Treatment of Malignant Glioma with G207, a Genetically Engineered HSV-1.* Sponsor: NeuroVir, Inc.

NIH/ORDA Receipt Date: 2-10-98. Publicly Reviewed at the June 18, 1998 RAC meeting

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9802-236 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Autologous Tumor Cells/Adenovirus Type 5/Replication-competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intratumoral Injection

Simons, Jonathan W.; Johns Hopkins University School of Medicine, Baltimore, Maryland; *A Phase I Study of the Intraprostatic Injections of CN706, a Prostate-Specific Antigen Gene-Regulated Cytolytic Adenovirus, in Patients with Locally Recurrent Cancer Following Definitive Radiotherapy*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 2-13-98. Publicly Reviewed at the June 19, 1998 RAC meeting

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9802-237 (Closed) Gene Therapy/Phase I/Rheumatoid Arthritis/In Vivo/Autologous Synovial Cells/Naked Plasmid DNA/Herpes Simplex Virus Thymidine Kinase Gene/Ganciclovir/Intra-Articular Administration

Roessler, Blake J; The University of Michigan Medical Center, Ann Arbor, Michigan; *Molecular Synovectomy by In Vivo Gene Transfer: A Phase I Trial*.

NIH/ORDA Receipt Date: 2-13-98. Publicly Reviewed at the June 18, 1998 RAC meeting
Closed to new enrollment: 5-15-02.

9802-238 (Closed) Gene Therapy/Phase I-II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration

Lee, Joon S.; University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *Phase 1/2 Study of the Effects of Ascending Doses of Adenovirus Mediated Human FGF-4 Gene Transfer in Patients with Stable Exertional Angina*. Sponsor: Cardium Therapeutics.

NIH/ORDA Receipt Date: 2-24-98. Publicly Reviewed at the June 18, 1998 RAC meeting
Completed: 06-03

9802-239 (Closed) Gene Therapy/Phase I-II/Cancer/Hepatic Metastasis of Colorectal Carcinoma/Immunotherapy/In Vitro/Autologous CD4+ and CD8+ Lymphocytes/Retrovirus/CC49-Zeta T Cell Receptor/Hepatic Artery Infusion

Bergsland, Emily K.; University of California, San Francisco, San Francisco, California; *A Phase I/II Study of Hepatic Infusion of Autologous CC49-Zeta Gene-Modified T Cells in Patients with Hepatic Metastasis from Colorectal Cancer*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 2-25-98. Not Selected for RAC Public Review: 3-17-98
Notification from sponsor that trial is closed: 4-09-01

9803-240 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Pro-Drug/In Vivo/Autologous Tumor Cells/Adenovirus/ Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection

Rom, William N.; New York University School of Medicine, New York, New York; and Woo, Savio L.C.; Mount Sinai School of Medicine, New York, New York; Phase *I Trial of Adenoviral Vector Delivery of the Herpes Simplex Thymidine Kinase Gene by Intratumoral Injection Followed by Intravenous Ganciclovir in Patients with Advanced Non-Small Cell Lung Cancer*.

NIH/ORDA Receipt Date: 3-3-98. Not Selected for RAC Public Review: 3-23-98

9803-241 (Closed) Gene Therapy/Phase I-II/Cancer/Chronic Myelogenous Leukemia/Multiple Myeloma/Non-Hodgkin's Lymphoma/Chronic Lymphocytic Leukemia/Adoptive Immunotherapy/In Vitro/Sibling Peripheral Blood Lymphocytes/Retrovirus/Herpes Simplex Virus Thymidine Kinase/Ganciclovir/Intravenous Infusion

Bensinger, William I.; University of Washington School of Medicine, Seattle, Washington; Parker, Pablo M.; City of Hope National Medical Center, Duarte, California; Henslee-Downey, Peggy J. and Abhyankar, Sunil; Richland Memorial Hospital, University of South Carolina, Columbia, South Carolina; Giralt, Sergio; University of Texas, M.D. Anderson Cancer Center, Houston, Texas; Cornetta, Kenneth; Indiana University-Purdue University, Indianapolis, Indiana; and Carabasi, Matthew; The University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I/II Outpatient, Multicenter, Intrapatient, Multiple Dose Escalation Study of Herpes Simplex Virus Thymidine Kinase (HSV-TK) Transduced Mononuclear Cells in Subjects with Persistent or Relapsed Chronic Myleogenous Leukemia, Chronic Lymphocytic Leukemia, Multiple Myeloma, and Non-Hodgkin's Lymphoma after HLA-Matched Sibling Allogeneic Stem Cell Transplant*. Sponsor: Chiron Corporation

NIH/ORDA Receipt Date: 3-27-98. Not Selected for RAC Public Review: 4-17-98
5-5-00: IND no longer active

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9803-242 (Closed) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous Leukemic Cells/Adenovirus/Serotype 5/CD 154 cDNA/Intravenous Infusion

Kipps, Thomas J.; University of California, San Diego, San Diego, California; *A Phase I Study of CD 154 Gene-Transduced Leukemia Cells in Patients with Chronic Lymphocytic Leukemia*.

NIH/ORDA Receipt Date: 3-30-98. Not Selected for RAC Public Review: 4-17-98
Notification from Immunogenex, now the sponsor of this trial, that study has been completed: 3-20-01

9804-243 (Closed) Gene Therapy/Phase I/Other/Peripheral Arterial Disease/In Vivo/Ischemic Lower Limb/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Crystal, Ronald G.; Cornell University Medical College, New York, New York; Deitcher, Steven and Goldman, Corey; The Cleveland Clinic Foundation, Cleveland, Ohio; Rajagopalan, Sanjay; The University of Michigan, Ann Arbor, Michigan; Mohler III, Emile R.; University of Pennsylvania Health System, Philadelphia, Pennsylvania; and Trachtenberg, Jeffrey; University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *Phase I Study of Direct Administration of a Replication Deficient Adenovirus vector (Ad_{GV}VEGF121.10) Containing the VEGF121 cDNA to the Ischemic Lower Limb of Individuals with Peripheral Vascular Disease.* Sponsor: Parke-Davis Pharmaceutical Research.

NIH/ORDA Receipt Date: 4-10-98. Not Selected for RAC Public Review: 4-30-98
Completed, long-term follow-up is ongoing: October 1999

9804-244 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Cationic Liposome Complex/Plasmid DNA/Interleukin-2 cDNA/Staphylococcus Enterotoxin B (SEB)/Intratumoral Injection

Walsh, Patrick; University of Colorado Health Sciences Center, Denver, Colorado; *A Phase I Study Using Direct Combination DNA Injections for the Immunotherapy of Metastatic Melanoma*.

NIH/ORDA Receipt Date: 4-10-98. Publicly Reviewed at the June 19, 1998 RAC meeting
Closed to enrollment, follow-up is continuing: 9-5-01

9804-245 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) cDNA/Aerosol Administration

Moss, Richard; Stanford University School of Medicine, Palo Alto, California; Aitken, Moira; University of Washington Medical Center, Seattle, Washington; and Waltz, David; Harvard Medical School, Boston, Massachusetts; *A Phase I Study of Aerosolized tgAAVCF for the Treatment of Cystic Fibrosis Patients with Mild Lung Disease.* Sponsor: Targeted Genetics Corporation.

NIH/ORDA Receipt Date: 4-18-98. Not Selected for RAC Public Review: 12-3-98

9804-246 (Closed) Gene Therapy/Phase II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Oncogene Regulation/HER-2/neu/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intratumoral Injection

Yoo, George H.; Wayne State University School of Medicine, Detroit, Michigan; Villaret, Douglass B.; University of Washington, Seattle, Washington; Gleich, Lyon; University of Cincinnati Medical Center, Cincinnati, Ohio; Hanna, Ehab; University of Arkansas Cancer Research Center, Little Rock, Arkansas; and Kenady, Daniel E. and Valentino, Joseph; University of Kentucky, Lexington, Kentucky; *A Multicenter Phase II Study of E1A Lipid Complex for the Intratumoral Treatment of Patients with Recurrent Head and Neck Squamous Cell Carcinoma.* Sponsor: Targeted Genetics Corporation.

NIH/ORDA Receipt Date: 4-18-98. Not Selected for RAC Public Review: 2-1-99
Completed: 8-08-02

9804-247 (Closed) Gene Therapy/Phase I/Monogenic Disease/Hemophilia A/In Vitro/Electroporation/Autologous Fibroblasts/Plasmid DNA/Factor VIII cDNA/Intraperitoneal Implantation

Roth, David A.; Beth Israel Deaconess Medical Center, Boston, Massachusetts; *A Phase I Safety Study of Autologous Transfected Human Fibroblasts Producing Human Factor VIII in Patients with Severe Hemophilia A.* Sponsor: Transkaryotic Therapies, Inc.

NIH/ORDA Receipt Date: 4-17-98. Publicly Reviewed at the June 19, 1998 RAC meeting
Closed to enrollment: 3-26-03

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9804-248 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Breast Cancer/Immunotherapy/In Vivo/Adenovirus/Serotype 5/B7.1 (CD80) cDNA/Intratumoral Injection

Schuchter, Lynn; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *Phase I Trial of Therapeutic Cancer Vaccine Using Intratumoral Injections of B7-1 (H5.030CMVhB7) in Patients with Metastatic Melanoma or Metastatic Breast Cancer*.

NIH/ORDA Receipt Date: 4-23-98. Not Selected for RAC Public Review: 5-13-98

9804-249 (Open) Gene Therapy/Phase I/Cancer/Adenocarcinoma Expressing Carcinoembryonic Antigen (CEA)/In Vitro/Autologous T Lymphocytes/Retrovirus/anti-CEA-sFv-Zeta T Cell Receptor/Intravenous Infusion

Junghans, Richard Paul; Beth Israel Deaconess Medical Center, Boston, Massachusetts; *Phase I Study of T Cells Modified with Chimeric AntiCEA Immunoglobulin-T Cell Receptors (IgTCR) in Adenocarcinoma*.

NIH/ORDA Receipt Date: 4-28-98. Not Selected for RAC Public Review: 5-18-98

9804-250 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injections

Swisher, Steven; University of Texas M.D. Anderson Cancer Center/Texas Heart Institute, Houston, Texas; *An Efficacy Study of Adenoviral Vector Expressing Wildtype p53 (Ad5CMV-p53) Administered Intralesionally as an Adjunct to Radiation Therapy in Patients with Non-Small Cell Lung Cancer.* Sponsor: Aventis (formerly Gencell)

NIH/ORDA Receipt Date: 4-28-98. Not Selected for RAC Public Review: 5-18-98

9805-251 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/MUC -1/Interleukin-2/Intramuscular Injection

Figlin, Robert; University of California at Los Angeles, Los Angeles, California; *Phase I/II Trial of Antigen-Specific Immunotherapy in MUC-1 Positive Patients with Adenocarcinoma of the Prostate Using Vaccinia Virus-MUC1-IL2 (TG 1031)*. Sponsor: Transgene, S.A.

NIH/ORDA Receipt Date: 5-1-98. Not Selected for RAC Public Review: 5-22-98
Completed, long-term follow-up continues: 5-05-03

9805-252 (Open) Gene Therapy/Phase I/Cancer/Colorectal/In Vitro/Allogeneic Tumor Cells and Fibroblasts/Lethally Irradiated/Plasmid DNA/Interleukin-2 cDNA/B7.1 (CD80)/Subcutaneous Injection

Sobol, Robert E.; Sidney Kimmel Cancer Center, San Diego, California; *A Phase I Study of Allogeneic Tumor Cells Genetically Modified to Express B7.1 (CD80) Mixed with Allogeneic Fibroblasts Genetically Modified to Secrete IL-2 in Patients with Colorectal Carcinoma.*

NIH/ORDA Receipt Date: 5-7-98. Not Selected for RAC Public Review: 5-27-98

9805-253 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD8+ T Cells/ Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous Infusion

Scadden, David T.; Massachusetts General Hospital Cancer Center, Harvard Medical School, Boston, Massachusetts; Mitsuyasu, Ronald; University of California, Los Angeles, Los Angeles, California; and Deeks, Steven; University of California, San Francisco, San Francisco, California; *A Phase II Study of Autologous CD4-Zeta Gene-Modified T Cells in HIV Infected Patients with Undetectable Plasma Viremia on Highly Active Anti-Retroviral Drug Therapy* Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 5-14-98. Not Selected for RAC Public Review: 6-3-98
Study closed to new accrual, follow-up is continuing: 7-13-01

9805-254 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/gp 100 Melanoma Antigen/Intradermal or Intramuscular Injection

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Immunization of Patients with Metastatic Melanoma Using DNA Encoding the GP100 Melanoma Antigen.* Sponsor: National Cancer Institute - Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 6-4-98. Not Selected for RAC Public Review: 6-24-98

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9806-255 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intraperitoneal Administration

Muller, Carolyn Y.; University of Texas Southwestern Medical School, Dallas, Texas; *Phase I Trial of Intraperitoneal Adenoviral p53 Gene Therapy in Patients with Advanced Recurrent or Persistent Ovarian Cancer*. Sponsor: National Cancer Institute - Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 6-2-98. Not Selected for RAC Public Review: 6-22-98
Closed to accrual: 4-15-02

9806-256 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal and Subcutaneous Injections

Suzuki, Tsuneo; University of Kansas Medical Center, Kansas City, Kansas; *Autologous, Irradiated, Melanoma Cells Transduced Ex Vivo with an Adenovirus Vector (Adv/GM-CSF) Expressing Granulocyte-Macrophage Colony Stimulating Factor Gene*.

NIH/ORDA Receipt Date: 6-3-98. Not Selected for RAC Public Review: 6-23-98

9806-257 (Open) Gene Therapy/Phase I/Cancer/Breast/Colon/Head and Neck/Soft Tissue Sarcoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/ Adenovirus/Serotype 5/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal and Subcutaneous Injections

Suzuki, Tsuneo; University of Kansas Medical Center, Kansas City, Kansas; *Autologous, Irradiated, Cancer Cells (Breast Cancer, Colon Cancer, Head and Neck Cancer, and Soft Tissue Sarcoma) Transduced Ex Vivo with an Adenovirus Vector (Adv/GM-CSF) Expressing Granulocyte-Macrophage Colony Stimulating Factor Gene.*

NIH/ORDA Receipt Date: 6-3-98. Not Selected for RAC Public Review: 6-23-98

9806-258 (Closed) Gene Therapy/Phase I/ Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration

Crystal, Ronald G.; Cornell University Medical College, New York, New York; *Phase I Study of Direct Administration of a Replication Deficient Adenovirus Vector (Ad_{GV}VEGF121.10) Containing the VEGF121 cDNA to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease Via Minimally Invasive Surgery.* Sponsor: Parke-Davis Pharmaceutical Research.

NIH/ORDA Receipt Date: 6-8-98. Not Selected for RAC Public Review: 8-13-98
Completed, long-term follow-up continued: October 1999

9806-259 (Closed) Gene Therapy/Phase II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1102/Interleukin-2 cDNA/Intratumoral Injection

Figlin, Robert; University of California at Los Angeles, Los Angeles, California; Thompson, John, A.; University of Washington, Seattle, Washington; Galanis, Evanthia; Mayo Clinic, Rochester, Minnesota; and Bukowski, Ronald; Cleveland Clinic Foundation, Cleveland, Ohio; *Phase II Study of Direct Gene Transfer of IL-2 Plasmid DNA/DMRIE/DOPE Lipid Complex (Leuvectin) as an Immunotherapeutic Regimen in Patients with Metastatic Renal Cell Carcinoma.* Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 6-15-98. Not Selected for RAC Public Review: 7-6-98

9806-260 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical-1005/HLA-B7/Beta 2-Microglobulin cDNA/Concurrent Interleukin-2 Injection/Direct Intratumoral Injection

Hersh, Evan; Arizona Cancer Center, Tucson, Arizona; *Phase I Study of HLA-B7/β2M Plasmid DNA/DMRIE/DOPE Lipid Complex (Allovectin-7) by Direct Gene Transfer with Concurrent Low-Dose Subcutaneous IL-2 Protein Therapy as an Immunotherapeutic Regimen in Malignant Melanoma*.

NIH/ORDA Receipt Date: 6-26-98. Not Selected for RAC Public Review: 7-16-98

9806-261 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/Retrovirus/Transdominant Rev or Rev and Antisense Pol 1/Intravenous Infusion

Amado, Rafael G.; University of California at Los Angeles, Los Angeles, California; Yuen, Alan R.; Stanford University Medical Center; Stanford, California; Scadden, David T.; Massachusetts General Hospital, Boston, Massachusetts; Lill, Michael; Cedars-Sinai Medical Center, Los Angeles, California; and Carabasi, Matthew; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I/II Study of the Safety and Feasibility of RevM10 or RevM10/Antisense Pol 1 Transduced Hematopoietic Stem Cells (HSC) in HIV-1 Related Non-Hodgkin's Lymphoma Patients Already Being Treated with High Dose Chemotherapy and Peripheral Blood Stem Cell Support*. Sponsor: Systemix, Inc.

NIH/ORDA Receipt Date: 6-30-98. Not Selected for RAC Public Review: 7-20-98

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9807-262 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intraperitoneal Administration

Wolf, Judith K.; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Study of Ad-p53 (NSC#683550) for Patients with Platinum- and Paclitaxel-Resistant Epithelial Ovarian Cancer.*

NIH/ORDA Receipt Date: 7-24-98. Not Selected for RAC Public Review: 8-13-98

9808-263 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection

Lang, Frederick F., Jr. and Yung, W. K. Alfred; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; and Greenberg, Harry; University of Michigan, Ann Arbor, Michigan; *Phase I Trial of Adenovirus-Mediated Wild Type p53 Gene Therapy for Malignant Gliomas.* Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 8-13-98. Not Selected for RAC Public Review: 9-2-98

9808-264 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Vaccinia Virus/MUC-1/Interleukin-2/Intramuscular Injection

Gitlitz, Barbara J.; University of California Los Angeles, Los Angeles, California; *Phase I/II Trial of Antigen-Specific Immunotherapy in MUC-1 Positive Patients with Advanced Non-Small Cell Lung Cancer Using Vaccinia-Virus-MUC1-IL2 (TG1031)*. Sponsor: Transgene, S.A.

NIH/ORDA Receipt Date: 8-27-98. Not Selected for RAC Public Review: 9-18-98

9809-265 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Chemoprotection/In Vitro/Peripheral Blood CD34+ Cells/Retrovirus/O^6-Methylguanine DNA Methyltransferase cDNA/Intravenous Infusion

Gerson, Stanton L.; Case Western Reserve University, Cleveland, Ohio; *Mutant ΔMGMT [also G156AMGMT] Gene Transfer Into Human Hematopoietic Progenitors to Protect Hematopoiesis During O^6-Benzylguanine (BG, NSC 637037) and BCNU Followed by Temozolomide Therapy of Advanced Solid Tumors.*

NIH/ORDA Receipt Date: 9-2-98. Not Selected for RAC Public Review: 9-23-98

9809-266 (Open) Gene Therapy/Phase I-II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Human Interferon-alpha cDNA/Intratumoral Injection

McQuone, Shelly J.; The University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Multi-Center, Open-Label, Multiple Administration, Rising Dose Study of the Safety, Tolerability, and Efficacy of IFN-alpha Gene Medicine in Patients with Unresectable or Recurrent/Refractory Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: GeneMedicine, Inc.

NIH/ORDA Receipt Date: 9-22-98. Not Selected for RAC Public Review: 3-19-99

9810-267 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection

Morris, John C.; National Institutes of Health, Bethesda, Maryland; *A Phase I Study of Intralesional Administration of an Adenovirus Vector Expressing the HSV-1 Thymidine Kinase Gene (AdV.RSV-TK) in Combination with Escalating Doses of Ganciclovir in Patients with Cutaneous Metastatic Malignant Melanoma.*

NIH/ORDA Receipt Date: 10-6-98. Not Selected for RAC Public Review: 10-27-98

9810-268 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Irradiated/Adenovirus/Serotype 5/B7.1 (CD80) cDNA/Subcutaneous Injection

Antonia, Scott J.; University of South Florida, Tampa, Florida; *Treatment of Patients with Stage IV Renal Cell Carcinoma with B7-1 Gene-Modified Autologous Tumor Cells and Systemic IL-2.*

NIH/ORDA Receipt Date: 10-26-98. Not Selected for RAC Public Review: 11-30-98
Closed to new accrual: 3-29-01

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9811-269 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Type 5/MART-1 Melanoma Antigen/Intravenous or Intradermal Injection

Economou, James S.; UCLA Medical Center, Los Angeles, California; *A Phase I Trial Testing MART-1 Genetic Immunization in Malignant Melanoma.*

NIH/ORDA Receipt Date: 11-17-98. Not Selected for RAC Public Review: 12-8-98

9811-270 (Closed) Gene Therapy/Phase II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1005/HLA-B7/Beta2-Microglobulin cDNA/Direct Intratumoral Injection

Hanna, Ehab; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Wagman, Lawrence D.; City of Hope National Medical Center, Duarte, California; Gluckman, Jack L.; University of Cincinnati Medical Center, Cincinnati, Ohio; and Wolf, Gregory T.; University of Michigan Medical Center, Ann Arbor, Michigan; *Phase II Study of the Safety, Efficacy, and Effect on Quality of Life of Allovectin-7 Immunotherapy for the Treatment of Recurrent or Persistent Squamous Cell Carcinoma of the Head and Neck.* Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 11-19-98. Not Selected for RAC Public Review: 2-5-99

9811-271 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with Moderate-Risk Critical Limb Ischemia.* Sponsor: Vascular Genetics, Inc.

NIH/ORDA Receipt Date: 11-23-98. Not Selected for RAC Public Review: 12-14-98
Follow-up has been completed: 11-29-01

9811-272 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/MUC-1/Intradermal Injection

Kufe, Donald W.; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Trial of Recombinant Vaccinia Virus that Expresses DF3/MUC1 in Patients with Metastatic Adenocarcinoma of the Breast.*

NIH/ORDA Receipt Date: 11-23-98. Not Selected for RAC Public Review: 12-24-98

9812-273 (Open) Gene Therapy/Phase I-II/Infectious Diseases/Human Immunodeficiency Virus-1 (HIV-1)/In Vitro/Immunotherapy/Autologous CD8+ HIV-Specific T Cells/Retrovirus/Neomycin Phosphotransferase Gene/Intravenous Infusion

Riddell, Stanley R.; Fred Hutchinson Cancer Research Center, Seattle, Washington; *The Safety and Antiviral Efficacy of Cellular Adoptive Immunotherapy with Autologous CD8+ HIV-Specific Cytotoxic T Cells Combined with Interleukin-2 for HIV Seropositive Individuals*.

NIH/ORDA Receipt Date: 12-3-98. Not Selected for RAC Public Review: 1-5-99

9812-274 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Fibroblast Growth Factor (FGF) cDNA/Intramuscular Injection

Comerota, Anthony J.; Temple University School of Medicine, Philadelphia, Pennsylvania; Laird, John R.; Washington Hospital Center, Washington, D.C.; Sequeira, Rafael F.; University of Miami, School of Medicine, Miami, Florida; Henry, Timothy; Hennepin County Medical Center, Minneapolis, Minnesota; and Chronos, Nicholas; Atlanta Cardiology Group, Atlanta, Georgia; *A Phase I, Multi-Center, Open Label, Safety and Tolerability Study of Increasing Single Dose of NV1FGF Administered by Intra-Muscular Injection in Patients with Severe Peripheral Artery Occlusive Disease.* Sponsor: Aventis (formerly Gencell).

NIH/ORDA Receipt Date: 12-17-98. Not Selected for RAC Public Review: 2-4-99

9812-275 (Open) Gene Therapy/Phase I/Cancer/Advanced Malignancies/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intravenous Injection

Eckhardt, S. Gail; Institute for Drug Development, Cancer Therapy and Research Center, San Antonio, Texas; *A Pharmacokinetic, Safety and Tolerability Study of Intravenous INGN in Patients with Advanced Cancer*. Sponsor: Introgen Therapeutics, Inc.

NIH/ORDA Receipt Date: 12-18-98. Not Selected for RAC Public Review: 5-13-99

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9812-276 (Open) Gene Therapy/Phase I/Cancer/Prostate/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Valacyclovir/Intratumoral Injection

Gardner, Thomas A. and Chung, Leland W. K.; University of Virginia Health, Charlottesville, Virginia; *Phase I Study of Ad-OC-TK Plus Valacyclovir for the Treatment of Metastatic or Recurrent Prostate Cancer.*

NIH/ORDA Receipt Date: 12-23-98. Not Selected for RAC Public Review: 1-14-99

9812-277 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/Retrovirus/Transdominant Rev/Antisense Pol 1/Intravenous Infusion

Amado, Rafael G.; University of California, Los Angeles, Los Angeles, California; Carabasi, Matthew; University of Alabama at Birmingham, Birmingham, Alabama; Swindells, Susan; University of Nebraska Medical Center, Omaha, Nebraska; and Scadden, David T.; Massachusetts General Hospital, Boston, Massachusetts; *A Phase I/II Study in HIV-1 Infected Patients Infused with CD34+Thy1+ Hematopoietic Stem Cells (HSC) from G-CSF Mobilized Peripheral Blood Retrovirally Transduced with RevM10 or RevM10/Antisense Pol1.* Sponsor: Systemix, Inc.

NIH/ORDA Receipt Date: 12-28-98. Not Selected for RAC Public Review: 1-19-99

9901-278 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/MART-1 Melanoma Antigen/Intramuscular Injection

Conry, Robert M.; University of Alabama at Birmingham, Birmingham, Alabama; *Phase I Dose Escalation Trial of Polynucleotide Immunization with Plasmid DNA Encoding MART-1 (Melanoma Antigen Recognized by T Cells-1) in Patients with Resected Melanoma at Significant Risk for Relapse.*

NIH/ORDA Receipt Date: 1-4-99. Not Selected for RAC Public Review: 1-25-99

9901-279 (Closed) Gene Therapy/Phase I/Monogenic Disease/Hemophilia B/In Vivo/Adeno-Associated Virus/Factor IX Gene/Intramuscular Injection

Manno, Catherine S.; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Phase I Safety Study in Patients with Severe Hemophilia B (Factor IX Deficiency) Using Adeno-Associated Viral Vector to Deliver the Gene for Human Factor IX to Skeletal Muscle.* Sponsor: Avigen.

NIH/ORDA Receipt Date: 1-7-99. Publicly Reviewed at the March 12, 1999 RAC meeting
Closed to enrollment, follow-up is ongoing: 1-26-05

9901-280 (Closed) Gene Therapy/Phase II-III/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intraperitoneal Administration

Buller, Richard; The University of Iowa Hospitals and Clinics, Iowa City, Iowa; Carson, Linda F.; University of Minnesota, Minneapolis, Minnesota; Weisberg, Tracey; Maine Center for Cancer Medicine, Scarborough, Maine; Christopherson, Wayne A.; Mercy Hospital of Pittsburgh, Pittsburgh, Pennsylvania; Molpus, Kelly; University of Nebraska Medical Center, Omaha, Nebraska; Davidson, Susan A.; University of Colorado Health Sciences Center, Denver, Colorado; Gutheil, John C.; Sharp HealthCare, Sidney Kimmel Cancer Center, San Diego, California; Bloss, Jeffrey D.; University of Missouri, Columbia, Missouri; Blum, Ronald; Beth Israel Medical Center, New York, New York; Puls, Larry E.; Greenville Hospital System, Greenville, South Carolina; Teng, Nelson Nan-Hsiung; Stanford University School of Medicine, Stanford, California; Pergram, Mark D.; University of California, Los Angeles, Los Angeles, California; Ueland, Federick; University of Kentucky Medical Center, Lexington, Kentucky; Rodriguez, Michael; University Hospitals of Cleveland, Cleveland, Ohio; Malfetano, John H.; Albany Medical College, Albany, New York; Edwards, Robert P.; University of Pittsburgh, Pittsburgh, Pennsylvania; Rader, Janet; Washington University, Saint Louis, Missouri; Benigno, Benedict B.; Northside Hospital, Atlanta, Georgia; Lucci, Joseph T.; University of Texas Medical Branch, Galveston, Texas; Delmore, James E.; University of Kansas School of Medicine, Wesley Medical Center, Wichita, Kansas; Smith, Harriet O.; University of New Mexico School of Medicine, Albuquerque, New Mexico; Bristow, Robert E.; Johns Hopkins School of Medicine, Baltimore, Maryland; Abbas, Fouad; Sinai Hospital of Baltimore, Baltimore, Maryland; Fort, Giles; Woman's Hospital, Baton Rouge, Louisiana; Berchuck, Andrew; Duke University Medical Center; Coleman, Robert; University of Texas Southwestern Medical Center, Dallas, Texas; Rocereto, Thomas; University of Medicine and Dentistry of New Jersey, Robert Wood Johnson Medical School, Camden, New Jersey; Hall, James; Carolinas Medical Center, Charlotte, North Carolina; Holloway, Robert; Walt Disney Memorial Cancer Institute, Orlando, Florida; Garcia, Agustin; University of Southern California, Norris Cancer Hospital, Los Angeles, California; Lentz, Samuel Wake Forest University School of Medicine, Winston-Salem, North Carolina; Swensen, Ron; Loma Linda University Cancer Institute, Loma Linda, California; Horowitz, Ira; Emory University School of Medicine, Atlanta, Georgia; Kline, Richard and Burroff, Janet; Alton Ochsner Medical Foundation, New Orleans, Louisiana; Scudder, Sidney; University of California, Davis, Sacramento, California; Noubisi, Boniface; University of Florida, Gainesville, Florida; and Celano, Paul; Greater Baltimore Medical Center, Baltimore, Maryland; *A Phase II/III Trial of Chemotherapy Alone Versus Chemotherapy Plus SCH 58500 in Newly Diagnosed Stage III Ovarian and Primary Peritoneal Cancer Patients with ≥0.5 cm and ≤2 cm Residual Disease Following Surgery.* Sponsor: Schering Corporation

NIH/ORDA Receipt Date: 1-12-99. Not Selected for RAC Public Review: 6-2-99
Closed to enrollment: 3-30-01

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9901-281 (Closed) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Type 5/MART-1 Melanoma Antigen/gp 100 Melanoma Antigen/Subcutaneous Injection

Haluska, Frank; Harvard Medical School, Boston, Massachusetts and Nemunaitis, John J.; US Oncology, Dallas, Texas; *Phase I/II Trial of the Safety, Immunogenicity, and Efficacy of Autologous Dendritic Cells Transduced with Adenoviruses Encoding the MART-1 and gp100 Melanoma Antigens Administered With or Without Low Dose Recombinant Interleukin-2 (rIL-2) in Patients with Stage IV Melanoma*. Sponsor: Genzyme Molecular Oncology.

NIH/ORDA Receipt Date: 1-12-99. Not Selected for RAC Public Review: 3-30-99
Closed: 6-17-02

9901-282 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen/Intramuscular Injection

Eder, Joseph Paul; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase II Randomized Trial of Recombinant Fowlpox and Recombinant Vaccinia Virus Expressing PSA in Patients with Adenocarcinoma of the Prostate.* Sponsor: National Cancer Institute-Cancer Therapy Evaluation Program (NCI-CTEP).

NIH/ORDA Receipt Date: 1-12-99. Not Selected for RAC Public Review: 1-24-00

9901-283 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection

Small, Eric J.; University of California, San Francisco, San Francisco, California; *Phase I/II Study of a Prime-Boost Schedule of Human GM-CSF Gene Transduced Irradiated Prostate Allogeneic Cancer Cell Vaccines (Allogeneic Prostate GVAX^{TM}) in Hormone-Naive Prostate Cancer Patients.* Sponsor: Cell Genesys

NIH/ORDA Receipt Date: 1-22-99. Not Selected for RAC Public Review: 2-11-99
Notification from sponsor that trial is closed: 4-09-01

9902-284 (Open) Gene Therapy/Phase I/Monogenic Disease/Hemophilia A/In Vivo/Retrovirus/Factor VIII cDNA/Intravenous Infusion

Ragni, Margret V.; University of Pittsburgh, Pittsburgh, Pennsylvania; Lusher, Jeanne M.; Children's Hospital of Michigan, Detroit, Michigan; Powell, Jerry S.; University of California, Davis, Medical Center, Sacramento, California; White, Gilbert; University of North Carolina School of Medicine, Chapel Hill, North Carolina and Ewenstein, Bruce M.; Brigham and Women's Hospital, Boston, Massachusetts; *Phase I Multi-Center, Single Treatment Dose Escalation Study of Factor VIII Vector [HFVIII(V)] for Treatment of Severe Hemophilia A*. Sponsor: Chiron Corporation

NIH/ORDA Receipt Date: 2-5-99. Publicly Reviewed at the September 3, 1999 RAC meeting

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9902-285 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/In Vivo/Cationic Liposome Complex with DC-Chol/Epidermal Growth Factor Receptor Antisense/Intratumoral Injection

Grandis, Jennifer Rubin; University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *A Phase I Trial of Intratumoral Antisense EGFR DNA and DC-Chol Liposomes in Advanced Oral Squamous Cell Carcinoma*.

NIH/ORDA Receipt Date: 2-12-99. Not Selected for RAC Public Review: 3-5-99

9902-286 (Open) Gene Therapy/Phase I/Cancer/Lung, Head and Neck/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1005/HLA-B7/Beta 2-Microglobulin cDNA/Concurrent Interleukin-2 Injection/Direct Intratumoral Injection

Stopeck, Alison; Arizona Cancer Center, University of Arizona, Tucson, Arizona; *Phase I Study of HLA-B7/beta2M Plasmid DNA/DMRIE/DOPE Lipid Complex (Allovectin-7) by Direct Gene Transfer with Concurrent Low-Dose Subcutaneous IL-2 Protein Therapy as an Immunotherapeutic Regimen in Lung and Head and Neck Cancers*. Sponsor: Vical Inc.

NIH/ORDA Receipt Date: 2-16-99. Not Selected for RAC Public Review: 3-8-99

9902-287 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Bronchoalveolar Lavage

Schiller, Joan; University of Wisconsin, Madison, Wisconsin; and Carbone, David, P.; Vanderbilt University Medical Center, Nashville, Tennessee; *Phase I Pilot Trial of Adenovirus p53 in Bronchioloalveolar Cell Lung Carcinoma (BAC) Administered by Bronchoalveolar Lavage*. Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 2-16-99. Not Selected for RAC Public Review: 3-25-99

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9902-288 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection (Endobronchial or Percutaneous)

Schiller, Joan; University of Wisconsin, Madison, Wisconsin; *Phase I Pilot Trial of Adenovirus p53 and Radiotherapy on Non-Small Cell Lung Cancer*. Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 2-18-99. Not Selected for RAC Public Review: 6-24-99

9902-289 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cationic Liposome Complex/Alpha-1 Antitrypsin cDNA/Intranasal Administration

Brigham, Kenneth L.; Vanderbilt University School of Medicine, Nashville, Tennessee; *Expression of an Exogenously Delivered Human Alpha-1 Antitrypsin Gene in Nasal Epithelium of Patients with Cystic Fibrosis*.

NIH/ORDA Receipt Date: 2-19-99. Not Selected for RAC Public Review: 4-2-99
Closed to enrollment: 5-02-02

9902-290 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Particle Mediated Gene Transfer (Accel®)/Plasmid DNA/gp 100 cDNA/Granulocyte-Macrophage Colony Stimulating Factor cDNA

Albertini, Mark R.; University of Wisconsin, Madison, Wisconsin; *Phase I Trial of Immunization Using Particle-Mediated Transfer of Genes for GP-100 and GM-CSF into Uninvolved Skin of Patients with Melanoma*.

NIH/ORDA Receipt Date: 2-22-99. Not Selected for RAC Public Review: 3-15-99
3-29-00: Closed to accrual and treatment; follow-up will continue

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9902-291 (Open) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Fanconi Anemia Complementation Group A cDNA/Intravenous

Walsh, Christopher E.; The University of North Carolina at Chapel Hill, Chapel Hill, North Carolina; *Retroviral-Mediated Gene Transfer of the Fanconi Anemia Group A Gene into Hematopoietic Progenitor Cells of Group A Patients*.

NIH/ORDA Receipt Date: 2-22-99. Not Selected for RAC Public Review: 3-15-99

9902-292 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/gp 100 Melanoma Antigen/Intramuscular or Intravenous Injection

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Immunization of Patients with Metastatic Melanoma Using a Recombinant Fowlpox Virus Encoding a GP 100 Peptide Preceded by an Endoplasmic Reticulum Insertion Signal Sequence*. Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 2-24-99. Not Selected for RAC Public Review: 3-22-99

9902-293 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen/Intramuscular or Intradermal Injection

Kaufman, Howard; Albert Einstein College of Medicine, Bronx, New York; *Phase II Randomized Study of Vaccine Treatment of Advanced Prostate Cancer*. Sponsor: Eastern Cooperative Oncology Group

NIH/ORDA Receipt Date: 2-24-99. Not Selected for RAC Public Review: 8-13-99

9902-294 (Closed) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Administration

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts, Henry, Timothy D.; Hennepin County Medical Center, Minneapolis, Minnesota and Schatz, Richard A.; Scripps Clinic, La Jolla, California; *A Multicenter, Open-Label, Dose-Escalating Study of Intramyocardial Vascular Endothelial Growth Factor 2 (VEGF-2) Gene Therapy in Refractory Patients with Stable Exertional Angina Who Are Not Candidates for Revascularization Procedures*. Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 2-26-99. Not Selected for RAC Public Review: 6-11-99

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9903-295 (Withdrawn from RAC Review) Gene Therapy/Phase I/Monogenic Disease/Gyrate Atrophy/In Vitro/Autologous Keratinocytes/Retrovirus/Ornithine Aminotransferase (OAT) cDNA/Skin Patch Administration

Nussenblatt, Robert B.; National Institutes of Health, Bethesda, Maryland; *Phase I Study in the Safety and Efficacy of Transduced Keratinocytes for Possible Treatment of Gyrate Atrophy*.

NIH/ORDA Receipt Date: 3-4-99. Withdrawn from RAC review: 9-18-00

9903-296 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Gamma Interferon cDNA/Intratumoral Injection

Rosenblatt, Joseph D.; University of Rochester Medical Center, Rochester, New York; *Phase I Trial of Immunotherapy with Adenovirus-Interferon-Gamma (TG1041) in Patients with Malignant Melanoma*. Sponsor: Transgene, Inc.

NIH/ORDA Receipt Date: 3-10-99. Not Selected for RAC Public Review: 3-30-99
Closed by sponsor to further enrollment: 06-29-01

9903-297 (Closed) Gene Marking/Autoimmune Disease/Multiple Sclerosis/In Vitro/CD34+ Autologous Peripheral Blood/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Infusion

Krance, Robert; Baylor College of Medicine, Houston, Texas; *Intensive Immunosuppression Followed by Rescue with CD34 Selected, T Cell Depleted, Leukopheresis Products in Patients with Multiple Sclerosis*.

NIH/ORDA Receipt Date: 3-24-99. Not Selected for RAC Public Review: 4-21-99
Gene marking portion of this study has been removed: 3-26-03

9903-298 (Open) Gene Therapy/Phase II/Cancer/Ovarian/Pro-Drug/In Vivo/PA317/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intraperitoneal/Catheter

Link, Charles J. and Morrman, Donald; Human Gene Therapy Research Institute, Des Moines, Iowa; *A Phase II Trial of In Vivo Gene Therapy with the Herpes Simplex Thymidine Kinase for the Treatment of Ovarian Cancer*.

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99

9903-299 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts, Baumgartner, Iris; Bern University, Bern Switzerland and Olin, Jeffrey Wayne; Cleveland Clinic Foundation, Cleveland, Ohio; *A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with Moderate-Risk Critical Limb Ischemia*. Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99
Closed: 2-11-00

9903-300 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with High-Risk Critical Limb Ischemia*.

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99
Follow-up has been completed: 11-29-01

9903-301 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts, Baumgartner, Iris, Bern University, Bern Switzerland and Olin, Jeffrey Wayne, Cleveland Clinic Foundation, Cleveland, Ohio; *A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with High-Risk Critical Limb Ischemia*. Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99
Closed: 2-11-00

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9903-302 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Open-Label, Rescue-Therapy Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with Moderate-Risk or High-Risk Critical Limb Ischemia*. Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 3-26-99. Not Selected for RAC Public Review: 4-15-99
Follow-up is complete: 11-29-01

9903-303 (Closed) Gene Marking/Cancer/Neuroblastoma/Sarcoma/Retinoblastoma/In Vitro/CD34+ Autologous Peripheral Blood or Bone Marrow/Dihydrofolate Reductase cDNA/Intravenous Infusion

Cunningham, John M.; St. Jude Children's Research Hospital, Memphis, Tennessee; *Tumor Purging of Autologous Stem Cell Grafts in Children with High-Risk Solid Tumors: Transplantation of Retrovirally Marked Stem Cell Grafts Purified by CD34+ Antibody Selection and High-Speed Cell Sorting*.

NIH/ORDA Receipt Date: 3-29-99. Not Selected for RAC Public Review: 5-18-99
Closed: 11-4-02

9904-304 (Closed) Gene Therapy/Phase I/Cancer/Retinoblastoma/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection (Intraocular Tumor)

Hurwitz, Richard L.; Baylor College of Medicine, Houston, Texas; *Pediatric Phase I Study of AdV/RSV-TK Followed by Ganciclovir for Retinoblastoma*

NIH/ORDA Receipt Date: 4-1-99. Publicly Reviewed at the June 14, 1999 RAC meeting
Closed: 7-15-04

9904-305 (Open) Gene Therapy/Phase I/Cancer/Breast/Tumor Suppressor Gene/In Vitro/Autologous CD34+ Cells/Adenovirus/Serotype 5/p53 cDNA/Intravenous Infusion

Baynes, Roy D.; Karmanos Cancer Institute, Wayne State University, Detroit, Michigan; *A Phase I Study of Infused Mobilized, Autologous Peripheral Blood Progenitor Cells, Which Have Been Incubated with a Recombinant Adenovirus-Wild-Type p53 Construct (SCH 58500) to Purge Any Contaminating Breast Cancer Cells, As Stem Cell Support After High-Dose Chemotherapy in Patients with Breast Cancer Metastatic to Bone and Bone Marrow*.

NIH/ORDA Receipt Date: 4-5-99. Not Selected for RAC Public Review: 5-3-99

9904-306 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Prostate Specific Antigen/Intravenous

Vieweg, Johannes; Duke University Medical Center, Durham North Carolina; *Safety and Feasibility Study of Active Immunotherapy in Patients with Hormone Refractory Prostate Cancer Using Autologous Dendritic Cells Pulsed with RNA Encoding Prostate Specific Antigen, PSA*

NIH/ORDA Receipt Date: 4-6-99. Not Selected for RAC Public Review: 4-26-99
Closed to enrollment: 2-13-03

9904-307 (Closed) Gene Therapy/Phase I/Cancer/Cervical/Immunotherapy/In Vivo/Vaccinia Virus/Human Papilloma Virus E6 and E7/Interleukin-2/Intramuscular Injection

Kaufman, Raymond H.; Baylor College of Medicine, Houston, Texas; *Phase I Trial of Immunotherapy with MVA-HPV-IL2 (TG4001) in Women with Cervical Intraepithelial Neoplasia (CIN) Grade 3.* Sponsor: Transgene, Inc.

NIH/ORDA Receipt Date: 4-8-99. Not Selected for RAC Public Review: 4-26-99

9904-308 (Closed) Gene Therapy/Phase I/Cancer/Leukemia/Adoptive Immunotherapy/In Vitro/Donor CD8+ Lymphocytes/Retrovirus/Hygromycin Phosphotransferase-Herpes Simplex Thymidine Kinase Fusion Gene/Intravenous Infusion

Warren, Edus; Fred Hutchinson Cancer Research Center, Seattle, Washington; *Phase I Study of Adoptive Immunotherapy with Gene-Modified and Unmodified CD8+ Minor Histocompatibility (H) Antigen-Specific CTL Clones for Patients with Relapse of AML or ALL After Allogeneic Hematopoietic Stem Cell Transplant*.

NIH/ORDA Receipt Date: 4-13-99. Not Selected for RAC Public Review: 5-3-99
Participants will no longer receive gene-modified cells: 10-27-01

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9904-309 (Closed) Gene Therapy/Phase I/Cancer/Cervical Cancer/Immunotherapy/In Vivo/Vaccinia Virus/Human Papilloma Virus E6 and E7/Interleukin-2/Intramuscular Injection

Goff, Barbara A.; University of Washington School of Medicine, Seattle, Washington; *Phase I Trial of Immunotherapy with MVA-HPV-IL2 (TG4001) in Women with Advanced Cervical Carcinoma*. Sponsor: Transgene, Inc.

NIH/ORDA Receipt Date: 4-22-99. Not Selected for RAC Public Review: 5-12-99

9904-310 (Closed) Gene Marking/Osteodysplasia/In Vitro/Stromal Cells for Donor Bone Marrow/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Infusion

Horwitz, Edwin M.; St. Jude Children's Research Hospital, Memphis, Tennessee; *Stromal Therapy of Osteodysplasia After Allogeneic Bone Marrow Transplantation: A Phase I Study*.

NIH/ORDA Receipt Date: 4-22-99. Not Selected for RAC Public Review: 5-12-99
Closed to accrual, follow-up continues: 5-03-02

9904-311 (Open) Gene Marking/Cancer/Neuroblastoma/In Vitro/Autologous Cytotoxic T-Lymphocytes from Peripheral Blood/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Infusion

Nuchtern, Jed; Baylor College of Medicine, Houston, Texas; *Administration of Neomycin Resistance Gene Marked Neuroblastoma Specific Cytotoxic T-Lymphocytes to Patients with Relapsed/Resistant Neuroblastoma*.

NIH/ORDA Receipt Date: 4-30-99. Not Selected for RAC Public Review: 5-20-99

9905-312 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1102/Leuvectin/Interleukin-2 cDNA/Intratumoral Injection

Belldegrun, Arie; University of California, Los Angeles, Los Angeles, California; Klein, Eric; Cleveland Clinic Foundation, Cleveland, Ohio; Corman, John; VA Puget Sound Health Care System, Seattle, Washington; and Moul, Judd; Walter Reed Army Medical Center, Washington, DC; *Phase II Study Evaluating the Safety and Efficacy of Neoadjuvant Leuvectin Immunotherapy for the Treatment of Prostate Cancer*. Sponsor: Vical, Inc.

NIH/ORDA Receipt Date: 5-7-99. Not Selected for RAC Public Review: 5-27-99
Closed: 6-27-03; follow-up is continuing

9905-313 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/Vaccinia Virus/Tyrosinase cDNA/Intramuscular Injection

Topalian, Suzanne L.; National Institutes of Health, Bethesda, Maryland; *Immunization of Patients with Metastatic Melanoma Using Recombinant Fowlpox and Vaccinia Viruses Encoding the Tyrosinase Antigen*.

NIH/ORDA Receipt Date: 5-11-99. Not Selected for RAC Public Review: 6-1-99

9905-314 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Vaccinia Virus/B7.1 (CD80)/Intratumoral Injection

Kaufman, Howard L.; Columbia University, New York, New York; *A Phase I Trial of Intralesional RV-B7.1 Vaccine in the Treatment of Malignant Melanoma.* Sponsor: NCI-Cancer Therapy Evaluation Program (NCI-CTEP)

NIH/ORDA Receipt Date: 5-12-99. Not Selected for RAC Public Review: 7-23-99
Closed: 5-15-02; follow-up is continuing

9905-315 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Retrovirus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection

Small, Eric J.; University of California, San Francisco, San Francisco, California and Smith, David C.; University of Michigan, Ann Arbor, Michigan; *A Phase I/II Study of a Prime-Boost Schedule of Human GM-CSF Gene Transduced Irradiated Prostate Allogeneic Cancer Vaccine (Allogeneic Prostate GVAX ™) in Hormone-Refractory Prostate Cancer (G9803)*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 5-14-99. Not Selected for RAC Public Review: 6-4-99
Notification from sponsor that trial is closed: 4-09-01

9905-316 (Closed) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Percutaneous Cardiac Catheterization

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *Multicenter, Randomized, Single-Blind, Placebo-Controlled, Dose-Escalating Study of Intramyocardial Vascular Endothelial Growth Factor 2 (VEGF2) Gene Therapy Administered Using Percutaneous Cardiac Catheterization in Patients with Refractory and Stable Exertional Angina Who Are Not Candidates for Revascularization Procedures*. Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 5-21-99. Not Selected for RAC Public Review: 8-30-99
Follow-up is complete: 11-29-01

9905-317 (Open) Gene Therapy/Phase I/Monogenic Disease/Muscular Dystrophy/In Vivo/Adeno-Associated Virus/ α, β, γ, Δ-Sarcoglycan cDNA/Intramuscular Injection

Mendell, Jerry; Ohio State University, Columbus, Ohio; *Phase I Clinical Trial Utilizing Gene Therapy for Limb Girdle Muscular Dystrophy: α, β, γ or Δ-Sarcoglycan Gene Delivered with Intramuscular Instillations of Adeno-Associated Vectors*.

NIH/ORDA Receipt Date: 5-26-99. Publicly Reviewed at the September 2, 1999 RAC meeting

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9905-318 (Closed) Gene Therapy/Phase II/Cancer/Colon/Hepatic Metastasis/Tumor Suppressor Gene/n Vivo/Adenovirus/Serotype 5/p53 cDNA/Intrahepatic/Hepatic Artery/Bolus Infusion

Venook, Alan P. and Warren, Robert S. Warren; University of California, San Francisco, San Francisco, California; Lenz, Heinz-Josef; University of Southern California, Los Angeles, California; Ravikumar, Thanjavur S.; Montefiore Medical Center, Bronx, New York; Kardinal, Carl; Alton Ochsner Medical Foundation, New Orleans, Louisiana; Roh, Mark S.; Allegheny General Hospital, Pittsburgh, Pennsylvania; Kemeny, Margaret; Stony Brook University Hospital, Stony Brook, New York; Gold, Philip J.; University of Washington, Seattle, Washington; Staley III, Charles; Emory University School of Medicine, Atlanta, Georgia; McMasters, Kelly M.; University of Louisville, Louisville, Kentucky; Elias, Laurence; University of New Mexico School of Medicine, Albuquerque, New Mexico; and Amado, Rafael G.; University of California, Los Angeles, Los Angeles, California; *A Phase II Study of SCH 58500 in Combination with Chemotherapy Alone in Patients with Colorectal Cancer Metastatic to the Liver*. Sponsor: Schering Corporation.

NIH/ORDA Receipt Date: 5-26-99. Not Selected for RAC Public Review: 6-16-99
Notification from sponsor that study is closed to new enrollment at all sites: 3-29-01

9905-319 (Closed) Gene Therapy/Phase I/Cancer/Acute Leukemia/Immunotherapy/In Vitro/Autologous Bone Marrow Fibroblasts/Lethally Irradiated/Adenovirus/Serotype 5/Interleukin-2 cDNA/CD40 Ligand cDNA/subcutaneous Injection

Brenner, Malcolm; Baylor College of Medicine, Texas Children's Hospital, Baylor College of Medicine, Houston, Texas; *Treatment of High Risk Acute Leukemia with CD40 Ligand and IL-2 Gene Modified Autologous Bone Marrow Fibroblasts and Tumor Cells*.

NIH/ORDA Receipt Date: 5-26-99. Not Selected for RAC Public Review: 6-16-99
Closed to new enrollment: 6-21-06

9905-320 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Carcinoembryonic Antigen/Intravenous

Lyerly, H. Kim; Duke University Medical Center, Durham, North Carolina; *Pilot Study of CEA RNA-Loaded, FLT3 Ligand-Mobilized Peripheral Blood Antigen Presenting Cells for Patients with Metastatic Malignancies Expressing CEA.*

NIH/ORDA Receipt Date: 5-26-99. Not Selected for RAC Public Review: 9-23-99

9906-321 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/E. coli Cytosine Deaminase cDNA/5-Fluorocytosine/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection

Kim, Jae Ho; Henry Ford Health System, Detroit, Michigan; *A Phase I Study of E1B-Attenuated Replication Competent Adenovirus Vector-Mediated Intratumoral Administration of the E. coli Cytosine Deaminase/HSV-1 Thymidine Kinase Fusion Gene in Conjunction with Two Prodrugs, 5-Fluorocytosine and Ganciclovir for Patients with Local Recurrence of Prostate Cancer after Radiation Therapy.*

NIH/ORDA Receipt Date: 6-9-99. Not Selected for RAC Public Review: 6-29-99
Study is completed: 7-3-02

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9906-322 (Closed) Gene Therapy/Phase I/Alzheimer's Disease/In Vitro/Autologous Fibroblasts/Retrovirus/Nerve Growth Factor cDNA/Intracerebral Implantation

Tuszynski, Mark H.; University of California, San Diego, La Jolla, California; *A Phase I Study of NGF Ex Vivo Gene Therapy for Alzheimer's Disease*

NIH/ORDA Receipt Date: 6-15-99. Publicly Reviewed at the December 1999 RAC meeting.
Closed to enrollment: 6-18-02

9906-323 (Open) Gene Therapy/Phase II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Cationic Liposome Complex/DOTMA-Cholesterol/Interleukin-2 cDNA/Intratumoral Injection

Zarrabi, M. H.; Veterans Affairs Medical Center, Northport, New York; Biel, Merrill A.; Ear, Nose & Throat SpecialtyCare of Minnesota, P.A., Minneapolis, Minnesota; Krasnow, Steven; Veterans Affairs Medical Center, Washington, D.C.; Cornett, Patricia; University of California, San Francisco/Veterans Affairs Medical Center, San Francisco, California; Robbins, K. Thomas; University of Tennessee, Memphis, Tennessee; O'Malley, Bert W.; University of Maryland School of Medicine, Baltimore, Maryland; Kabbinavar, Fairooz; University of California, Los Angeles, Los Angeles, California; McCaffery, Thomas; University of South Florida, Tampa, Florida; and Cordero, Joehassin; Texas Tech University, Lubbock, Texas; *A Multi-Center, Open-Label, Study of the Safety and Efficacy of Multiple Intratumoral Injections of hIl-2 Plasmid (1.8 mg) Formulated with DOTMA/Cholesterol [Ratio 1:0.5 (-/+)] Liposomes in Patients with Unresectable or Recurrent/Refractory Squamous Cell Carcinoma of the Head and Neck*. Sponsor: Valentis, Inc.

NIH/ORDA Receipt Date: 6-17-99. Not Selected for RAC Public Review: 7-8-99

9906-324 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection

Butler, E. Brian and Aguilar-Cordova, Estuardo; Baylor College of Medicine, Houston, Texas; *Phase I-II Study Evaluating HSV-tk + Valacyclovir Gene Therapy in Combination with Radiotherapy for Prostate Cancer*.

NIH/ORDA Receipt Date: 6-22-99. Not Selected for RAC Public Review: 7-13-99

9906-325 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Interferon-Beta cDNA/Stereotactic Injection

Eck, Stephen L.; University of Pennsylvania, Philadelphia, Pennsylvania; *Treatment of Recurrent or Progressive Malignant Glioma with a Recombinant Adenovirus Expressing Human Interferon-Beta (H5.010CMVhIFN-β): A Phase I Trial*.

NIH/ORDA Receipt Date: 6-30-99. Not Selected for RAC Public Review: 7-21-99

9906-326 (Closed) Gene Therapy/Phase I/Cancer/Skin Metastasis/Immunotherapy/In Vivo/Plasmid DNA/Interleukin-12 cDNA/Intratumoral Injection

Mahvi, David M.; University of Wisconsin, Madison, Wisconsin; *Treatment of Spontaneous Tumor Metastases with IL-12 DNA: A Phase IB Trial*.

NIH/ORDA Receipt Date: 6-30-99. Not Selected for RAC Public Review: 7-21-99
Closed to accrual, follow-up continues: 10-28-03

9907-327 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1α/VP16 cDNA/Intramuscular Injection

Losordo, Douglas W.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; Chronos, Nicholas; Atlanta Cardiology Group, Saint Joseph's Hospital, Atlanta, Georgia; Deitcher, Steven; Cleveland Clinic Foundation, Cleveland, Ohio; Rajagopalan, Sanjay; University of Michigan; and Laird, John; Washington Hospital Center, Washington, DC; *A Phase I Double-Blind, Placebo Controlled, Escalating Dose, Multi-Center Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administered by Intramuscular Injection to Patients with Critical Limb Ischemia Who are Not Candidates for Surgical or Percutaneous Revascularization*. Sponsor: Genzyme Corporation.

NIH/ORDA Receipt Date: 7-6-99. Not Selected for RAC Public Review: 10-5-99
Study completed: 11-25-03

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9907-328 (Open) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1α/VP16 cDNA/Intramuscular Injection

Losordo, Douglas W.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; Chronos, Nicholas; Atlanta Cardiology Group, Saint Joseph's Hospital, Atlanta, Georgia; Deitcher, Steven; Cleveland Clinic Foundation, Cleveland, Ohio; Rajagopalan, Sanjay; University of Michigan; and Laird, John; Washington Hospital Center, Washington, DC; *A Phase I, Open-Label, Multi-Center Extension Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administered by Intramuscular Injection to Patients with Critical Limb Ischemia Who are Not Candidates for Surgical or Percutaneous Revascularization*. Sponsor: Genzyme Corporation.

NIH/ORDA Receipt Date: 7-6-99. Not Selected for RAC Public Review: 10-5-99

9907-329 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1α/VP16 cDNA/Intramuscular Injection

Losordo, Douglas W.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; Chronos, Nicholas; Atlanta Cardiology Group, Saint Joseph's Hospital, Atlanta, Georgia; Deitcher, Steven; Cleveland Clinic Foundation, Cleveland, Ohio; Rajagopalan, Sanjay; University of Michigan; and Laird, John; Washington Hospital Center, Washington, DC; *A Phase I, Open-Label, Escalating Dose, Multi-Center Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administered by Intramuscular Injection to Patients with Critical Limb Ischemia Who are Not Candidates for Surgical or Percutaneous Revascularization*. Sponsor: Genzyme Corporation.

NIH/ORDA Receipt Date: 7-6-99. Not Selected for RAC Public Review: 10-5-99
Enrollment complete: 11-25-03

9907-330 (Closed) Gene Therapy/Phase I/Cancer/CD20+ Lymphoma/In Vitro/Autologous T Lymphocytes/Plasmid DNA/Electroporation/CD20-Specific scFvFc-Zeta T Cell Receptor/Intravenous Infusion

Jensen, Michael; City of Hope National Medical Center, Duarte, California; *Pilot Phase I Study to Evaluate the Safety of Cellular Immunotherapy Using Genetically Modified Autologous CD20-Specific CD8+ T Cell Clones for Patients with Recurrent/Refractory CD20+ Lymphoma Undergoing Autologous Peripheral Blood Stem Cell Transplantation*.

NIH/ORDA Receipt Date: 7-8-99. Not Selected for RAC Public Review: 7-28-99
Accrual closed: 1-18-02

9907-331 (Withdrawn-replaced by protocol # 0004-393) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer/Antisense/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid DNA/Electroporation/TGF–β/Subcutaneous Injection

Gutheil, John C. and Fakhrai, Habib; Sharp HealthCare, Sidney Kimmel Cancer Center, San Diego, California; *Phase II Study of Antisense TGF-β +/- IL-2 Gene Transfected Allogeneic Tumor Cells as a Vaccine in Patients with Stage IIIB and IV Non-Small Cell Lung Cancer*. Sponsor: NovaRx Corporation.

NIH/ORDA Receipt Date: 7-8-99.

9907-332 (Closed) Gene Therapy/Phase I-II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Interleukin-12 cDNA/Intratumoral Injection

Colevas, Alexander Dimitrios; Dana-Farber Cancer Institute, Harvard Medical School, Boston, Massachusetts; *A Multi-Center, Open-Label, Multiple Administration, Rising Dose Study of the Safety, Tolerability, and Efficacy of IL-12 Gene Medicine in Patients with Unresectable or Recurrent/Refractory Squamous Cell Carcinoma of the Head and Neck (SCCHN)*. Sponsor: Valentis, Inc.

NIH/ORDA Receipt Date: 7-16-99. Not Selected for RAC Public Review: 8-5-99
Closed to enrollment: 10-13-00

9908-333 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD34+ Hematopoietic Stem Cells/Retrovirus/Transdominant Rev/Antisense Pol 1/Intravenous Infusion

Swindells, Susan; University of Nebraska Medical Center, Omaha, Nebraska; Scadden, David; Massachusetts General Hospital, Boston, Massachusetts; Holodniy, Mark; Veterans Affairs Palo Alto Health Care System, Palo Alto, California; and MacGregor, Rob Roy; University of Pennsylvania Hospitals, Philadelphia, Pennsylvania; *A Multicenter Evaluation of the Safety and Efficacy of Hematopoietic Stem Cells Transduced with RevM10polAS (RevM10polAS HSCIP) as Therapy for HIV-1 Infected Persons.* Sponsor: Systemix, Inc.

NIH/ORDA Receipt Date: 8-16-99. Not Selected for RAC Public Review: 9-3-99

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9908-334 (Under Review) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine cDNA/Ganciclovir/Intraperitoneal Injection

Alvarez, Ronald D.; Barnes, Mack N.; and Curiel, David T.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Study of FGF2-Fab' Modified Adenovirus Vector Mediated Intraperitoneal Delivery of Herpes Simplex Virus Thymidine Kinase (HSV-TK) Gene and Intravenous Ganciclovir in Previously Treated Ovarian and Extraovarian Patients.*

NIH/ORDA Receipt Date: 8-17-99. Review at a RAC meeting pending; investigators have requested postponement of public review.

9908-335 (Closed) Gene Therapy/Phase I/Immunotherapy/Cancer/Ovarian/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous or Intradermal Injection

Dranoff, Glenn; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Study of Vaccination with Lethally Irradiated, Autologous Ovarian Carcinoma Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor*

NIH/ORDA Receipt Date: 8-18-99. Not Selected for RAC Public Review: 9-8-99
Closed to accrual: 12-31-04

9908-336 (Closed) Gene Marking/Leukemia/In Vitro/CD 34+ Autologous Cord Blood Cells/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous

Croop, James and Cornetta, Kenneth.; Indiana University School of Medicine; and Kelly, Patrick; Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio; *Post-Transplant Infusion of Fibronectin-Assisted, Retroviral-Mediated Gene-Marked and Ex Vivo Expanded CD34+ Placental and Umbilical Cord Blood Cells*

NIH/ORDA Receipt Date: 8-19-99. Not Selected for RAC Public Review: 9-9-99
Closed: 5-24-05

9908-337 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Severe Combined Immune Deficiency due to adenosine Deaminase Deficiency/In Vitro/Autologous CD34+ Cells from Cord Blood or Bone Marrow/Retrovirus/Adenosine Deaminase cDNA/Intravenous Infusion

Kohn, Donald B.; University of at Los Angeles; Los Angeles, California; Brochstein, Joel; Hackensack University Medical Center, Hackensack, New Jersey; and Candotti, Fabio; National Institutes of Health; Bethesda, Maryland; *Transduction of CD34+ Cells from the Umbilical Cord Blood of Infants or the Bone Marrow of Children with Adenosine Deaminase (ADA)-Deficient Severe Combined Immunodeficiency (SCID)*

NIH/ORDA Receipt Date: 8-26-99. Publicly Reviewed at the March 2000 RAC meeting

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9909-338 (Open) Gene Therapy/Phase I/Cancer/Prostate/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p 16 cDNA/Intratumoral Injection

Gingrich, Jeffrey R.; University of Tennessee, Memphis, Tennessee; *A Tolerance and Efficacy Study of Neoadjuvant Intraprostatic GTx-001 Followed by Radical Prostatectomy in Patients with Locally Advanced Prostate Cancer*. Sponsor: Genotherapeutics, Inc.

NIH/ORDA Receipt Date: 9-2-99. Not Selected for RAC Public Review: 9-29-99

9909-339 (Open) Gene Therapy/Phase I-II/Cancer/Ovarian/Tumor Suppressor Gene/In Vivo/Retrovirus/BRCA1 Gene/Intraperitoneal Administration

Holt, Jeffrey T.; Vanderbilt University, Nashville, Tennessee, and Tait, David L.; East Carolina University, Greenville, North Carolina; *Ovarian Cancer Gene Therapy with BRCA1*.

NIH/ORDA Receipt Date: 9-13-99. Not Selected for RAC Public Review: 10-1-99

9909-340 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD34+ Hematopoietic Stem Cells/Retrovirus/Transdominant Rev/Antisense Pol 1/Intravenous Infusion

Carabasi, Mathew H.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I/II Study to Evaluate the Safety and Effectiveness of RevM10polAS HSCIP in Late-Stage AIDS Patients Given Intensive Myelosuppressive Conditioning*. Sponsor: Systemix Inc.

NIH/ORDA Receipt Date: 9-17-99. Not Selected for RAC Public Review: 10-7-99

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9909-341 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD34+ Cells/Retrovirus/Antisense TAT/Transdominant Rev cDNA/Intravenous

Tisdale, John; National Institutes of Health, Bethesda, Maryland; *Low Intensity Non-Myeloablative Preparative Conditioning Followed by Transplantation of Genetically Modified HLA-Matched Peripheral Blood Hematopoietic Precursor Cells (PBPC) for Hematologic Malignancies in HIV Positive Adults*

NIH/ORDA Receipt Date: 9-20-99.

9910-342 (Open) Gene Therapy/Phase I/Other/Ulcer/In Vivo/Adenovirus/Serotype 5/Platelet Derived Growth Factor (PDGF) cDNA/Intra-Ulcer Injection

Margolis, David J.; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *Phase I Trial to Evaluate the Safety of H5.020CMVPDGF-B for the Treatment of a Diabetic Insensate Foot Ulcer*. Sponsor: Institute for Human Gene Therapy, University of Pennsylvania

NIH/ORDA Receipt Date: 10-1-99. Publicly Reviewed at the December 1999 RAC meeting.

9910-343 (Open) Gene Therapy/Phase I/Other/Ulcer/In Vivo/Adenovirus/Serotype 5/Platelet Derived Growth Factor (PDGF) cDNA/Intra-Ulcer Injection

Margolis, David J.; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *Phase I Trial to Evaluate the Safety of H5.020CMVPDGF-B and Limb Compression Bandage for the Treatment of Venous Leg Ulcer (Trial A).* Sponsor: Institute for Human Gene Therapy, University of Pennsylvania

NIH/ORDA Receipt Date: 10-1-99. Publicly Reviewed at the December 1999 RAC meeting.

9910-344 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Adenovirus Type 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intratumoral Injection

Terris, Martha K.; Palo Alto Veterans Administration Medical Center, Stanford University, Palo Alto, California; *A Phase I/II Dose Finding Trial of the Intraprostatic Injection of Calydon CV787, a Prostate-Specific Antigen Cytolytic Adenovirus, in Patients with Locally Recurrent Prostate Cancer Following Definitive Radiotherapy.* Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 10-13-99. Not Selected for RAC Public Review: 11-2-99

9910-345 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Metastatic Prostate Cancer/Vector-Directed Cell Lysis/In Vivo/Adenovirus Type 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate-Specific Antigen/Intravenous Injection

Wilding, George; University of Wisconsin Comprehensive Cancer Center, Madison, Wisconsin; *A Phase I/II Dose Finding Trial of the Intravenous Injection of Calydon CV787, a Prostate-Specific Antigen Cytolytic Adenovirus, in Patients with Hormone Refractory Metastatic Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/ORDA Receipt Date: 10-13-99. Publicly Reviewed at the March 2000 RAC meeting

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9910-346 (Closed) Gene Therapy/Phase II/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration

Stewart, Duncan J.; St. Michael's Hospital, University of Toronto, Toronto, Canada; Buller, Christopher; University of British Columbia, Vancouver, British Columbia, Canada; Rivard, Alain; University of Montreal, Montreal, Canada; Gregoire, Jean C.; Montreal Heart Institute, Montreal, Canada; Page, Pierre; Hopital du Sacre-Coeur de Montreal, Montreal, Canada; Plante, Sylvain; Laval Hospital, Sainte-Foy, Canada; Archer, Stephen L.; University of Alberta, Alberta, Canada; Sullivan, John; QEII Health Science Center, Halifax, Canada ; Dangoisse, Vincent; Hopital Royal Victoria Hospital, Montreal, Canada; Ducas, John; University of Manitoba, Manitoba, Canada; Hilton, J. David; Victoria Heart Institute, Victoria, Canada; Cohen, Eric A. and Bhatnagar, Gopal; Sunnybrook & Women's College Health Sciences Centre, Toronto, Canada; Langlois, Yves; Jewish General Hospital, Montreal, Quebec; Curtis, Michael; Foothills Hospital/University of Calgary, Alberta, Canada; Arnold, J. Malcolm O.; University of Western Ontario, London, Ontario, Canada; Dib, Nabil; Arizona Heart Institute & Foundation; Rajakumar, A. R. J.; Royal University Hospital, Saskatoon, Canada; Frank, Michael; Evanston Northwestern Healthcare, Evanston, Illinois; Lowe, James E.; Duke University Medical Center, Durham, North Carolina; and Mendelsohn, Farrell O.; Cardiology, P.C., Birmingham, Alabama; *A Phase II, Randomized, Multicenter, 26-Week Study to Assess the Efficacy and Safety of CI-1023 Delivered Through Minimally Invasive Surgery Versus Maximum Medical Treatment in Patients with Severe Angina, Advanced Coronary Artery Disease, and No Options for Revascularization.* Sponsor: GenVec, Inc.

NIH/ORDA Receipt Date: 10-12-99. Not Selected for RAC Public Review: 11-5-99
Study complete: 2-04-03

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9910-347 (Withdrawn from RAC Review) Gene Therapy/Phase I/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration

Rosengart, Todd K.; Northwestern Healthcare, Northwestern University, Evanston, Illinois; *Assessment of Direct Administration Via Minimally Invasive Surgery of a Replication Deficient Adenovirus Vector (Ad_{CU}VEGF.1) Containing the VEGF cDNA to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease.* Sponsor: R. Crystal, Institute of Genetic Medicine, The New York Presbyterian Hospital-Weill College of Cornell University

NIH/ORDA Receipt Date: 10-14-99. Withdrawn from RAC review: 10-16-00

9910-348 (Withdrawn from RAC Review) Gene Therapy/Phase I/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration

Crystal, Ronald G.; Institute of Genetic Medicine, The New York Presbyterian Hospital-Weill College of Cornell University, New York, New York; *Assessment of Direct Administration Via Minimally Invasive Surgery of a Replication Deficient Adenovirus Vector (Ad_{CU}VEGF.1) Containing the VEGF cDNA to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease.*

NIH/ORDA Receipt Date: 10-14-99. Withdrawn from RAC review: 10-16-00

9910-349 (Withdrawn-replaced by protocol # 0010-427) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Sweat Duct Epithelium/Adenovirus/Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intradermal Administration

Crystal, Ronald G.; Institute of Genetic Medicine, The New York Presbyterian Hospital-Weill College of Cornell University, New York, New York; *Effect of Ad_{GV}CFTR.10 on the Cystic Fibrosis Phenotype*.

NIH/ORDA Receipt Date: 10-14-99.

9910-350 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Oncogene Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal Administration

Alberts, David S.; Arizona Cancer Center, University of Arizona, Tucson, Arizona; Wolf, Judith K.; University of Texas, M.D. Anderson Cancer Center, Houston, Texas; and Muntz, Howard; Virginia Mason Medical Center, Seattle, Washington; *A Phase I Dose Escalation Study of Intraperitoneal E1A-Lipid Complex (1:3) with Combination Chemotherapy in Women with Epithelial Ovarian Cancer.* Sponsor: Targeted Genetics Corporation

NIH/ORDA Receipt Date: 10-14-99. Not Selected for RAC Public Review: 11-3-99
No longer active: 11-22-02

9910-351 (Open) Gene Therapy/Phase II/Cancer/Angioendothelioma/Immunotherapy/In Vivo/Plasmid DNA/Polyvinlypyrrolidone (PVP)/Human Interferon-α cDNA/Intratumoral Injection

Baker, Laurence H.; University of Michigan Medical School, Ann Arbor, Michigan; *An Open-Label, Multiple Administration, Study of the Safety, Tolerability, and Efficacy of IFN–α Gene Medicine in Patients with Malignant Angioendothelioma.* Sponsor: Valentis, Inc.

NIH/ORDA Receipt Date: 10-19-99. Not Selected for RAC Public Review: 11-8-99

9910-352 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1102/Leuvectin/Interleukin-2 cDNA/Intratumoral Injection

Belldegrun, Arie; University of California, Los Angeles Medical Center, Los Angeles, California; Klein, Eric A.; Cleveland Clinic Foundation, Cleveland, Ohio; Corman, John; Virginia Mason Medical Center, Seattle, Washington and Moul, Judd; Walter Reed Army Medical Center, Washington, DC; *Phase I/II Study Evaluating the Safety and Efficacy of Leuvectin Immunotherapy for the Treatment of Locally Recurrent Prostate Cancer Following Radiation Therapy*. Sponsor: Vical Inc.

NIH/ORDA Receipt Date: 10-25-99. Not Selected for RAC Public Review: 11-12-99
Closed: 6-27-03; follow-up is continuing

9911-353 (Closed) Gene Therapy/Phase I-II/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/VEGF2-PAD-CL-009/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Annex, Brian H.; Durham VA Medical Center, Durham, North Carolina; *An Open Label Study of Intramuscular Vascular Endothelial Growth Factor-2 (VEGF-2) Gene Therapy in Patients with Critical Limb Ischemia.* Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 11-5-99.
Closed: 2-11-00

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9911-354 (Closed) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/VEGF2-CAD-CL-005/Vascular Endothelial Growth Factor (VEGF) cDNA/Percutaneous Cardiac Catheterization

Isner, Jeffrey M.; St. Elizabeth's Medical Center, Boston, Massachusetts; *A Placebo-Controlled, Dose-Escalating Study of Intramyocardial Vascular Endothelial Growth Factor 2 (VEGF2) Gene Therapy Administered Using Percutaneous Cardiac Catheterization in Patients with Class III or IV Angina.* Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/ORDA Receipt Date: 11-5-99. Not Selected for RAC Public Review: 1-28-00
Follow-up has been completed: 11-29-01

9911-355 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Anaplastic Astrocytoma/Immunotherapy/In Vitro/Allogeneic Fibroblasts/Lethally Irradiated/Plasmid DNA-Electroporation/IR850-170/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal Injection

Black, Keith L.; Cedars-Sinai Medical Center, Los Angeles, California; *A Phase I, Open Label, Safety Study of Allogeneic Glioblastoma Tumor Cell Lines (IR850) Mixed with Allogeneic Fibroblasts Genetically Modified to Secrete GM-CSF (IR851) in Patients with Glioblastoma Multiforme or Anaplastic Astrocytoma.* Sponsor: The Immune Response Corporation

NIH/ORDA Receipt Date: 11-12-99. Not Selected for RAC Public Review: 2-3-00

9911-356 (Closed) Gene Therapy/Phase I/Cancer/MUC-1 Expressing Tumors/Immunotherapy/In Vivo/Vaccinia Virus/TG4010.01/MUC-1/Interleukin-2/Intramuscular Injection

Figlin, Robert and Belldegrun, Arie; University of California, Los Angeles Medical Center, Los Angeles, California; *Phase I Bridging Trial of TG4010 as Antigen-Specific Immunotherapy in Patients with MUC-1 Positive Advanced Cancer*. Sponsor: Transgene, Inc.

NIH/ORDA Receipt Date: 11-16-99. Not Selected for RAC Public Review: 12-6-99
Completed: 9-11-00

9911-357 (Closed) Gene Therapy/Phase I-II/Cancer Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical VCL-1102/Leuvectin/Interleukin-2 cDNA/Intratumoral Injection

Galanis, Evanthia; Mayo Clinic, Rochester, Minnesota; and Hawkins, Michael; Washington Hospital Center, Washington Cancer Institute, Washington, D.C.; *Protocol for Retreatment with Leuvectin Immunotherapy for Cancer*. Sponsor: Vical Inc.

NIH/ORDA Receipt Date: 11-18-99. Not Selected for RAC Public Review: 12-8-99
Closed: 6-27-03

9911-358 (Never Implemented, RAC Reviewed with Recommendations; replaced by 0707-869) Gene Therapy/Phase I/Cancer/Liver/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection

Sung, Max W. and Woo, Savio L. C.; Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenoviral Vector Delivery of the Human Interleukin-12 cDNA by Intratumoral Injection in Patients with Metastatic Breast Cancer to the Liver*.

NIH/ORDA Receipt Date: 11-22-99. Publicly Reviewed at the March 2000 RAC meeting
Closed, never initiated: 6-22-07

9911-359 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Liver/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection

Sung, Max W. and Woo, Savio L. C.; Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenoviral Vector Delivery of the Human Interleukin-12 cDNA by Intratumoral Injection in Patients with Primary or Metastatic Colorectal Cancer to the Liver*.

NIH/ORDA Receipt Date: 11-22-99. Publicly Reviewed at the March 2000 RAC meeting

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9912-360 (Open) Gene Marking/Cancer/Melanoma/In Vitro/Syngeneic Peripheral Blood Lymphocytes/Retrovirus/Neomycin Phosphotransferase Gene/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma Using Cloned Lymphocytes following the Administration of a Nonmyeloablative but Lymphocyte Depleting Regimen.*

NIH/ORDA Receipt Date: 11-22-99. Not Selected for RAC Public Review: 12-31-99

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9912-361 (Closed) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/B7 (CD80), HLA-A1 or A2 cDNAs/Subcutaneous Injection

Podack, Eckhard R.; Cassileth, Peter A.; Sridhar, Kasi; and Savaraj, Niramol; University of Miami, Miami, Florida; *Elicitation of a Cellular Immune Response in Patients with Non-Small Cell Lung Cancer by Immunogenic Tumor Cell Vaccination - A Phase I Study***.**

NIH/ORDA Receipt Date: 12-1-99. Not Selected for RAC Public Review: 12-21-99
Data collection ongoing: 6-15-07

9912-362 (Open) Gene Marking/Cancer/Melanoma/In Vitro/Syngeneic Peripheral Blood Lymphocytes/Retrovirus/Neomycin Phosphotransferase Gene/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma Using Cloned Peripheral Blood Lymphocytes Sensitized In Vitro to the gp209-2M Immunodominant Peptide*

NIH/ORDA Receipt Date: 12-16-99. Not Selected for RAC Public Review: 1-7-00

9912-363 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Penile Carcinoma/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/Serotype 5/E. coli Cytosine deaminase Gene/Herpes Simplex Thymidine Kinase cDNA/Valacyclovir/Intratumoral Injection

Miles, Brian J.; Ayala, Gustavo; and Aguilar-Cordova, Estuardo; Baylor College of Medicine, Texas Children's Hospital, Houston, Texas; *Phase I Study of the Replication-Competent, E1B-Attenuated Adenovirus with a CD/HSV-1 TK Fusion Gene and the Oral Administration of Valacyclovir in Adults with Penile Cancer*.

NIH/ORDA Receipt Date: 12-20-99. Publicly Reviewed at the March 2000 RAC meeting

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9911-364 (Open) Gene Therapy/Phase I-II/Infectious Disease/Epstein-Barr Virus (EBV) and Cytomegalovirus Diseases/In Vitro/EBV and CMV-Specific Cytotoxic T Lymphocytes/Retrovirus/Cytomegalovirus pp65 Gene/Intravenous

Lucas, Kenneth G.; University of Alabama at Birmingham, Birmingham, Alabama; and Long, Gwynn Douglas; Duke University; Durham, North Carolina; *A Phase I-II Trial to Examine the Toxicity of CMV and EBV Specific Cytotoxic T Lymphocytes When Used for Prophylaxis Against EBV and CMV Disease in Recipients of CD34+ Selected/T Cell Depleted Stem Cell Transplants*.

NIH/ORDA Receipt Date: 11-26-99. Not Selected for RAC Public Review: 1-3-00

9912-365 (Open) Gene Therapy/Phase I-II/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous CD4+ T Cells/Retrovirus/CD4-Zeta Chimeric Receptor/Intravenous Infusion

Aronson, Naomi; Walter Reed Army Medical Center, Washington, D.C.; *A Phase I/II Study of the Safety, Survival, and Trafficking of Autologous CD4-zeta Gene-Modified T Cells With and Without Exogenous Interleukin-2 in HIV-Infected Patients*. Sponsors: University of Pennsylvania and Cell Genesys, Inc.

NIH/ORDA Receipt Date: 12-22-99. Not Selected for RAC Public Review: 4-14-00

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

9912-366 (Open) Gene Therapy/Phase III/Cancer/Squamous Cell Carcinoma of the Head and Neck (SCCHN)/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5 p53 cDNA/Intratumoral Injections

Hamm, John T.; University of Louisville, Norton Healthcare, Louisville, Kentucky; Haigentz, Missak; Montefiore Medical Center, Bronx, New York; Arquette, Mathew; Washington University School of Medicine, Barnard Cancer Center, St. Louis, Missouri; Cullen, Kevin J.; Georgetown University Medical Center, Washington, D.C.; Goodwin, W. Jarrard; University of Miami, Miami, Florida; Flood, William A.; The Milton S. Hershey Medical Center, Hershey, Pennsylvania; Yoo, George; University Health Center, Detroit, Michigan; Krempl, Greg; The University of Oklahoma, Oklahoma City, Oklahoma; Turpeenniemi-Hujanen, Taina; OULU University Hospital, Oulu, Finland; Kellokumpu-Lehtinen, Pirkko; Tampere University Hospital, Tampere, Finland; Brockstein, Bruce; Evanston Northwestern Healthcare, Evanston, Illinois; Cobb, Patrick; Billings Oncology Associates, Billings, Montana; Williamson, Stephen; University of Kansas Medical Center, Kansas City, Kansas; Burkey, Brian; The Vanderbilt Clinic/Vanderbilt University Medical Center, Nashville, Tennessee; Carrato Mena, Alfredo; Hospital General De Elche, Elche (Spain); Barnadas, Agusti; Hospital Universitari Germans Trias i Pujol, Barcelona, Spain; Trigo, J. Ma.; Hospital General Vall d'Hebron, Barcelona, Spain; Cortes-Funes, Hernan; Hospital 12 de Octubre, Madrid, Spain; Constenla, Manuel; Complejo Hospitalario De Pontevedra, Pontevedra, Spain; Sanchez, Emilio Fonseca; Hospital Clinico de Salamanca, Salamanca, Spain; Ruiperez, Andres Cervantes; Hospital Clinico Universitaro, Valencia, Spain; Guillem, Vicente; Instituto Valenciano de Oncologia, Valencia, Spain; Nathan, Cherie-Ann; Louisiana State University, Shreveport, Louisiana; Agarwala, Sanjiv; University of Pittsburgh, Pittsburgh, Pennsylvania; Rosen, Fred; The University of Illinois at Chicago, Chicago, Illinois; Breau, Randall; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Giguere, Jeffrey; Cancer Center of the Carolinas, Greenville, South Carolina; Trask, Douglas; University of Iowa Hospitals and Clinics, Iowa City, Iowa; Zitsch, Robert; University of Missouri Health Care, Columbia, Missouri; Hrushesky, William; J. M., Dorn Veterans Affairs Medical Center, Columbia, South Carolina; Clayman, Gary; University of Texas, M.D. Anderson Cancer Center, Houston, Texas; Guthrie, Troy H., Jr.; University of Florida, Jacksonville, Florida; Slolomon, William; SUNY Health Science Center at Brooklyn, Brooklyn, New York; Law, Amy; Geisinger Medical Center, Danville, Pennsylvania; Trask, Douglas; University of Iowa Health Care, Iowa City, Iowa; Nemechek, Andrew; Tulane University School of Medicine, New Orleans, Louisiana; Villaret, Douglas; University of Florida, Gainesville, Florida; Van Echo, David; University of Maryland School of Medicine, Baltimore, Maryland; McCaffrey, Thomas V.; H. Lee Moffitt Cancer Center and Research Institute, Tampa, Florida; Wheeler, Richard H.; Huntsman Cancer Institute, Salt Lake City, Utah; Chen, Amy; Emory University, Atlanta, Georgia; Gal, Thomas, Jr.; University of Washington Medical Center, Seattle, Washington; Axelrod, Rita; Thomas Jefferson University, Philadelphia, Pennsylvania; Kane, Madeleine; Denver Veteran's Administration Medical Center, Denver, Colorado; Levine, Marshall; Greater Baltimore Medical Center, Baltimore, Maryland; Weisman, Robert; University of California, San Diego, San Diego, California; and Bier-Laning, Carol M.; Edward Hines, Jr. VA Hospital, Hines, Illinois; *A Phase III Multi-Center, Open-Label, Randomized Study to Compare the Overall Survival and Safety of Bi-Weekly Intratumoral Administration of INGN 201 Versus Weekly Methotrexate in 240 Patients with Refractory Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: Aventis Pharmaceuticals - Gencell Division (formerly Rhone-Poulenc Rorer)

NIH/ORDA Receipt Date: 12-28-99. Publicly Reviewed at the March 2000 RAC meeting

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9912-367 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous

Vieweg, Johannes; Duke University Medical Center, Durham, North Carolina; *Active Immunotherapy of Metastatic Renal Cell Carcinoma Using Autologous Dendritic Cells Transfected with Autologous Renal Tumor RNA.*

NIH/ORDA Receipt Date: 12-28-99. Not Selected for RAC Public Review: 1-14-00
Closed to enrollment: 2-13-03

9912-368 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen/B7.1 (CD80)/Intramuscular or Intradermal Injection

Dahut, Bill; National Naval Medical Center, Bethesda, Maryland; and Gulley, James; National Institutes of Health, Bethesda, Maryland; *A Randomized Phase II Study of a PSA-Based Vaccine in Patients with Localized Prostate Cancer Receiving Standard Radiotherapy.*

NIH/ORDA Receipt Date: 12-29-99. Not Selected for RAC Public Review: 3-7-00
Closed: March 2006

0001-369 (Closed) Gene Therapy/Phase I/Immunotherapy/Cancer/Myelodysplasia or Acute Myelogenous Leukemia (AML)/In Vitro/Autologous Acute Myeloblastic Leukemia Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous or Intradermal Injection

DeAngelo, Daniel J.; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Study of Vaccination with Lethally Irradiated, Autologous Acute Myeloblastic Leukemia Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Human Granulocyte-Macrophage Colony Stimulating Factor in Patients with Advanced Myelodysplasia or Acute Myelogenous Leukemia.*

NIH/OBA Receipt Date: 1-3-00. Not Selected for RAC Public Review: 1-24-00
Closed to accrual: 12-27-06

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0001-370 (Closed) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Fanconi Anemia Complementation Group A and C cDNA/Intravenous

Croop, James M.; Indiana University School of Medicine, Indianapolis, Indiana; *Gene Therapy for Patients with Fanconi Anemia: A Pilot Study.*

NIH/OBA Receipt Date: 1-6-00. Not Selected for RAC Public Review: 2-4-00
Closed: 2-29-08

0001-371 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Hemophilia B/In Vivo/Adeno-Associated Virus/Factor IX Gene/Intrahepatic Artery Administration

Glader, Bertil; Stanford University, Stanford, California; and Konkle, Barbara A.; The Children's Hospital of Philadelphia, Philadelphia, Pennsylvania; *A Phase I Safety Study in Patients with Severe Hemophilia B (Factor IX Deficiency) Using Adeno-Associated Viral Vector to Deliver the Gene for Human Factor IX into the Liver.*

NIH/OBA Receipt Date: 1-7-00. Publicly Reviewed at the March 2000 RAC meeting
Closed to enrollment: 3-22-05

0001-372 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Hemophilia A/In Vivo/Helper-Dependent (Gutted) Adenovirus/Factor VIII cDNA/Intravenous Injection

White II, Gilbert; University of North Carolina School of Medicine, Chapel Hill, North Carolina; Thompson, Arthur; University of Washington, Seattle, Washington; and Gruppo, Ralph A.; Children's Hospital Medical Center, Cincinnati, Ohio; *A Phase 1, Single-Dose, Dose-Escalation Study of MiniAdFVIII Vector in Patients with Severe Hemophilia A.* Sponsor: Corautus Genetics, Inc. (formerly GenStar Therapeutics Corporation)

NIH/OBA Receipt Date: 1-12-00. Publicly Reviewed at the September 2000 RAC meeting
Closed: 2-13-03

0001-373 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen/B7.1 (CD80)/Intramuscular or Intradermal Injection

Arlen, Philip M.; National Naval Medical Center and National Institutes of Health, Bethesda, Maryland; *A Randomized Phase II Study of Either Immunotherapy with a Regimen of Recombinant Pox Viruses that Express PSA/B7.1 Plus Adjuvant GM-CSF and IL-2 or Hormone Therapy with Nilutamide in Patients with Hormone Refractory Prostate Cancer and No Radiographic Evidence of Disease.*

NIH/OBA Receipt Date: 1-10-00. Not Selected for RAC Public Review: 3-7-00

0001-374 (Withdrawn-replaced by 0007-407) Gene Therapy/Phase I/Coronary Artery Disease/In Vivo/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1α/VP16 cDNA/Cardiac Administration

A Phase I Open Label, Escalating Dose, Multi-Center Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administered by Intramyocardial Injection During Coronary Artery Bypass Grafting (CABG) Surgery in Patients with Areas of Viable and Underperfused Myocardium not Amenable to Bypass Grafting or Percutaneous Intervention and the related follow-up study *A Phase I Open Label, Multi-Center Extension Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administered by Intramyocardial Injection During Coronary Artery Bypass Grafting (CABG) Surgery in Patients with Areas of Viable and Underperfused Myocardium not Amenable to Bypass Grafting or Percutaneous Intervention*. Sponsor: Genzyme Corporation.

NIH/OBA Receipt Date: 1-13-00.

0001-375 (Withdrawn-replaced by protocol # 0010-425) Gene Therapy/Phase I/Other Disorders/Hip Fracture/In Vivo/Plasmid DNA/Collagen Sponge/Parathyroid Hormone cDNA/Bone Administration

A Phase I Safety, Tolerance and Pharmacokinetic Study of Mat-100 in Elderly Patients with Fresh Fracture of the Hip. Sponsor: Selective Genetics, Inc.

NIH/OBA Receipt Date: 1-13-00.

0001-376 (Open) Gene Therapy/Phase I/Cancer/Non-Hodgkin's Lymphoma/Chemoprotection/Fusion Gene of a Mutant Dihydrofolate Reductase and Cytidine Deaminase/In Vitro/Autologous Peripheral Blood CD34+ Cells/Retrovirus/Intravenous Infusion

Bertino, Joseph; Memorial Sloan Kettering Cancer Center, New York, New York; *A Gene Therapy Based Myeloprotection Strategy Using a Mutant Dihydrofolate Reductase - Cytidine Deaminase Fusion Gene for the Treatment of Refractory or Relapsed Non-Hodgkin's Lymphoma.*

NIH/OBA Receipt Date: 1-13-00. Not Selected for RAC Public Review: 2-3-00

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0001-377 (Withdrawn from RAC Review) Gene Therapy/Phase I/Monogenic Disease/Fabry Disease/In Vitro/Autologous Mesenchymal Stem Cells/Retrovirus/α-Galactosidase A cDNA/Immunoisolation Device/Subcutaneous Implantation

Medin, Jeffrey A.; University of Illinois at Chicago, Chicago, Illinois; *A Phase I Trial of Retroviral Transduction of Autologous Mesenchymal Stem Cells from Patients with Fabry Disease with Alpha-Galactosidase A cDNA and Implantation Via an Immunoisolation Device.* Sponsor: Osiris Therapeutics, Inc.

NIH/OBA Receipt Date: 1-13-00. Withdrawn from RAC review: 3-1-00

0002-378 (Open) Gene Therapy/Phase II/Cancer/Squamous Cell Carcinoma of the Head and Neck/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Interferon-α/Interleukin-12 cDNA/Intratumoral Injection

McQuone, Shelly J.; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Multicenter, Open-Label, Multiple Administration, Study of the Safety, Tolerability and Efficacy of IFNα/IL-12 Combination Gene Therapy in Patients with Squamous Cell Carcinoma of the Head and Neck (SCCHN).* Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 2-9-00. Not Selected for RAC Public Review: 8-8-00

0001-379 (Submission Not Complete) Gene Therapy/Phase I/Immunotherapy/Cancer/Colon/Adenovirus/Serotype 5/GA733-2 Antigen cDNA/Intradermal Injection

Eck, Stephen L.; University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *Phase I Trial of Intradermal Adenovirus GA733 Vaccine for Advanced Colorectal Cancer.*

NIH/OBA Receipt Date: 1-13-00.

0001-380 (Under Review) Gene Therapy/Phase I/Monogenic Disease/Amyotrophic Lateral Sclerosis/In Vivo/Adeno-Associated Virus/Excitatory Amino Acid Transporter 2 (EAAT2) cDNA/Percutaneous Cervical Injection

During, Matthew J. and Simeone, Frederick A.; Thomas Jefferson University, Philadelphia, Pennsylvania; *Clinical Trial in Amyotrophic Lateral Sclerosis Patients Using Gene Transfer of the EAAT2 Gene in the Cervical Spinal Cord.*

NIH/OBA Receipt Date: 1-13-00. Review at a RAC meeting pending; investigators have requested postponement of public review.

0001-381 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Canavan Disease/In Vivo/Adeno-Associated Virus/Aspartoacylase cDNA/Stereotactic Intracranial Administration

Leone, Paola and Feely, Michael; Cooper Health System, Camden, New Jersey; and Goldman, Warren H.; University of Medicine and Dentistry; Camden, New Jersey; *Gene Therapy of Canavan Disease Using AAV for Brain Gene Transfer*.

NIH/OBA Receipt Date: 1-13-00. Publicly Reviewed at the March 2000 RAC meeting

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0001-382 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous Neuroblastoma Cells/Lethally Irradiated/Adenovirus/Serotype 5/Interleukin-2 cDNA/Subcutaneous Injection

Russell, Heidi; Baylor College of Medicine, Houston, Texas; *A Pilot Study of Gene Modified Autologous Neuroblastoma Vaccine for the Post-Chemotherapy Treatment of High Risk Neuroblastoma.*

NIH/OBA Receipt Date: 1-14-00.
Closed to new accrual, follow-up continues: 3-17-04

0001-383 (Withdrawn) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Catheterization

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Phase IIb Multicenter, Randomized, Controlled Study of Direct Intramyocardial Injection of pVGI.1 (VEGF2) Versus Maximum Medical Therapy in Patients with Class III or IV Angina.* Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/OBA Receipt Date: 1-18-00.
Withdrawn from consideration, no individuals enrolled: 11-29-01

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0001-384 (Withdrawn) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Cardiac Catheterization

Isner, Jeffrey M.; Tufts University School of Medicine and St. Elizabeth's Medical Center, Boston, Massachusetts; *A Double-Blind, Placebo-Controlled, Continuation Study of Intramyocardial pVGI.1 (VEGF2) Administered by Percutaneous Cardiac Catheterization in Patients with Class III or IV Angina.* Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/OBA Receipt Date: 1-18-00.
Withdrawn from consideration, no individuals enrolled: 11-29-01

0001-385 (Closed) Gene Therapy/Phase I-II/Immunotherapy/Cancer/Non-Small Cell Lung Carcinoma (NSCLC)/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Subcutaneous Injection

Smith II, John W.; Providence Portland Medical Center, Portland, Oregon; Jablons, David; University of California, San Francisco, San Francisco, California; and Sterman, Daniel; University of Pennsylvania, Philadelphia, Pennsylvania; *Phase I/II Study of GM-CSF Gene-Modified Autologous Tumor Vaccines in Early and Advanced Stage Non-Small Cell Lung Cancer (NSCLC).* Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 1-20-00. Not Selected for RAC Public Review: 2-9-00
Closed to enrollment: 4-21-03

0001-386 (Closed) Gene Therapy/Phase II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Irradiated/Canarypox Virus/B7.1 (CD80) cDNA/Subcutaneous Injection

Antonia, Scott J.; H. Lee Moffitt Cancer Center, University of South Florida, Tampa, Florida; *Phase II Study of a B-7.1 Gene Modified Autologous Tumor Cell Vaccine and Systemic IL-2 for Patients with Stage IV Renal Cell Carcinoma.*

NIH/OBA Receipt Date: 1-24-00. Not Selected for RAC Public Review: 2-29-00
Closed: 06-28-12

0001-387 (Closed) Gene Therapy/Phase II/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration

Epstein, Stephen; Cardiovascular Research Institute, Washington, D.C.; Dib, Nabil; Arizona Heart Institute & Foundation, Phoenix, Arizona; Cohen, Barry M.; Morristown Memorial Hospital, Morristown, New Jersey; and Moses, Jeffrey W.; Lenox Hill Heart Hospital, New York, New York; *A Randomized, Double-Blind, Placebo-Controlled, Multicenter, 12-Week Follow-up, Pilot Study of the Tolerability and Feasibility of Administering $AD_{GV}VEGF_{121.10}$ (CI-1023) Via the Biosense Intramyocardial Injection Device to Patients with Advanced Coronary Artery Disease.* Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 1-27-00. Not Selected for RAC Public Review: 2-24-00
Closed: 8-18-03

0002-388 (Closed) Gene Therapy/Phase II/Other/Peripheral Arterial Disease/In Vivo/Ischemic Lower Limb/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Intramuscular Injection

Rajagopalan, Sanjay; University of Michigan Medical Center, Ann Arbor, Michigan; Chaikof, Elliot; Emory University School of Medicine, Atlanta, Georgia; Deitcher, Steven; The Cleveland Clinic Foundation, Cleveland, Ohio; Rhee, Robert Y.; University of Pittsburgh, Pittsburgh, Pennsylvania; Corson, John D.; The University of Iowa Hospitals and Clinics, Iowa City, Iowa; Mohler, Emile R.; University of Pennsylvania Health System, Philadelphia, Pennsylvania; Jaff, Michael; Cardiovascular Research Institute, Washington, DC; Lowman, Bruce G.; Watson Clinic Center for Research, Lakeland, Florida; Blebea, John; Penn State College of Medicine, Hershey, Pennsylvania; Hirsch, Alan T.; University of Minnesota, Minneapolis, Minnesota; Annex, Brian H.; Duke University Medical Center, Durham, North Carolina; Guzman, Raul; Vanderbilt University Medical Center, Nashville, Tennessee; Tenaglia, Alan; Tulane University Health Sciences Center, New Orleans, Louisiana; Azrin, Michael; University of Connecticut Health Center, Farmington, Connecticut; Gagne, Paul; New York University School of Medicine, New York, New York; Dib, Nabil; Arizona Heart Institute & Foundation, Phoenix, Arizona; Garza, Luis; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Hermiller, James; The Care Group, Indianapolis, Indiana; Mendelsohn, Farrell; Baptist Health System, Birmingham, Alabama; Miller, Julie M.; Johns Hopkins University, Baltimore, Maryland; Anderson, R. David; Sarasota Memorial Healthcare System, Sarasota, Florida; and Davies, Mark G.; University of Rochester Medical Center, Rochester, New York; *A Double-Blind, Randomized, Placebo-Controlled, Dose-Ranging, 26-Week Study to Assess the Safety and Efficacy of CI-1023 ($AD_{GV}VEGF_{121.10}$) in Peripheral Arterial Disease Patients with Severe, Disabling Intermittent Claudication*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 2-2-00. Not Selected for RAC Public Review: 3-27-00
Closed: 8-18-03

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0002-389 (Closed) Gene Therapy/Phase I/Cancer/Liver Metastasis of Colorectal Carcinoma/Immunotherapy/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Interleukin-2 cDNA/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection

Sung, Max W.; Mount Sinai School of Medicine, New York, New York; *Phase I/IB Trial of Combination Adenoviral Vector Delivery of the Human Recombinant Interleukin-2 Gene and the Herpes Simplex Virus Thymidine Kinase Gene by Intratumoral Injection and Followed by Intravenous Ganciclovir in Patients with Hepatic Metastases from Colorectal Cancer.*

NIH/OBA Receipt Date: 2-4-00. Not Selected for RAC Public Review: 3-10-00
No subjects received study agent: May 2000

0003-390 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/CD 34+ Hematopoietic Stem Cells/Retrovirus/Transdominant Rev/Intravenous Infusion

Kohn, Donald B.; Childrens Hospital Los Angeles, University of Southern California, Los Angeles, California; *Retroviral-Mediated Transfer of the RevM10 and FX Genes into CD 34+ Cells from the Bone Marrow of HIV-1 Infected Children*.

NIH/OBA Receipt Date: 3-1-00. Not Selected for RAC Public Review: 3-21-00

0002-391 (Closed) Gene Therapy/Phase II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Vical-1102/Leuvectin/Interleukin-2 cDNA/Intratumoral Injection/Vical Protocol VCL-1102-204

Thompson, John A.; University of Washington School of Medicine, Seattle, Washington; Hawkins, Michael; Washington Hospital Center, Washington Cancer Institute, Washington, D.C.; Figlin, Robert A.; University of California Los Angeles Medical Center, Los Angeles, California; Lee, Fa-Chyi; University of New Mexico Cancer Research and Treatment Center and University Hospital, Albuquerque, New Mexico; Ernstoff, Marc S.; Dartmouth Hitchcock Medical Center, Lebanon, New Hampshire; Bukowski, Ronald; The Cleveland Clinic Foundation, Cleveland, Ohio; Morse, Michael A.; Duke University Medical Center, Durham, North Carolina; and Amato, Robert; Baylor College of Medicine, Houston, Texas; *Phase II Study of Leuvectin in Patients with Metastatic Renal Cell Carcinoma*. Sponsor: Vical Inc.

NIH/OBA Receipt Date: 2-14-00. Not Selected for RAC Public Review: 3-6-00
Notification from sponsor that study is closed: 6-29-01.

0003-392 (Closed) Gene Therapy/Phase I-II/Cancer/Non-Hodgkin's B-Cell Lymphoma/Mantle Cell Lymphoma/Immunotherapy/In Vivo/Naked Plasmid DNA/Tumor Idiotype/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intramuscular and Intradermal Injections/Vical Protocol VCL-1642-101

Levy, Ronald; Stanford University School of Medicine, Stanford, California; *Phase I/II Study of Vaccine Therapy for B-Cell Lymphoma Utilizing Plasmid DNA Coding for Tumor Idiotype*. Sponsor: Vical Inc.

NIH/OBA Receipt Date: 3-17-00. Not Selected for RAC Public Review: 4-6-00

0004-393 (Open) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer/Antisense/In Vitro/Allogeneic Tumor Cells/Irradiated/Plasmid DNA-Electroporation/TGF-β/Subcutaneous Injection

Sobol, Robert and Bodkin, David; Sharp Health Care, Sidney Kimmel Cancer Center, San Diego, California; Batra, Raj K.; University of California, Los Angeles and West Los Angeles Veteran's Administration Medical Center, Los Angeles, California; Dillman, Robert O.; Hoag Cancer Center, Newport Beach, California; Nemunaitis, John J.; Mary Crowley Medical Research Center, Dallas, Texas; Schwarzenberger, Paul O.; Louisiana State University Medical Center, New Orleans, Louisiana; and Gurtler, Jayne S.; Metairie, Louisiana; *Phase II Study of a TGF-β2 Antisense Gene Modified Allogeneic Tumor Cell Vaccine in Patients with Stages II-IV Non-Small Cell Lung Cancer*. Sponsor: NovaRx

NIH/OBA Receipt Date: 4-3-00. Not Selected for RAC Public Review: 5-2-00

0005-394 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/Tyrosinase cDNA/Intramuscular Injection

Wolchok, Jedd; Memorial Sloan-Kettering Cancer Center, New York, New York; *Vaccination of AJCC Stage III and IV Melanoma Patients with Human and Mouse Tyrosinase DNA Vaccines: A Phase I Trial to Assess Safety and Immune Response.*

NIH/OBA Receipt Date: 5-1-00. Not Selected for RAC Public Review: 5-19-00
Closed to accrual: 5-25-04

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0005-395 (Closed) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Type 5/MART-1 Melanoma Antigen/gp100 Melanoma Antigen/Intradermal Injection

Haluska, Frank G; Harvard Medical School, Boston, Massachusetts; Cunningham, Charles; US Oncology, Dallas, Texas; Ernstoff, Marc; Dartmouth Hitchcock Medical Center, Lebanon, New Hampshire; and Richards, Jon M.; Oncology Specialists, S. C., Chicago, Illinois; *A Phase I/II Trial Investigating the Safety and Immunogenicity of Adenoviruses Encoding the Melan-A/MART-1 and gp100 Melanoma Antigens Administered Intradermally to Patients with Stage II-IV Melanoma*. Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 5-1-00. Not Selected for RAC Public Review: 9-14-00
Closed to new enrollment, follow-up is ongoing: 7-21-03

0005-396 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Colon Carcinoma (Hepatic Metastasis)/Herpes Simplex Virus Type 1/Tumor Lysis/Intrahepatic Artery Administration

Fong, Yuman; Memorial Sloan Kettering Cancer Center, New York, New York; *A Phase I, Open -Label, Dose-Escalating Study of the Safety, Tolerability, and Anti-tumor Activity of a Single Intrahepatic Injection of a Genetically Engineered Herpes Simplex Virus, NV1020, in Subjects with Adenocarcinoma of the Colon with Metastasis to the Liver* and the associated, long-term follow-up protocol: *Long-Term Follow-Up of the Safety and Survival of subjects with Adenocarcinoma of the Colon with Metastasis to the Liver Who Enrolled in a Phase I Dose-Escalating Study Evaluating a Genetically Engineered Herpes Simplex Virus, NV1020.* Sponsor: NeuroVir Therapeutics, Inc.

NIH/OBA Receipt Date: 5-2-00. Publicly Reviewed at the June 2000 RAC meeting
Enrollment completed: November 2002

0005-397 (Closed) Gene Therapy/Phase I/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration (Catheter)

Sanborn, Timothy A.; Joan and Sanford I. Weill Medical College, Cornell University, New York, New York; *A Feasibility Study of Catheter-Based Administration of a Replication Deficient Adenovirus Vector (Ad_{CU}VEGF.1) to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease.* Sponsor: R. Crystal, M.D.

NIH/OBA Receipt Date: 5-3-00. Not Selected for RAC Public Review: 5-23-00
Closed, never initiated, no individuals enrolled: 8-22-03

0005-398 (Replaced by 0807-929) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/Tropism-Modified Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Somatostatin Receptor cDNA/Ganciclovir/Intraperitoneal Injection

Barnes, Mack N.; University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Study of a Tropism Modified Adenovirus Vector for Intraperitoneal Delivery of Therapeutic Genes in Ovarian and Extraovarian Cancer Patients*.

NIH/OBA Receipt Date: 5-3-00. Publicly Reviewed at the December 2000 RAC meeting

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0005-399 (Closed) Gene Therapy/Phase I/Cancer/Solid Tumors/Immunotherapy/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection

Guha, Chandan and Mani, Sridhar; Albert Einstein College of Medicine, Bronx, New York; Kenady, Daniel E; University of Kentucky Medical Center, Lexington, Kentucky; Nemunaitis, John; US Oncology, Dallas, Texas; Richards, Donald A.; Tyler Cancer Center, Tyler, Texas; and Rosemurgy, Alexander; University of South Florida, Tampa, Florida; *An Open-Label, Phase I, Dose-Escalation Study of Tumor Necrosis Factor-alpha (TNFerade™ Biologic) Gene Therapy with Radiation Therapy for Locally Advanced, Recurrent, or Metastatic Solid Tumors*. Sponsor: GenVec

NIH/OBA Receipt Date: 5-3-00. Not Selected for RAC Public Review: 5-23-00
Closed: January 2003

0005-400 (Closed) Gene Therapy/Phase I/Cancer/Lymphoma/Chemoprotection/In Vitro/CD34+ Autologous Peripheral Blood Cells/Retrovirus/Multi-Drug Resistance-1 cDNA/Intravenous Infusion

Becker, Pamela S.; University of Massachusetts Memorial Health Care, Worcester, Massachusetts; *Transfer of the Multidrug Resistance Gene, MDR-1, to Hematopoietic Progenitors from Patients with High Risk Lymphoma.*

NIH/OBA Receipt Date: 5-3-00. Not Selected for RAC Public Review: 6-5-00

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0005-401 (Open) Gene Therapy/Phase II/Cancer/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous Leukemic Cells/Adenovirus/Serotype 5/CD154 cDNA/Intravenous Infusion

Gribben, John; Dana-Farber Cancer Institute, Boston, Massachusetts; and Millard, Frederick; University of California-San Diego Medical Center, San Diego, San Diego, California; *Open-Label, Multicenter, Phase II Study of Autologous Ad-CD154 Expressing Transduced CLL Cells in B Cell Chronic Lymphocytic Leukemia Subjects Enrolled in Two Parallel Arms*. Sponsor: Tragen Pharmaceuticals (formerly Immunogenex, Inc.)

NIH/OBA Receipt Date: 5-3-00. Not Selected for RAC Public Review: 5-23-00

0006-402 (Closed) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Plasmid DNA/Electroporation/CE7R-Specific scFvFc-Zeta T Cell Receptor/Intravenous Infusion

Jensen, Michael; City of Hope National Medical Center, Duarte, California; *Phase I Study to Evaluate the Safety of Cellular Immunotherapy for Recurrent/Refractory Neuroblastoma Using Genetically-Modified Autologous CD8+ T Cell Clones*.

NIH/OBA Receipt Date: 6-2-00**.** Not Selected for RAC Public Review: 6-22-00
Closed to enrollment: 1-4-02

0006-403 (Closed) Gene Therapy/Phase IIb/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration

Iskandrian, Ami E.; University of Alabama at Birmingham, Birmingham, Alabama; Churchill, David; North West Arkansas Heart and Vascular Center, Fayetteville, Arkansas; Gammon, Roger S.; Austin Heart, P.A., Austin, Texas; Ghali, Jalal K.; Cardiac Centers of Louisiana, LLC, Shreveport, Louisiana; Grines, Cindy L.; William Beaumont Hospital, Royal Oak, Michigan; Helmer, Gregory A.; Minnesota Heart Clinic, P. A., Edina, Minnesota; Kleiman, Neal S.; Baylor College of Medicine, Houston, Texas; Rade, Jeffrey J.; The Johns Hopkins Hospital, Baltimore, Maryland; Rowe, Steven K.; Heartland Health Center, St. Joseph, Missouri; Watkins, Matthew W.; Fletcher Allen Health Care, Burlington, Vermont; and Uretsky, Barry; University of Texas, Galveston, Galveston, Texas; *A Randomized, Double-Blind, Placebo Controlled Study to Evaluate the Effect of Ad5FGF-4 on Myocardial Perfusion Defect Size and Safety in Patients with Stable Angina.* Sponsor: Cardium Therapeutics

NIH/OBA Receipt Date: 6-5-00. Not Selected for RAC Public Review: 8-18-00
Completed: 09-03

0006-404 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase II/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) cDNA/Aerosol Administration

Moss, Richard B.; Stanford University School of Medicine, Palo Alto, California; Waltz; David, Children's Hospital, Boston, Massachusetts; Rodman, David; University of Colorado Health Sciences Center, Denver, Colorado; Spencer, L. Terry; Virella-Lowell, Isabel; Brantly, Mark; and Flotte, Terry; University of Florida, Gainesville, Florida; Zeitlin, Pamela; Johns Hopkins University, Baltimore, Maryland; Aitken, Moira; University of Washington, Seattle, Washington; Milla, Carlos; University of Minnesota, Minneapolis, Minnesota; and Clancy, John Paul; University of Alabama at Birmingham, Birmingham, Alabama; *A Multicenter, Double-Blind, Placebo-Controlled, Phase II Study of Aerosolized AAVCF in Cystic Fibrosis Patients with Mild Lung Disease.* Sponsor: Targeted Genetics

NIH/OBA Receipt Date: 6-12-00. Publicly Reviewed at the September 2000 RAC meeting
Enrollment is complete: 10-3-02

0006-405 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD 80)/ICAM-1/LFA-3/Intramuscular Or Intradermal Injection

Marshall, John L.; Georgetown University Medical Center, Washington, D.C.; *A Phase I Study of Sequential Vaccinations with Fowlpox-CEA(6D)-TRICOM (B7.1/ICAM-1/LFA-3) Alone, OR in Combination with Vaccinia-CEA(6D)-TRICOM, and the Role of GM-GSF, in Patients with CEA Expressing Carcinomas.*

NIH/OBA Receipt Date: 6-12-00. Not Selected for RAC Public Review: 11-7-00

0006-406 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Peripheral Artery Disease/Anemia of End Stage Renal Disease (ESRD)/In Vitro/Autologous Vascular Smooth Muscle Cells/Retrovirus/Erythropoietin (EPO) cDNA/Vascular Grafts Lined with Transduced Smooth Muscle Cells

Muczynski, Kimberly A. and Osborne, William R. A.; University of Washington School of Medicine, Seattle, Washington; *Erythropoietin Administration in Hemodialysis Patients Using Vascular Grafts Lined with Transduced Smooth Muscle Cells.*

NIH/OBA Receipt Date: 6-13-00. Publicly Reviewed at the September 2000 RAC meeting

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0007-407 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Coronary Artery Disease/In Vivo/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1α/VP16 cDNA/Cardiac Administration/CAD-HIF-004-99

Rosengart, Todd K; Northwestern University Medical School, Evanston, Illinois; McCurry, Kenneth; University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; Cmolik, Brian L.; University Hospitals of Cleveland, Cleveland, Ohio; Landolfo, Kevin P.; Duke University Medical Center, Durham, North Carolina; Dullum, Mercedes; Washington Hospital Center, Washington, DC; Lattouf, Omar; Emory University School of Medicine, Atlanta, Georgia; Fontana, Gregory; Cedars-Sinai Medical Center, Beverly Hills, California; Chronos, Nicolas; Atlanta Cardiology Research Institute, Atlanta, Georgia; Henry, Timothy; Minneapolis Heart Institute Foundation, Minneapolis, Minnesota; and DiScipio, Anthony; Dartmouth-Hitchcock Medical Center Hanover, New Hampshire; *A Phase I, Double-blind, Placebo Controlled, Escalating Dose, Multi-center Study of Ad2/Hypoxia Inducible Factor (HIF)-1α/VP16 Gene Transfer Administration by Intramyocardial Injection During Coronary Artery Bypass Grafting (CABG) Surgery in Patients with Areas of Viable and Underperfused Myocardium not Amenable to Bypass Grafting or Percutaneous Intervention.* Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 7-31-00. Publicly Reviewed at the September 2000 RAC meeting
Closed to enrollment: 1-28-04

0007-408 (Closed) Gene Therapy/Phase I-II/Cancer/B-Cell Chronic Lymphocytic Leukemia/Immunotherapy/In Vivo/Naked Plasmid DNA/Tumor Idiotype/Intramuscular Injection

Garcia-Manero, Guillermo; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I/II Study of Idiotypic Vaccination for Chronic Lymphocytic Leukemia Using a Genetic Approach.*

NIH/OBA Receipt Date: 7-31-00. Not Selected for RAC Public Review: 8-18-00
Closed to accrual, follow-up continues: 11-9-04

0007-409 (Open) Gene Therapy/Phase I/Cancer/Lung Cancer/Immunotherapy/In Vivo/Cationic Liposome Complex/Interleukin-2 cDNA/Intravenous Injection

Hainsworth, John Daniel; Sarah Cannon Cancer Center, Centennial Medical Center, Nashville, Tennessee; and Antonia, Scott; H. Lee Moffitt Cancer Center, Tampa, Florida; *A Phase I, Multi-Center, Open-Label, Dose-Escalation Study of the Safety and Tolerability of Intravenously Administered VLTS-587 in Patients with Solid Tumors and the Presence of Metastases or Primary Cancer in the Lungs.* Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 7-28-00. Not Selected for RAC Public Review: 9-7-00

0008-410 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous

Vieweg, Johannes; Duke University, Durham, North Carolina; *A Safety and Feasibility Study of Active Immunotherapy in Patients with Metastatic Prostate Carcinoma Using Stably Matured Dendritic Cells Transfected Amplified Autologous Tumor RNA.*

NIH/OBA Receipt Date: 8-22-00. Not Selected for RAC Public Review: 9-12-00
Closed to enrollment: 2-13-03

0009-411 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Disorders/Restenosis/In Vivo/Vascular Smooth Muscle Cells/Cationic Liposome Complex/Inducible Nitric Oxide Synthase (iNOS) cDNA/Barath® Intramural Local Drug Delivery Device (Infiltrator®)

Kuntz, Richard E.; Brigham and Women's Hospital, Boston, Massachusetts; *Restenosis Gene Therapy Trial - Phase I Study (REGENT I)*. Sponsor: Cardion AG

NIH/OBA Receipt Date: 9-20-00. Publicly Reviewed at the December 2000 RAC meeting
Closed: 10-01 (no individuals enrolled)

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0009-412 (Open) Gene Therapy/Phase III/Cancer/Squamous Cell Carcinoma of the Head and Neck (SCCHN)/Tumor Suppressor Gene/In Vivo/Adenovirus Serotype 5/p53 cDNA/Intratumoral Injections [INGN 201 (Ad5CMV-p53)-T302]

Haigentz, Missak; Montefiore Medical Center, Albert Einstein College of Medicine of Yeshiva University, Bronx, New York; Nemunaitis, John J.; Mary Crowley Medical Research Center, Dallas, Texas; Hamm, John T.; Louisville Oncology, Norton Healthcare, Inc., Louisville, Kentucky; Spiro, Jeffrey; University of Connecticut Health Center, Farmington, Connecticut; Van Echo, David; University of Maryland, Baltimore, Maryland; Yoo, George; Wayne State University, Detroit, Michigan; Cobb, Patrick; Billings Oncology Associates, Billings, Montana; Brockstein, Bruce; Evanston Hospital, Evanston, Illinois; Flood, William; The Milton S. Hershey Medical Center, Hershey, Pennsylvania; Krempl, Greg; University Hospital, Oklahoma City, Oklahoma; Goodwin, W. Jarrard; University of Miami Hospital and Clinics, Miami, Florida; Trask, Douglas; University of Iowa Hospitals and Clinics, Iowa City, Iowa; Zitsch, Robert; University of Missouri Health Care, Columbia, Missouri; Breau, Randall; University of Arkansas for Medical Sciences, Little Rock, Arkansas; Cullen, Kevin; Georgetown University Medical Center, Washington, D.C.; Hrushesky, William; J. M., Dorn Veterans Affairs Medical Center, Columbia, South Carolina; Clayman, Gary; University of Texas, M.D. Anderson Cancer Center, Houston, Texas; Nemechek, Andrew; Tulane Cancer Center, New Orleans, Louisiana; Guthrie, Troy H., Jr.; University of Florida, Jacksonville, Florida; Trask, Douglas; University of Iowa Health Care, Iowa City, Iowa; Arquette, Matthew; Washington University School of Medicine, St. Louis, Missouri; Villaret, Douglas; University of Florida, Gainesville, Florida; Levine, Marshall; Greater Baltimore Medical Center, Baltimore, Maryland; Kane, Madeleine; University of Colorado Health Sciences Center, Denver, Colorado; Arseneau, James C.; New York Oncology Hematology, P.C., Albany, New York; Berman, Barry S.; Cancer Centers of Florida, Orlando, Florida; Lydiatt, Daniel; Methodist Hospital, Omaha, Nebraska; McGuire, Elizabeth; New Mexico VA Health Care System, Albuquerque, New Mexico; Raju, Robert; Dayton Oncology & Hematology, PA, Kettering, Ohio; Richards, Donald; Tyler Cancer Center, Tyler, Texas; and Weisman, Robert; University of California, San Diego, La Jolla, California; *A Phase III, Multi-Center, Open-Label, Randomized Study to Compare the Effectiveness and Safety of Intratumoral Administration of INGN 201 in Combination with Chemotherapy Versus Chemotherapy Alone in 288 Patients with Recurrent Squamous Cell Carcinoma of the Head and Neck (SCCHN)*. Sponsor: Aventis Pharmaceuticals - Gencell Division

NIH/OBA Receipt Date: 9-22-00. Not Selected for RAC Public Review: 10-13-00

0009-413 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/Tyrosinase cDNA/Intra-lymphnodal Injection

Weber, Jeffrey; Keck/USC School of Medicine, USC Norris Center and Hospital, Los Angeles, California; Smith II, John W.; Earl A. Chiles Research Institute Providence Portland Medical Center, Portland, Oregon; and Johnson, Denise; Stanford University, Stanford, California; *A Phase I Dose Ranging Safety Study Using Intra-Nodal Delivery of a Plasmid DNA (Synchrotope TA2M) in Adult Stage IV Melanoma Patients*.

NIH/OBA Receipt Date: 9-29-00. Not Selected for RAC Public Review: 11-7-00
Study complete: 4-4-02

0010-414 (Closed) Gene Therapy/Phase I-II/Cancer/Hepatocellular Carcinoma/In Vitro/Autologous Dendritic Cells/Adenovirus/Alpha Fetoprotein/Intravenous Infusion

Economou, James S.; Glapsy, John A.; and McBride, William H.; UCLA Medical Center, Los Angeles, California; *A Phase I/II Trial Testing Alpha-Fetoprotein (AFP) Genetic Immunization in Hepatocellular Carcinoma*.

NIH/OBA Receipt Date: 10-2-00. Not Selected for RAC Public Review: 10-23-00
No longer active: 11-15-07

0010-415 (Open) Gene Therapy/Phase II/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chl-DOPE/E1A/Intraperitoneal Administration

Ueno, Naoto; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase II Study of Intraperitoneal E1A-Lipid Complex for Patients with Advanced Epithelial Ovarian Cancer without HER-2/neu Overexpression*. Sponsor: Targeted Genetics Corporation

NIH/OBA Receipt Date: 10-6-00. Not Selected for RAC Public Review: 11-2-00

0010-416 (Closed) Gene Therapy/Phase II/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chl-DOPE/E1A/Intraperitoneal Administration

Ueno, Naoto; University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase II Study of Intraperitoneal E1A-Lipid Complex for Patients with Advanced Epithelial Ovarian Cancer with HER-2/neu Overexpression*. Sponsor: Targeted Genetics Corporation

NIH/OBA Receipt Date: 10-6-00. Not Selected for RAC Public Review: 11-2-00
No longer active: 11-25-02

0010-417 (RAC Reviewed with Recommendations; Withdrawn, Replaced by 0503-701) Gene Therapy/Phase I/Cancer/Colorectal/Hepatic or Pulmonary Metastasis/Dominant Negative Mutation/In Vivo/Retrovirus/dnG1 Cyclin/Peripheral Arterial Infusion

Lenz, Heinz-Josef; Norris Cancer Center, University of Southern California, Los Angeles, California; *Tumor Site Specific Phase I Evaluation of Safety and Efficacy of Infusion of a Matrix-Targeted Retroviral Vector Bearing a Dominant Negative Cyclin G1 (dnG1) Construct as Intervention for Metastatic Colorectal Carcinoma.*

NIH/OBA Receipt Date: 10-13-00. Publicly Reviewed at the December 2000 RAC meeting
Closed, never initiated: 03-9-05

0010-418 (Open) Gene Therapy/Phase II/Cancer/Prostate/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Percutaneous Transperineal Intraprostatic Injection

Pollack, Alan; The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Randomized Phase II Study of Ad5CMV-p53 plus Radioactive Seed Implant vs Seed Implant Alone for PSA Relapse after External Beam Radiotherapy for Prostate Cancer*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 10-16-00. Not Selected for RAC Public Review: 11-7-00

0010-419 (Open; RAC Reviewed with Recommendations) Gene Therapy/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/fhVII/hFc cDNA/Intratumoral Injection

Deisseroth, Albert; Sidney Kimmel Cancer Center, San Diego, California; *Intratumoral Injections of a Replication-Incompetent Adenoviral Vector Encoding a Factor VII Immunoconjugate to Induce a Cytolytic Immune Response against Melanoma Tumors: A Pilot Trial.*

NIH/OBA Receipt Date: 10-18-00. Publicly Reviewed at the December 2000 RAC meeting

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0010-420 (Closed) Gene Therapy/Phase I-II/Other Disorders/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration

Crystal, Ronald G; Cornell University Medical College, New York, New York; and Rosengart, Todd; Evanston Northwestern Healthcare, Evanston, Illinois; *Phase I/II, Prospective, Placebo Controlled, Randomized Assessment of Direct Administration of a Replication Deficient Adenovirus Vector (Ad_{CU}VEGF121.1) Containing the VEGF121 cDNA to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease as an Adjunct to Coronary Bypass Surgery*.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00
Closed, never initiated, no individuals enrolled: 5-14-03

0010-421 (Open) Gene Therapy/Phase I/Other Disorders/Ulcer/In Vivo/Adenovirus/Serotype 5/Platelet Derived Growth Factor (PDGF) cDNA/Intra Ulcer Administration

Mozingo, David; University of Florida College of Medicine, Gainesville, Florida; Mulder, Gerit D.; University of California, San Diego Medical Center, San Diego, California; and Tallis, Arthur; Associated Foot and Ankle Specialists, Phoenix, Arizona; *A Dose Escalating Phase I Study of AdPDGF-B/GAM in the Treatment of Diabetic Ulcers of the Lower Extremity*. Sponsor: Selective Genetics, Inc. (no longer in business: 10-04)

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

0010-422 (Open) Gene Therapy/Phase I/Infectious Diseases/HIV-1/Replication Inhibition/Single Chain Antibody Gene/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/sFvhutat2 ant-HIV-1 Tat Protein Antibody/Intravenous Infusion

Marasco, Wayne; Dana-Farber Cancer Institute, Boston, Massachusetts; *A Pilot Study to Evaluate the Safety and Effects of Autologous Lymphocytes Transduced with a Human Single-Chain Antibody Directed against the HIV-1 Tat Protein in HIV-1 Infected Human Subjects with Advanced Disease*.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

0010-423 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Monogenic Diseases/Junctional Epidermolysis Bullosa/In Vitro/Autologous Keratinocytes/Retrovirus/Laminin 5-beta3 cDNA/Skin Graft

Kimball, Alexa B.; Stanford University Medical Center; *Laminin 5 Beta 3 Gene Therapy for Junctional Epidermolysis Bullosa*.

NIH/OBA Receipt Date: 10-18-00. Publicly Reviewed at the December 2000 RAC meeting

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0010-424 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/Plasmid DNA/Poloxamer 188/Del-1 cDNA/Intramuscular Injection

Hinohara, Tomoaki; Cardiovascular Medicine and Coronary Interventions, Redwood City, California; Litt, Marc R.; Jacksonville Heart Center, Jacksonville, Florida; Karlsberg, Ronald P.; Cardiovascular Research Institute, Beverly Hills, California; Schaer, Gary L.; Rush-Presbyterian-St. Luke's Medical Center, Chicago, Illinois; Piana, Robert; Vanderbilt University Medical Center, Nashville, Tennessee; Jaff, Michael; The Heart and Vascular Institute of New Jersey, Morristown, New Jersey; and Rajagopalan, Sanjay; University of Michigan Health System, Ann Arbor, Michigan; *Developmentally Regulated Endothelial Locus (Del-1) Gene Medicine (VLTS-589) A Phase I Multi-Center, Open-Label, Single-Dose Escalation Clinical Safety Trial of VLTS-589 for the Treatment of Patients with Peripheral Arterial Disease*. Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00
Closed: 6-9-06

0010-425 (Under Review) Gene Therapy/Phase I/Other Disorders/Bone Fracture/In Vivo/Plasmid DNA/Collagen Sponge/Parathyroid Hormone cDNA/Bone Administration

Goulet, James Alan; University of Michigan, Ann Arbor, Michigan; *A Prospective, Randomized Study to Assess the Safety of MAT-100 in Open Tibia Fractures Requiring an Intramedullary Rod (Phase I)*. Sponsor: EBI, L.P./Biomed, Inc. and Selective Genetics, Inc.

NIH/OBA Receipt Date: 10-18-00. Review at a RAC meeting pending; sponsor has requested postponement of public review.

0010-426 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Adenovirus Serotype 5/Replication-Competent Virus/Osteocalcin Promoter/Intratumoral Injection

Gardner, Thomas A.; Indiana School of Medicine, Indianapolis, Indiana; *A Phase I Study of Intratumoral Injections of OCaP1 for Metastatic or Locally Recurrent Prostate Cancer, Part 1: Dose Finding, Part 2: Index Lesion Escalation*. Sponsor: DirectGene, Inc.

NIH/OBA Receipt Date: 10-18-00. Publicly Reviewed at the December 2000 RAC meeting

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0010-427 (Open) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Adenovirus Serotype 5/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Intradermal Administration

Crystal, Ronald G.; Cornell University Medical College, New York, New York; *Effect of Ad_{GV}CFTR.10 on the Cystic Fibrosis Phenotype*.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

0010-428 (Open) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/Pro-Drug/In Vivo/Adenovirus Serotype 5/Replication-Competent Virus/Cytosine Deaminase cDNA/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection

Kim, Jae Ho and Freytag, Svend; Henry Ford Health System, Detroit, Michigan; *Phase I Study of Intraprostatic Administration of a Replication-Competent, Oncolytic Adenovirus Using Various Vector formulations to Patients with Localized Prostate Cancer Prior to Radical Prostatectomy*.

NIH/OBA Receipt Date: 10-18-00. Not Selected for RAC Public Review: 11-7-00

0010-429 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma (SCCHN)/Immunotherapy/In Vivo/Fowlpox Virus/B7.1 (CD 80)/ICAM-1/LFA-3/Intratumoral Injection

Van Waes, Carter; National Institutes of Health, Bethesda, Maryland; *Phase I/Pilot Study of Intralesional Immunotherapy with a Recombinant Avipox Virus Engineered to Express a Triad of Co-stimulatory Molecules in Patients with Advanced Squamous Cell Carcinoma of the Head and Neck.*

NIH/OBA Receipt Date: 10-26-00. Not Selected for RAC Public Review: 5-10-01

0011-430 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Serotype-5/Interleukin-7 cDNA/Intratumoral Injection

Dubinett, Steven M.; UCLA School of Medicine, Los Angeles, California; *A Phase I/II Trial Evaluating Intratumoral Injection of Interleukin-7 Gene Modified Autologous Dendritic Cells for the Treatment of Non-Small Cell Lung Cancer*.

NIH/OBA Receipt Date: 11-1-00. Not Selected for RAC Public Review: 11-22-00

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0011-431 (Closed) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Tumor Cells/Cationic Liposome Complex/DMRIE-DOPE/Vical-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection

Gonzalez, Rene; University of Colorado Health Sciences Center; Aurora, Colorado; Yazadi, G. Paul.; Missouri Baptist Medical Center, The Melanoma Center of St. Louis, St. Louis, Missouri; Amatruda, Thomas; North Memorial Health Care/Hubert H. Humphrey Cancer Center, Robbinsdale, Minnesota; Morse, Michael; Duke University Medical Center, Durham, North Carolina; Atkins, Michael B.; Beth Israel Deaconess Medical Center, Boston, Massachusetts; Bedikian, Agop Y.; The University of Texas, M.D. Anderson Cancer Center, Houston, Texas; Lutzky, Jose; Mt. Sinai Comprehensive Cancer Center, Miami, Florida; Hutchins, Laura; University of Arkansas for Medical Sciences and Central Arkansas Veteran's Healthcare System, Little Rock, Arkansas; Schwarzenberger, Paul; Louisiana State University Health Sciences Center Lions Clinic, and the Medical Center of Louisiana at New Orleans, New Orleans, Louisiana; Patel, Ravi; Comprehensive Blood and Cancer Center, Bakersfield, California; Thant, Myo; Maryland Hematology/Oncology Associates, Baltimore, Maryland; Thompson John A.; University of Washington Medical Center and the Seattle Cancer Care Alliance, Seattle Washington; Galanis, Evanthia; Mayo Clinic, Rochester, Minnesota; Ernstoff, Marc; Dartmouth-Hitchcock Medical Center, Lebanon, New Hampshire; Richards, Jon M.; Oncology Specialities, S. C., Park Ridge, Illinois; Venook, Alan; University of California, San Francisco Comprehensive Cancer Center at Mount Zion, San Francisco, California; Hersh, Evan M.; Arizona Cancer Center, Tucson, Arizona; and Blum, Ronald; Beth Israel Medical Center, New York, New York; *A Phase II Study of High-Dose Allovectin-7 in Patients with Advanced Metastatic Melanoma*. Sponsor: Vical Inc.

NIH/OBA Receipt Date: 11-16-00. Not Selected for RAC Public Review: 12-7-00
Closed to enrollment: 4-23-04

0011-432 (Closed) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE.Vical-1005/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection

Gleich, Lyon, University of Cincinnati Medical Center, Cincinnati, Ohio; Khan, Mumtaz, Henry Ford Health System, Detroit, Michigan; Wolf, Gregory T., University of Michigan Health System, Ann Arbor, Michigan; Stenson, Kerstin M., The University of Chicago, Chicago, Illinois; Weinstein, Gregory, The Hospital of the University of Pennsylvania, Philadelphia, Pennsylvania; Lavertu, Pierre, Case Western University, University Hospitals of Cleveland, Cleveland, Ohio; Carroll, William, University of Alabama-Birmingham, Birmingham, Alabama; Hanna, Ehab, University of Arkansas for Medical Sciences, Little Rock, Arkansas; McCaffrey, Thomas, H. Lee Moffitt Cancer Center and Research Institute, Tampa, Florida; and Friedlander, Paul, Louisiana State University Health Sciences Center, New Orleans, Louisiana; *A Phase II Study of Safety and Efficacy of Allovectin-7 Immunotherapy for the Treatment of Primary Resectable Squamous Cell Carcinoma of the Oral Cavity or Oropharynx*. Sponsor: Vical Inc.

NIH/OBA Receipt Date: 11-16-00. Not Selected for RAC Public Review: 12-7-00
Closed to enrollment: 6-23-03

0011-433 (Open) Gene Marking/Cancer/Acute or Chronic Myelogenous Leukemia, Non-Hodgkin's Lymphoma, Myelodysplastic Syndrome/In Vitro/Epstein-Barr Virus Specific Allogeneic Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Bone Marrow Transplantation

Brenner, Malcolm, Baylor College of Medicine, Houston, Texas; *A Phase I Trial Evaluating the Use of RFT5-dgA to Deplete Alloreactive Cells Prior to Haploidentical Stem Cell Transplantation*.

NIH/OBA Receipt Date: 11-27-00. Not Selected for RAC Public Review: 12-15-00

0011-434 (Open) Gene Therapy/Phase I/Cancer/Various Types/Immunotherapy/In Vitro/Allogeneic Fibroblasts/Lethally Irradiated/Plasmid DNA/Interleukin-2 cDNA/Intratumoral Injection

Sobol, Robert E., Sidney Kimmel Cancer Center, San Diego, California; *A Phase I Study of Intra-Tumoral Injection with Allogeneic Fibroblasts Genetically Modified to Secrete IL-2 in Patients with Cancer Who Have Failed Standard Therapy*

NIH/OBA Receipt Date: 11-27-00. Not Selected for RAC Public Review: 12-22-00

0011-435 (Closed) Gene Therapy/Phase I-II/Cancer/Myeloma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Borrello, Ivan, Johns Hopkins University School of Medicine, Baltimore, Maryland; *Vaccination in Peripheral Stem Cell Transplant Setting for Multiple Myeloma: The Use of Autologous Tumor Cells with an Allogeneic GM-CSF Producing Bystander Cell Line*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 11-29-00. Not Selected for RAC Public Review: 12-28-00
Closed to further enrollment: 2-13-04

0012-436 (Open) Gene Therapy/Phase I/Cancer/Glioma/Immunotherapy/In Vitro/Autologous Fibroblasts/In Combination with Untransduced Autologous Tumor and Dendritic Cells/Interleukin-4 cDNA/Intradermal Injection

Okada, Hideho, University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; *Gene Therapy of Malignant Gliomas: A Pilot Study of Vaccination with Autologous Glioma-Lysate and Dendritic Cells Admixed with IL-4 Transduced Fibroblasts to Elicit an Immune Response*.

NIH/OBA Receipt Date: 12-5-00. Not Selected for RAC Public Review: 12-27-00

0012-437 (Closed) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Adenovirus/Serotype 5/CD40 Ligand cDNA/Intratumoral Injection

Harvey, Ben-Gary and Crystal, Ronald G., New York Presbyterian Hospital-Weill Medical College, Cornell University, New York, New York; *In Vivo Transfer of the CD40 Ligand Gene to Primary Lung Tumors to Activate Dendritic Cells and Induce Anti-Tumor Immunity*.

NIH/OBA Receipt Date: 12-7-00. Not Selected for RAC Public Review: 12-28-00
Never initiated, no enrollment: 12-07-11

0012-438 (Closed) Gene Therapy/Cancer/Head and Neck Squamous Cell Carcinoma/Oncogene Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intratumoral Injection

Arseneau, James C., Albany Regional Cancer Center, Albany, New York; Berman, Barry S., Cancer Centers of Florida, Orlando, Florida; Anthony, Stephen P., Cancer Care Northwest, Spokane, Washington; Richards, Donald A., Tyler Cancer Center, Tyler, Texas; and Nemunaitis, John and Senzer, Neil, US Oncology, Dallas, Texas; *A Multicenter, Phase II Study of Intratumoral Injections of E1A-Lipid Complex and Re-Irradiation for Treatment of Patients with Recurrent Head and Neck Squamous Cell Carcinoma*. Sponsor: Targeted Genetics

NIH/OBA Receipt Date: 12-22-00. Not Selected for RAC Public Review: 3-2-01
No longer active: 11-23-02

0012-439 (Closed) Gene Therapy/Phase I/Other/Peripheral Artery Disease/In Vivo/Ischemic Lower Limb/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Intramuscular Injection

Crystal, Ronald G., Weill Medical College, Cornell University, New York, New York; *Gene Therapy in Conjunction with Operative Bypass Grafting for Severe Peripheral Vascular Ischemia in Individuals with Insulin-Dependent Diabetes*.

NIH/OBA Receipt Date: 12-26-00. Not Selected for RAC Public Review: 1-16-01
Closed, never initiated, no individuals enrolled: 11-20-03

0101-440 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Murine gp75 Melanoma Antigen/Intramuscular Injection

Wolchok, Jedd D., Memorial Sloan Kettering Cancer Center, New York, New York; *Phase I Study of gp75 DNA Vaccine in Patients with AJCC Stage III and IV Melanoma*. Sponsor: ImClone Systems, Inc.

NIH/OBA Receipt Date: 1-3-01. Not Selected for RAC Public Review: 1-24-01
Closed, no longer recruiting: 3-23-06

0101-441 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Vaccinia Virus/B7.1 (CD80)/ICAM-1/LFA-3/Intratumoral Injection

Kaufman, Howard L., Columbia University, New York, New York; *A Phase I Trial of Intralesional rV-TRICOM Vaccine in the Treatment of Malignant Melanoma*.

NIH/OBA Receipt Date: 1-4-01. Not Selected for RAC Public Review: 1-26-01

0101-442 (Closed) Gene Therapy/Phase I-II/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration

Crystal, Ronald G., Weill Medical College, Cornell University, New York, New York; and Rosengart, Todd K., Evanston Northwestern Healthcare, Evanston, Illinois; *Phase I/II, Prospective Placebo Controlled, Randomized Assessment of Adenoviral Mediated VEGF121 cDNA Myocardial Angiogenesis Therapy as an Adjunct to Individuals with Diffuse Coronary Artery Disease Undergoing Repeat Coronary Artery Bypass Surgery*.

NIH/OBA Receipt Date: 1-9-01. Not Selected for RAC Public Review: 1-30-01
Closed: 9-30-03, no participants enrolled

0101-443 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/Antisense TAR/Antisense tat-rev/In Vitro/CD34+ Cells/Intravenous

Laurence, Jeffrey C., Cornell University Medical College, New York, New York; *Evaluation of the Safety and Effects of ex vivo Modification and Re-Infusion of CD34+ Cells by an Antisense Construct Against HIV-1 in a Retrovirus Vector*. Sponsor: Enzo Therapeutics, Inc.

NIH/OBA Receipt Date: 1-10-01. Publicly Reviewed at the March 2001 RAC meeting

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0101-444 (Open) Gene Therapy/Phase I/Coronary Artery Disease/In Vivo/Muscle Cells/Plasmid DNA/Poloxamer 188/Del-1 cDNA/Retrograde Intravenous (rIV) Injection into the Heart

Dreiling, Roger J., Cardiovascular Consultants of Oregon, Corvallis, Oregon; *A Phase I Multi-Center, Open-Label, Single-Dose Escalation Clinical Trial of VLTS-589 for Treatment of Patients with Coronary Artery Disease*. Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 4-20-01

0101-445 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Head and Neck Squamous Cell Carcinoma/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection

Clayman, Gary, The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Clinical Protocol for Wild Type p53 Gene Induction in Premalignancies of Squamous Epithelium of the Oral Cavity Via an Adenoviral Vector*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 1-10-01. Publicly Reviewed at the March 2001 RAC meeting

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0101-446 (Open) Gene Therapy/Phase I/Monogenic Disease/Severe Combined Immune Deficiency Due to JAK3 Deficiency/In Vitro/Autologous CD34+ Cells from Peripheral Blood or Bone Marrow/Retrovirus/JAK3 cDNA/Intravenous Infusion

Sorrentino, Brian P. and Cunningham, John M., St. Jude Children's Research Hospital, Memphis, Tennessee; and Buckley, Rebecca, Duke University School of Medicine, Durham, North Carolina; *Transplantation of Gene-Corrected Autologous CD34+ Hematopoietic Stem Cells in Previously Transplanted Patients with JAK3 Deficiency and Persistent Humoral Immune Defects*.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

0101-447 (Open) Gene Therapy/Phase I/Cancer/Prostate Cancer/In Vivo/Dendritic Cells/Adenovirus/Serotype 5/PSA cDNA/Subcutaneous Injection

Lubaroff, David, University of Iowa, Iowa City, Iowa; *Phase I Study of Adenovirus/PSA Vaccine in Men with Metastatic Prostate Cancer*.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

0101-448 (Open) Gene Therapy/Phase II-III/Cancer/Non-Small Cell Lung Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection

Swisher, Stephen, University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase II/III, Multi-Center, Open-Label, Randomized Study to Compare the Effectiveness and Safety of Intralesional Administration of RPR/INGN 201 in Combination with Taxotere® and Carboplatin and Radiotherapy Versus Taxotere® and Carboplatin and Radiotherapy Alone in Patients with Locally Advanced Unresectable Non-Small Cell Lung Cancer (NSCLC*). Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

0101-449 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection

Miles, Brian J., Baylor College of Medicine, Houston, Texas; *Phase I Study of Adenoviral Vector Delivery of the IL-12 Gene in Men with Recurrence or Persistent Cancer of the Prostate after Primary Therapy with or without Metastatic Disease (SPORE).*

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0101-450 (Closed) Gene Therapy/Phase II/Cancer/Prostate Cancer/Vector Directed Cell Lysis/In Vivo/Autologous Tumor Cells/Adenovirus/Serotype 5/Replication Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intratumoral Injection

DeWeese, Theodore, Johns Hopkins Oncology Center, Baltimore, Maryland; Roach III, Mack, University of California, San Francisco, San Francisco, California; and Michalski, Jeff, Washington University Medical School, Saint Louis, Missouri; *A Phase II Randomized Comparison Study of an Intraprostatic Injection of CV7606 Followed by External Beam Radiotherapy (EBRT) Versus EBRT Alone in Patients with Intermediate Risk, Clinically Localized Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01
Study was never initiated

0101-451 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Vector Directed Cell Lysis/In Vivo/Adenovirus/Serotype 5/Replication Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intravenous Injection

Small, Eric, University of California, San Francisco, San Francisco, California; *A Randomized, Placebo Controlled Phase II Study of an Intravenous Injection of CV787, a Prostate-Specific Antigen Oncolytic Adenovirus, Plus Weekly Docetaxel in Patients with Metastatic Hormone Refractory Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01
Study was never initiated

0101-452 (Closed) Gene Therapy/Phase IIb-III/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration

Grines, Cindy L., William Baumont Hospital, Royal Oak, Michigan; Bethala, Vasanth, Medical Research Institute, Slidell, LA; Erenrich, Norman, Florida Cardiovascular Research, Atlantis, FL; Gammon, Roger, Austin Heart, Austin, TX; Henry, Timothy, Abbott Northwestern Hospital, Minneapolis, MN; Licandro, Rudolph, Louisville Cardiology Medical Group, Louisville, KY; Saucedo, Jorge, University of Arkansas for Medical Sciences, Little Rock, AR; Tonkon, Melvin, Anaheim Heart and Research Institute, Santa Ana, CA; Watkins, Matthew , The University of Vermont, Burlington, VT; Grossman, P. Michael, The University of Michigan Health System, Ann Arbor, Michigan; Butman, Samuel, University Medical Center, University of Arizona, Tucson, Arizona; Churchill, David A., Washington Regional Medical Center, Fayetteville, Arkansas; Conn, Eric H., The Chattanooga Heart Institute, Chattanooga, Tennessee; Coppola, John T., Saint Vincent Catholic Medical Centers of New York, New York, New York; Fuchs, Shmuel, Washington Hospital Center, Washington, D.C.; Ghali, Jalal K., Cardiac Centers of Louisiana, Shreveport, Louisiana; Hodes, Zachary I., The Care Group, LLC, Indianapolis, Indiana; Nadar, Venkatesh K., Heritage Cardiology Associates, Camp Hill, Pennsylvania; Rowe, Steven K., Heartland Regional Medical Center, St. Joseph, Missouri; Brennan, Theresa, University of Iowa Healthcare, Iowa City, Iowa; Browne, Jr., Kevin F., Watson Clinic LLP, Lakeland, Florida; Dib, Nabil, Arizona Heart Institute, Phoenix, Arizona; Ellis, Stephen, Cleveland Clinic Foundation, Cleveland, Ohio; Hart, Kevin, Stucky Research Center, Fort Wayne, Indiana; Iskandrian, Ami E., University of Alabama at Birmingham, Birmingham, Alabama; Kleiman, Neal S., Baylor College of Medicine, Houston, Texas; Marmur, Jonathan, Mount Sinai Hospital, New York, New York; Marshall, J. Jeffrey, Crawford Long Hospital, Atlanta, Georgia; Penny, William F., San Diego VA Medical Center, San Diego, California; Pepine, Carl J., University of Florida, Gainesville, Florida; Saenz, Carlos and Taussig, Andrew, Florida Hospital, Orlando, Florida; Schaer, Gary L., Rush-Presbyterian St. Luke's Medical Center, Chicago, Illinois; Sequeira, Rafael F., University of Miami-Jackson Memorial Hospital, Miami, Florida; Baran, Kenneth W., United's John Nasseff Heart Hospital, Saint Paul, Minnesota; Helmer, Gregory A., Minnesota Heart Clinic, P.A., Edina, Minnesota; Mendelsohn, Farrell O., Cardiology, P.C., Birmingham, Alabama; Moran, John F., Loyola University Medical Center, Maywood, Illinois; Sanborn, Timothy, Evanston Northwestern Healthcare, Evanston, Illinois; Sharaf, Barry L., Rhode Island Hospital, Providence, Rhode Island; Moreyra, Abel E., Robert Wood Johnson Medical School, New Brunswick, New Jersey; Cohen, Eric, Cardiovascular Associates, P.C., Birmingham, Alabama; Lee, Joon, University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; Levine, Glenn, Houston VA Medical Center, Houston, Texas; Lopez, John, University of Chicago Medical Center, Chicago, Illinois; McGrew, Frank, III, The Stern Cardiovascular Center, Memphis, Tennessee; Thai, Hoang, Southern Arizona Veterans Affairs Health Care System, Tucson, Arizona; Zabalgotia, Miguel, The University of Texas Health Science Center at San Antonio; San Antonio, Texas; Laham, Roger, Beth Israel Deaconess Medical Center, Boston, Massachusetts; Murphy, Patrick, L., The Heart Group, PC, Mobile, Alabama; Rade, Jeffrey J., Johns Hopkins University School of Medicine, Baltimore, Maryland; Simari, Robert D., Mayo Clinic, Rochester, Minnesota; Savage, Michael P., Jefferson Heart Institute, Philadelphia, Pennsylvania; Zoble, Robert G., James A. Haley Veterans Hospital, Tampa, Florida; Kellett, Mirle, Maine Medical Center, Portland, Maine; Moreyra, Abel, Robert Wood Johnson University Hospital, New Brunswick, New Jersey; Niles, Nathaniel, Dartmouth-Hitchcock Medical Center, Lebanon, New Hampshire; Ohman, Erik, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina; Murray, Conrad, Global Cardiovascular Associates, Inc., Las Vegas, Nevada; Furman, Mark I., University of Massachusetts Medical Center, Worchester, Massachusetts; Grossman, P Michael, University of Michigan Health Systems, Ann Arbor, Michigan; Chronos, Nicholas, Atlanta Cardiology Research Institute, Atlanta, Georgia; Mahmud, Ehtisham, University of California, San Diego, Medical Center, San Diego, California; Molk, Barry, Aurora Denver Cardiology Associates, P.C., Denver, Colorado; Bhoopalam, Vishwajeth, Nebraska Heart Institute, Lincoln, Nebraska; West, Andrew J., Heart & Vascular Institute of Texas, PA, San Antonio, Texas; Corbelli, John C., Buffalo Cardiology & Pulmonary Associates PC, Williamsville, New York; Lieberman, Scott, East Texas Medical Center, Tyler, Texas; Goldberg, Steven, University of Washington Medical Center, Seattle, Washington; and Durkin, Raymond A., Lehigh Valley Hospital, Allentown, Pennsylvania; *A Multicenter, Randomized, Double-Blind, Placebo Controlled, Dose-Response Study to Evaluate the Efficacy and Safety of Ad5.1FGF-4 in Patients with Stable Angina*. Sponsor: Cardium Therapeutics

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01
Closed to enrollment: 05-14-04

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0101-453 (Closed) Gene Therapy/Phase I/Cancer/Glioblastoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Interferon-beta cDNA/Stereotactic Injection

Eck, Stephen L., University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; Rosenfeld, Steven S., University of Alabama at Birmingham, Birmingham, Alabama; Chiocca, E. Antonio, Massachusetts General Hospital, Boston, Massachusetts; Hamilton, Allan, University of Arizona, Tucson, Arizona; and Lillehei, Kevin, University of Colorado Health Sciences Center, Denver, Colorado; *A Multi-Center, Open Label, Two Part, Dose Escalation Study to Determine the Tolerability of Interferon-beta Gene Transfer in the Treatment of Recurrent or Progressive Glioblastoma Multiforme*. Sponsor: Biogen Idec, Inc..

NIH/OBA Receipt Date: 1-12-01. Not Selected for RAC Public Review: 1-31-01
Closed: 7-28-04

0101-454 (Closed) Gene Therapy/Phase II/Cancer/Head and Neck Squamous Cell Carcinoma/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection

Yoo, George H., Wayne State University, Detroit, Michigan; Taylor, Sarah A., The University of Kansas Medical Center, Kansas City, Kansas; Valentino, Joseph; University of Kentucky, Lexington, Kentucky; Wein, Richard; University of Mississippi Medical Center; Jackson, Mississippi; and Hana, Ehab; University of Texas, M.D. Anderson Cancer Center; Austin, Texas; *Phase II Trial of Surgery with Perioperative RPR/INGN 201 (Ad5CMV-p53) Gene Therapy Followed by Chemoradiotherapy for Advanced Resectable Squamous Cell Carcinoma of the Oral Cavity, Oropharynx, Hypopharynx, and Larynx*. Sponsor: Southwest Oncology Group.

NIH/OBA Receipt Date: 1-10-01. Not Selected for RAC Public Review: 1-31-01
Closed: 7-19-06

0101-455 (Open) Gene Therapy/Phase II/Cancer/Breast/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p53 cDNA/Intratumoral Injection

Cristofanilli, Massimo, The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Phase II, Single Arm, Single Institution Clinical Trial of Docetaxel and Doxorubicin in Combination with Local Administration of Ad5CMV-p53 (RPR/INGN-201) in Locally Advanced Breast Cancer (LABC)*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 1-16-01. Not Selected for RAC Public Review: 1-31-01

0101-456 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD 80)/ICAM-1/LFA-3/GM-CSF/Intramuscular or Intradermal Injection

von Mehren, Margaret, Fox Chase Cancer Center, Philadelphia, Pennsylvania; *Phase I Study of a Recombinant Fowlpox Vaccine rF-CEA(6D)/TRICOM alone or with GM-CSF in Patients with Advanced CEA Expressing Adenocarcinoma*.

NIH/OBA Receipt Date: 1-25-01. Not Selected for RAC Public Review: 2-27-01

0101-457 (Closed) Gene Therapy/Phase I/Cancer/Soft Tissue Sarcoma/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection

Kenady, Daniel E., University of Kentucky Chandler Medical Center, Lexington, Kentucky; Nemunaitis, John, US Oncology, Dallas, Texas; Sandler, Alan B., Vanderbilt University, Nashville, Tennessee; Vijayakumar, Srinivasan and Warso, Michael, University of Illinois at Chicago, Chicago, Illinois; Mundt, Arno, University of Chicago, Chicago, Illinois; and Richards, Donald A., Tyler Cancer Center, Tyler, Texas; *An Open-Label, Phase I, Dose-Escalation Study of TNFerade™ Biologic with Radiation Therapy as an Adjunct to Surgery or for Palliation of Soft Tissue Sarcoma of the Extremities*. Sponsor: GenVec.

NIH/OBA Receipt Date: 1-29-01. Not Selected for RAC Public Review: 2-26-01
Enrollment completed, long-term follow-up continues: 1-15-04

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0102-458 (Closed) Gene Therapy/Phase II/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Canarypox Virus/Carcinoembryonic Antigen/B 7.1 (CD80)/Intramuscular and Intradermal Injections

Kaufman, Howard L., Columbia University, New York, New York; von Mehren, Margaret, Fox Chase Cancer Center, Philadelphia, Pennsylvania; Conry, Robert M., The University of Alabama at Birmingham, Birmingham, Alabama; Marshall, John, Georgetown University Medical Center, Washington D.C.; Heim, William J., Hematology & Oncology Associates of Northeastern PA, Dunmore, Pennsylvania; Lenz, Heinz-Josef, University of Southern California/Norris Comprehensive Cancer Center, Los Angeles, California; Kindler, Hedy, The University of Chicago, Chicago, Illinois; Garrett, Christopher, H. Lee Moffitt Cancer Center, Tampa, Florida; Urba, Walter, Providence Portland Medical Center, Portland, Oregon; and Benson, Al B., III; Northwestern University, Chicago, Illinois; *Pilot Phase II Study of Safety and Immunogenicity of a ALVAC-CEA/B7.1 Vaccine Administered with Chemotherapy, Alone or in Combination with Tetanus Toxoid, as Compared to Chemotherapy Alone, in Patients with Metastatic Colorectal Adenocarcinoma*. Sponsor: Aventis Pasteur Limited.

NIH/OBA Receipt Date: 2-15-01. Not Selected for RAC Public Review: 4-12-01
Completed: April 2004

0103-459 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection

Corman, John M., Virginia Mason Medical Center, Seattle, Washington; Dula, Eugene, West Coast Clinical Research, Van Nuys, California; and Young, Jay M., South Orange County Medical Research Center, Laguna Woods, California; *A Phase I Dose Escalation Study of Human GM-CSF Gene Transduced Irradiated Allogeneic Prostate Cancer Cell Vaccine (GVAX® Prostate Cancer Vaccine (PC-3)) in Patients with Hormone-Refractory Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 3-6-01. Not Selected for RAC Public Review: 7-27-01
Closed: 7-07-03

0103-460 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/Autologous Lymphoma Cells/Adenovirus/Serotype 5/Interleukin-2 cDNA/CD40 Ligand cDNA/Subcutaneous Injection

Takahashi, Satoshi and Brenner, Malcolm, Baylor College of Medicine, Houston, Texas; *Treatment of Chronic Lymphocytic Leukemia (CLL) with IL-2 Gene Modified and Human CD40 Ligand-Expressing Autologous Tumor Cells*.

NIH/OBA Receipt Date: 3-19-01. Not Selected for RAC Public Review: 4-6-01

0104-461 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Polyvinylpyrrolidone (PVP)/Interferon-alpha/Interleukin-12 cDNA/Intratumoral Injection

Posner, Marshall R., Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I/II Multi-Center, Open-Label, Multiple Administration Trial of the Safety, Tolerability, and Efficacy of an IFN-alpha/IL-12 Plasmid-Based Therapeutic*. Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 4-9-01. Not Selected for RAC Public Review: 4-27-01

0104-462 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Pro-Drug/In Vivo/Tumor Cells/Salmonella typhimurium/E. coli Cytosine Deaminase cDNA/Intratumoral Injection/Combined with 5fluorocytosine

Nemunaitis, John J. and Cunningham, Charles, Mary Crowley Medical Research Center (US Oncology), Dallas, Texas; *A Phase I Trial of Genetically Modified Salmonella typhimurium Expressing Cytosine Deaminase (TAPET-CD, VNP20029) Administered by Intra-Tumoral Injection in Combination with 5-fluorocytosine for Patients with Advanced or Metastatic Cancer*. Sponsor: Vion Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 4-16-01. Publicly Reviewed at the June 2001 RAC meeting.

0104-463 (Closed) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/Fibroblast Growth Factor 1 cDNA/Intramuscular Injection

Comerota, Anthony J., Toledo Hospital, Toledo, Ohio; Henry, Tim, Minneapolis Heart Institute, Minneapolis, Minnesota; and Chronos, Nicolas, Atlanta Cardiovascular Research Institute, Atlanta, Georgia; *Phase I Double Blind, Parallel-Group, Multi-Center, Gene Expression (Synthesis of FGF-1 mRNA), Safety and Tolerability Study of Increasing Single Doses of NV1FGF Administered by Intra-Muscular Injection in Patients with Severe Peripheral Artery Occlusive Disease (PAOD) Planned to Undergo Amputation Above the Ankle*. Sponsor: Aventis Pharma Recherche-Développement.

NIH/OBA Receipt Date: 4-16-01. Not Selected for RAC Public Review: 5-4-01
Study terminated: 10-03

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0104-464 (Open) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/E. coli Cytosine Deaminase cDNA/5-Fluorocytosine/Herpes Simplex Thymidine Kinase cDNA/Ganciclovir/Intratumoral Injection

Kim, Jae Ho and Freytag, Svend O., Henry Ford Health System, Detroit, Michigan; *Phase I Study of Combined Suicide Gene Therapy and Radiation Therapy for Locally Advanced Carcinoma of the Prostate*.

NIH/OBA Receipt Date: 4-17-01. Not Selected for RAC Public Review: 5-7-01

0104-465 (Open) Gene Therapy/Phase I/Monogenic Disease/Alpha-1 Antitrypsin Deficiency/In Vivo/Adeno-Associated Virus/Alpha-1 Antitrypsin cDNA/Intramuscular Injection

Flotte, Terence R., University of Florida, Gainesville, Florida; *A Phase I Trial of Intramuscular Injection of a Recombinant Adeno-Associated Virus Alpha-1-Antitrypsin (rAAV-AAT) Gene Vector to AAT-Deficient Adults*.

NIH/OBA Receipt Date: 4-18-01. Not Selected for RAC Public Review: 5-8-01

0104-466 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Chemoprotection/In Vivo/Cationic Liposome Complex/Cholesterol/DOTIM/Manganese Super Oxide Dismutase (MnSOD)/Intraesophageal Administration

Belani, Chandra P., University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *Concurrent Chemotherapy (Paclitaxel and Carboplatin) and Thoracic Radiotherapy with Swallowed Manganese Superoxide Dismutase (MnSOD) Plasmid Liposome (PL) Protection in Patients with Locally Advanced Stage III Non-Small Cell Lung Cancer. A Phase I-II Study*.

NIH/OBA Receipt Date: 4-18-01. Not Selected for RAC Public Review: 5-8-01

0104-467 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Other/Peripheral Neuropathy/In Vivo/Endothelial Cells/Plasmid DNA/VEGF$_{165}$ cDNA/Intramuscular Injection

Ropper, Allan, St. Elizabeth's Medical Center, Boston, Massachusetts; *VEGF Gene Transfer for Diabetic Neuropathy*.

NIH/OBA Receipt Date: 4-18-01. Publicly Reviewed at the June 2001 RAC meeting.
Closed to recruitment: 7-31-04

0104-468 (Open) Gene Therapy/Phase I-II/Coronary Artery Disease/In Vivo/Endothelial Cells/Plasmid DNA/VEGF$_{165}$ cDNA/Intramyocardial Injection

Smedira, Nicholas; The Cleveland Clinic Foundation, Cleveland, Ohio; *VEGF Gene Transfer to Promote Angiogenesis in Patients with Advanced Heart Failure*.

NIH/OBA Receipt Date: 4-18-01. Not Selected for RAC Public Review: 5-8-01

0104-469 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other/Parkinson's Disease/In Vivo/Adeno-Associated Virus/Glutamic Acid Decarboxylase 65-67 cDNA/Intracerebral Administration

During, Matthew J., Jefferson Medical College, Philadelphia, Pennsylvania; Kaplitt, Michael, New York Hospital-Weill Medical College of Cornell University, New York, New York; and Eidelberg, David, North Shore-Long Island Jewish Research Institute, Manhasset, New York; *Subthalamic GAD Gene Transfer in Parkinson Disease Patients Who Are Candidates for Deep Brain Stimulation*.

NIH/OBA Receipt Date: 4-18-01. Publicly Reviewed at the June 2001 RAC meeting.
Closed to enrollment: 8-01-06

0104-470 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Osteosarcoma Metastasis to Lung/Vector-Directed Cell Lysis/In Vivo/Adenovirus Serotype 5/Replication-Competent Virus/Promoter of Osteocalcin/Intravenous Injection

Meyers, Paul A., Memorial Sloan-Kettering Cancer Center, New York, New York and Reaman, Gregory H., George Washington University School of Medicine and Children's National Medical Center, Washington, D.C.; *A Phase I/II Dose Escalation and Activity Study of Intravenous Injections of OCaP1 in Subjects with Refractory Osteosarcoma Metastatic to Lung*. Sponsor: DirectGene, Inc.

NIH/OBA Receipt Date: 4-18-01. Publicly Reviewed at the June 2001 RAC meeting.

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0104-471 (Open) Gene Therapy/Cancer/Breast Cancer/In Vivo/Tumor Suppressor/Adenovirus/Melanoma Differentiation Associated Protein-7 cDNA/Intratumoral Injection

Buchholz, Thomas A., M.D. Anderson Cancer Center, Houston, Texas; *A Phase I/II Dose-Escalation Trial of Intratumoral Injection with a Replication-Deficient Adenovirus Vector, Ad-mda7 (INGN 241), in Combination with Radiation Therapy in Patients with Locally Recurrent Breast Cancer*. Sponsor: Introgen Therapeutics, Inc.
NIH/OBA Receipt Date: 4-18-01. Not Selected for RAC Public Review: 5-8-01

0105-472 (Closed) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Smith II, John W., Providence Portland Medical Center, Portland, Oregon; Aboulafia, David, Virginia Mason Medical Center, Seattle, Washington; Sternman, Daniel H., University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; and Jablons, David M., University of California, San Francisco, San Francisco, California; *Phase I/II Study of Vaccination with Irradiated Autologous Lung Tumor Cells Mixed with a GM-CSF Secreting Bystander Cell Line (Lung Bystander GVAX®) in Advanced Non-Small Cell Lung Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 5-14-01. Not Selected for RAC Public Review: 6-4-01
Dosing portion of study completed, follow-up continues: 12-10-02

0105-473 (Open) Gene Marking/Cancer/EBV-Positive Hodgkin Disease/In Vitro/LMP2A-Specific Cytotoxic T Lymphocytes/Retrovirus/Neomycin Phosphotransferase cDNA/Adenovirus/LMP2A cDNA/Intravenous Administration

Gahn, Benedikt, Heslop, Helen, and Rooney, Cliona, Baylor College of Medicine, Houston, Texas; *Administration of Neomycin Resistance Gene Marked LMP2A-Specific Cytotoxic T Lymphocytes to Patients with Relapsed EBV-Positive Lymphoma*.

NIH/OBA Receipt Date: 5-14-01. Not Selected for RAC Public Review: 6-4-01

0105-474 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid DNA/Human and Mouse Prostate Specific Membrane Antigen cDNAs/Intramuscular Injection

Scher, Howard I., and Wolchok, Jedd, D., Memorial Sloan-Kettering Cancer Center, New York, New York; *Vaccination of Prostate Cancer Patients with Human and Mouse Specific Membrane Antigen (PSMA) DNA Vaccine: A Pilot Trial to Assess Safety and the Immune Response*.

NIH/OBA Receipt Date: 5-24-01. Not Selected for RAC Public Review: 6-14-01

0106-475 (Closed) Gene Therapy/Phase II/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Jaffee, Elizabeth M., Johns Hopkins University School of Medicine, Baltimore, Maryland; *A Safety and Efficacy Trial of Lethally Irradiated Allogeneic Pancreatic Tumor Cells Transfected with the GM-CSF Gene in Combination with Adjuvant Chemotherapy for the Treatment of Adenocarcinoma of the Pancreas*.

NIH/OBA Receipt Date: 6-11-01. Not Selected for RAC Public Review: 6-29-01
Closed to accrual: 4-22-05

0106-476 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) cDNA/Aerosol Administration

Virella-Lowell, Isabel, University of Florida, College of Medicine, Gainesville, Florida; *Evaluation of Anti-Inflammatory and Anti-Protease Pretreatment on the Delivery of Aerosolized tgAAVCF to Cystic Fibrosis Patients with Mild Lung Disease*. Sponsor: Targeted Genetics.

NIH/OBA Receipt Date: 6-11-01. Not Selected for RAC Public Review: 7-12-01
Closed, no enrollment: 8-5-04

0106-477 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Immunotherapy/In Vivo/Fowlpox Virus/B7.1 (CD80)/ICAM-1/LFA-3/Intratumoral Injection

Kaufman, Howard L, Columbia University, New York, New York; *Intra-Lesional rF-B7.1 Versus rF-TRICOM Vaccine in the Treatment of Metastatic Cancer*.

NIH/OBA Receipt Date: 6-12-01. Not Selected for RAC Public Review: 7-2-01

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0106-478 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vitro/Autologous Dendritic Cells/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/GM-CSF/Intravenous

Lyerly, H. Kim, Duke University Medical Center, Durham, North Carolina; *A Phase I Study of Active Immunotherapy with Autologous Dendritic Cells Infected with CEA-6D Expressing Fowlpox-TRICOM in Patients with Advanced or Metastatic Malignancies Expressing CEA*.

NIH/OBA Receipt Date: 6-28-01. Not Selected for RAC Public Review: 7-19-01

0106-479 (Closed) Gene Therapy/Phase I-II/Cancer/Acute Myelogenous Leukemia/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Borrello, Ivan, Johns Hopkins University School of Medicine, Baltimore, Maryland; Stock, Wendy, University of Chicago, Chicago, Illinois; Damon, Lloyd, University of California, San Francisco, San Francisco, California; and DeAngelo, Daniel, Dana Farber Cancer Institute, Boston, Massachusetts; *Vaccination in Peripheral Stem Cell Transplant Setting for Acute Myelogenous Leukemia: The Use of Autologous Tumor Cells with an Allogeneic GM-CSF Producing Bystander Cell Line*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 6-29-01. Not Selected for RAC Public Review: 7-20-01

0107-480 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase IIb/Other/Peripheral Artery Disease/End Stage Renal Disease/Stenosis Prevention/In Vivo/Adenovirus/Vascular Endothelial Growth Factor D/Perivascular Collagen Collar Device

Lawson, Jeffrey H., Duke University, Durham, North Carolina; and Schild, Frederick; Jackson Memorial Hospital, University of Miami School of Medicine; Miami, Florida; *A Phase II, Open-Label, Ascending-Dose Study of the Safety and Efficacy of Trinam™ (EG004) in Stenosis Prevention at the Graft-Vein Anastomosis Site in Dialysis Patients*. Sponsor: Ark Therapeutics, Ltd.

NIH/OBA Receipt Date: 7-6-01. Publicly Reviewed at the September 2001 RAC meeting.
Closed: 8-2-07

0107-481 (Closed) Gene Therapy/Phase Ib-II/Cancer/Brain Tumors/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection

Markert, James M., University of Alabama at Birmingham, Birmingham, Alabama; *An Open-Label, Phase Ib/II Study of the Safety, Tolerability and Efficacy of G207, a Genetically Engineered Herpes Simplex Type-1 Virus, Administered Intracerebrally to Subjects with Recurrent Malignant Glioma*. Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 7-9-01. Not Selected for RAC Public Review: 7-31-01
Closed: 05-25-12

0107-482 (Closed) Gene Therapy/Phase Ib-II/Cancer/Brain Tumors/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection

Markert, James M., University of Alabama at Birmingham, Birmingham, Alabama; *Long-Term Follow-Up of the Safety and Survival of Subjects with Recurrent Malignant Glioma Who Enrolled in a Phase Ib/II Study (protocol 0107-481) of the Safety, Tolerability and Efficacy of G207, a Genetically Engineered Herpes Simplex Type-1 Virus, Administered Intracerebrally*. Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 7-9-01. Not Selected for RAC Public Review: 7-31-01
Closed: 05-25-12

0107-483 (Closed) Gene Therapy/Phase Ib-II/Cancer/Brain Tumors/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection

Markert, James M., University of Alabama at Birmingham, Birmingham, Alabama; *Long-Term Follow-Up of the Safety and Survival of Subjects with Recurrent Malignant Glioma Who Enrolled in a Phase Ib/II Study (protocol 0107-481) of the Safety, Tolerability and Efficacy of G207, a Genetically Engineered Herpes Simplex Type-1 Virus, Administered Intracerebrally*. Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 7-9-01. Not Selected for RAC Public Review: 7-31-01
Closed: 05-25-12

0107-484 (Closed) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vivo/Plasmid in Poly (DL-lactide-coglycolide) (PLG) Microparticles/Cytochrome P450 isoenzyme 1B1 (CYP1B1) Gene/Intramuscular Injection

Gribben, John G., Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Open-Label Study of the Safety and Feasibility of Vaccinating Cancer Patients with Repeated Doses of ZYC300*. Sponsor: ZYCOS, Inc.

NIH/OBA Receipt Date: 7-11-01. Not Selected for RAC Public Review: 7-31-01
Closed to further enrollment: 8-14-03

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0107-485 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Breast/In Vitro/Autologous Bone Marrow Cells/Adenovirus/bcl-2 Dominant Negative Mutant/Bone Marrow Transplant

Clarke, Michael F., University of Michigan, Ann Arbor, Michigan; *Purging of Autologous Stem Cell Sources with bcl-x_s Adenovirus for Women Undergoing High-Dose Chemotherapy for Stage IV Breast Carcinoma*.

NIH/OBA Receipt Date: 7-11-01. Publicly Reviewed at the September 2001 RAC meeting.

0107-486 (Closed) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus-1/Replication Inhibition/In Vitro/Autologous CD34+ Cells/Retrovirus/Hammerhead Ribozyme/Intravenous

Mitsuyasu, Ronald, UCLA Medical Center, Los Angeles, California; Merigan, Thomas C., Jr., Stanford Medical Center, Stanford, California; Cooper, David, University of New South Wales/St. Vincent's Hospital, Sydney, Australia; Workman, Cassy, AIDS Research Initiative, Sydney, Australia; Bloch, Mark, Holdsworth House General Practice, Sydney, Australia; McFarlane, Robert, 407 Doctors and St. Vincent Hospital, Sydney, Australia; Finlayson, Robert, Taylor Square Private Clinic, Sydney, Australia; Kelly, Mark, Albion Street Centre, Sydney, Australia; Hoy, Jennifer, The Alfred Hospital, Melbourne, Australia; Lalezari, Jacob; Quest Clinical Research; San Francisco, California; Akil, Bisher; Health Innovations Research; New York, New York; and Smith, Don Edward; Surry Hills; Sydney, Australia; *A Randomized Phase II, Double-Blind, Controlled Trial to Evaluate the Safety and Efficacy of Autologous CD34+ Hematopoietic Progenitor Cells Transduced with Either a Delivery Gene Construct (LNL6) or LNL6 that Contains an Anti-HIV-1 Ribozyme in Patients with HIV-1 Infection*. Sponsor: Johnson & Johnson Research Pty Limited.

NIH/OBA Receipt Date: 7-11-01. Not Selected for RAC Public Review: 7-31-01
Long-term follow-up for those who received modified T cells: 1-27-09

0107-487 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other/Age-related Macular Degeneration (AMD)/Adenovirus/Pigment-Epithelium Derived Factor (PEDF) cDNA/Intravitreal Administration

Campochiaro, Peter A., Johns Hopkins University School of Medicine, Baltimore, Maryland; Klein, Michael, Oregon Health & Science University, Portland, Oregon; Holz, Eric R., Baylor College of Medicine, Houston, Texas; Gupta, Anurag; University of California, Los Angeles, Los Angeles, California; Saperstein, David, University of Washington School of Medicine, Seattle, Washington; Azrin, Michael, University of Connecticut Health Center, Farmington, Connecticut; Frank, Robert; Kresge Eye Institute, Detroit, Michigan; Katz, Randy S.; Florida Eye Microsurgical Institute, Inc.; Boynton Beach, Florida; Galllemore, Ron P.; Beverly Hills, California; Sadda, SriniVas R.; University of Southern California; Los Angeles, California; Boyer, David Stuart; Retina Vitreous Associates Medical Group; Beverly Hills, California; and Holz, Eric R.; Baylor College of Medicine; Houston, Texas; *An Open-Label, Phase I, Single Administration, Dose Escalation Study of AD_{GV}PEDF.11D (ADPEDF) in Neovascular Age-Related Macular Degeneration (AMD)*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 7-11-01. Publicly Reviewed at the September 2001 RAC meeting.

0107-488 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Infectious Diseases/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Lentivirus/HIV-1/Antisense env/Intravenous

MacGregor, Rob Roy, University of Pennsylvania Medical Center, Philadelphia, Pennsylvania; *A Phase I Open-Label Clinical Trial of the Safety and Tolerability of a Single Dose of Autologous T Cells Transduced with VRX496 in HIV Positive Patient-Subjects*. Sponsor: VIRxSYS Corporation.

NIH/OBA Receipt Date: 7-12-01. Publicly Reviewed at the September 2001 RAC meeting.
Closed to new enrollment, follow-up is ongoing: 1-24-05

0107-489 (Closed) Gene Therapy/Phase I/Other/Restenosis In Vivo/Plasmid DNA/Vascular Endothelial Growth Factor cDNA/Intraarterial/Angioplasty Catheter

Losordo, Douglas W., St. Elizabeth's Medical Center, Boston, Massachusetts; *VEGF Gene Transfer to Prevent Coronary Artery Restenosis*.

NIH/OBA Receipt Date: 7-12-01. Not Selected for RAC Public Review: 7-31-01
Recruitment discontinued: 4-20-04

0107-490 (Closed) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/Melan-A/MART-1/Intralymphnodal Injection

Weber, Jeffrey S., University of Southern California/Norris Comprehensive Cancer Center, Los Angeles, California; Hersh, Evan M., Arizona Cancer Center, Tucson, Arizona; Smith II, John W., Providence Portland Medical Center, Portland, Oregon; and Lerner, Adam, Boston University School of Medicine, Boston, Massachusetts; *A Pilot Phase I/II Study of Intranodal Delivery of a Plasmid DNA (Synchrovax SEM Vaccine) in Stage IV Melanoma Patients*. Sponsor: MannKind Corporation.

NIH/OBA Receipt Date: 7-19-01. Not Selected for RAC Public Review: 8-8-01.
Completed: 10-02-03

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0107-491 (Open) Gene Therapy/Phase I/Cancer/Follicular Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/ Autologous T Lymphocytes/Plasmid DNA/Electroporation/CE7R-Specific scFvFc-Zeta T Cell Receptor/Intravenous Infusion

Press, Oliver W., University of Washington Medical Center, Seattle Washington; *A Phase I Study to Evaluate the Safety of Cellular Immunotherapy Using Genetically-Modified Autologous CD20-Specific CD8+ T Cell Clones for Patients with Relapsed CD20+ Indolent Lymphomas*.

NIH/OBA Receipt Date: 7-26-01. Not Selected for RAC Public Review: 8-15-01

0107-492 (Withdrawn-replaced by protocol # 0110-499) Gene Therapy/Phase I/Cancer/Liver (Hepatic) Metastases/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection

Sung, Max W., Mount Sinai Medical Center, New York, New York; *Clinical Trial of Adenoviral Vector Delivery of the Herpes Thymidine Kinase (HSV-TK) Gene by Intratumoral Injection Followed by Intravenous Ganciclovir with Imaging of HSV1-tk Gene Expression in Patients with Hepatic Metastases from Colorectal Cancer*.

NIH/OBA Receipt Date: 7-27-01.

0107-493 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Subcutaneous Injection

Corman, John M., Virginia Mason Medical Center, Seattle, Washington; Simons, Jonathan, Emory University, Atlanta, Georgia; Small, Eric, University of California, San Francisco, San Francisco, California; Higano, Celestia, University of Washington, Seattle, Washington; Smith, David, University of Michigan Medical Center, Ann Arbor, Michigan; Hudes, Gary R., Fox Chase Cancer Center, Philadelphia, Pennsylvania; Centeno, Arthur, Urology San Antonio Research, San Antonio, Texas; Gittelman, Marc, South Florida Medical Research, Aventura, Florida; Steidle, Christopher, Northeast Indiana Research, Fort Wayne, Indiana; Dula, Eugene, West Coast Clinical Research, Van Nuys, California; Young, Jay M., South Orange County Medical Research Center, Laguna Woods, California; and Rowland, Kendrith M., Jr., Carle Cancer Center, Urbana, Illinois; *A Phase I/II Dose Escalation and Efficacy Trial of GVAX® Prostate Cancer Vaccine in Patients with Metastatic Hormone-Refractory Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 7-18-01. Not Selected for RAC Public Review: 8-31-01
Closed: 7-14-06

0108-494 (Open) Gene Therapy/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/In Vitro/Autologous CD34+ Cells from Cord Blood or Bone Marrow/Retroviral Vector/γc cDNA/Intravenous Infusion

Weinberg, Kenneth I., Children's Hospital of Los Angeles, University of Southern California School of Medicine, Los Angeles, California; *Gene Transfer of the γcDNA into CD34+ Hematopoietic Cells of Infants or Children with X-Linked Severe Combined Immune Deficiency (X-SCID)*.

NIH/OBA Receipt Date: 8-27-01. Not Selected for RAC Public Review: 9-17-01

0108-495 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/DF3/MUC1/B7.1 (CD 80)/ICAM-1/LFA-3/Intradermal Injection

Eder, Joseph Paul, Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase I Trial of Recombinant Vaccinia Viruses that Express DF3/MUC1 and TRICOM (B7.1, ICAM-1, and LFA-3) in Patients with Metastatic Adenocarcinoma of the Breast*.

NIH/OBA Receipt Date: 8-27-01. Not Selected for RAC Public Review: 3-6-02

0108-496 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Plasmid DNA/Electroporation/IL13R α 2-Specific scFvFc-Zeta T Cell Receptor/Intracavity Administration

Jensen, Michael, City of Hope National Medical Center, Duarte, California; *Cellular Immunotherapy Using Autologous CD8+ T Cell Clones Genetically Modified to Express the IL13-Zetakine and HyTK for Recurrent Malignant Glioma*.

NIH/OBA Receipt Date: 8-30-01. Not Selected for RAC Public Review: 9-21-01

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0109-497 (Closed) Gene Therapy/Phase II/Cancer/MUC-1 Expressing Prostate Cancer/Immunotherapy/In Vivo/Vaccinia Virus/MUC-1/Interleukin-2/Subcutaneous Injection

Pantuck, Allan J., University of California, Los Angeles, Los Angeles, California; Dreicer, Robert, Cleveland Clinic Foundation, Cleveland, Ohio; Conlon, Kevin, Rush-Presbyterian-St. Luke's Medical Center, Chicago, Illinois; Sahasrabudhe, Deepak, University of Rochester Medical Center, Rochester, New York; Higano, Celestia, Seattle Cancer Care Alliance, Seattle, Washington; Ahmann, Frederick, University of Arizona Cancer Center, Tucson, Arizona; and Stadler, Walter, University of Chicago, Chicago, Illinois; *Randomized Multicenter Phase II Study Evaluating Two Dosing Schedules of TG4010 (MVA-MUC1-IL2) in Patients with Adenocarcinoma of the Prostate*. Sponsor: Transgene, Inc.

NIH/OBA Receipt Date: 9-12-01. Not Selected for RAC Public Review: 10-9-01
Closed to enrollment: 5-05

0109-498 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfusion/Human Telomerase Reverse Transcriptase (hTERT)/Intradermal Injections

Vieweg, Johannes; University of Florida; Gainesville, Florida; *Phase I Study of Active Immunotherapy of Metastatic, Hormone Refractory Prostate Carcinoma Using Autologous Mature Dendritic Cells (DC) Transfected with RNA Encoding Human Telomerase Reverse Transcriptase (hTERT)*.

NIH/OBA Receipt Date: 9-21-01. Not Selected for RAC Public Review: 10-26-01

0110-499 (Open) Gene Therapy/Phase I/Cancer/Liver (Hepatic) Metastases of Colorectal Cancer/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Ganciclovir/Intratumoral Injection

Sung, Max W., Mount Sinai Medical Center, New York, New York; *Clinical Trial of Adenoviral Vector Delivery of the Herpes Thymidine Kinase (HSV-tk) Gene by Intratumoral Injection Followed by Intravenous Ganciclovir with Imaging of HSV-tk Gene Expression in Patients with Hepatic Metastases from Colorectal Cancer*.

NIH/OBA Receipt Date: 10-9-01. Not Selected for RAC Public Review: 10-30-01

0110-500 (Open) Gene Therapy/Phase I/Cancer/Bladder/Pro-Drug/Ganciclovir/In Vivo/Adenovirus/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection

Lerner, Seth P., Baylor College of Medicine, Houston, Texas; *Phase I Trial of Adenoviral Mediated Suicide Gene Therapy with HSV-tk Followed by Intravenous Administration of Ganciclovir in Patients with Locally Advanced and Refractory Superficial Bladder Cancer*.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

0110-501 (Open) Gene Marking/Osteogenesis Imperfecta/In Vitro/CD34+ Cells from Donor Bone Marrow/Retrovirus/Neomycin Phosphotransferase cDNA/Intravenous Infusion

Horowitz, Edwin M., St. Jude Children's Research Hospital, Memphis, Tennessee; *Treatment of Children with Severe Osteogenesis Imperfecta by Stem Cell Transplantation and Mesenchymal Cell Graft Augmentation (Pilot Study)*.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

0110-502 (Closed) Gene Therapy/Phase II/Peripheral Artery Disease/Plasmid DNA/Fibroblast Growth Factor 1 cDNA/Intramuscular Injection

Comerota, Anthony J., Jobst Vascular Center, Toledo, Ohio; Mendelsohn, Farrell O., Cardiology, P.C., Birmingham, Alabama; Garza, Luis., Central Arkansas Veterans Healthcare System, Little Rock, Arkansas; Goldman, Corey K, Watson Clinic LLP, Lakeland, Florida; Greenbaum, Adam, Henry Ford Hospital, Detroit, Michigan; Sequeira, Rafael, Jackson Memorial Hospital, Miami, Florida; Henry, Timothy, Minneapolis Heart Institute Foundation, Minneapolis, Minnesota; Miller, Julie, The Johns Hopkins University, Baltimore, Maryland; Gray, John, Durham VA Medical Center, Durham, North Carolina; Hermiller, James and Irwin, Randy, St. Vincent Hospital and Health Care Center, Indianapolis, Indiana; Moneta, Gregory, Oregon Health & Science University, Portland, Oregon; Laird, John, Washington Hospital Center, Washington, DC; Chronos, Nicolas, Atlanta Cardiology Group, Atlanta, Georgia; Kent, K. Craig, Weill Medical College of Cornell University, New York, New York; Grossman, P. Michael, The University of Michigan Health Systems, Ann Arbor, Michigan; Eslami, Mohammad, Temple University, Philadelphia, Pennsylvania; Azrin, Michael, University of Connecticut Health Center, Farmington, Connecticut; Goldman, Corey, Ochsner Clinic Foundation, New Orleans, Louisiana; Rocha-Singh, Krishna, Prairie Heart Institute, Springfield, Illinois; and Lowman, Bruce G., Watson Clinic LLP, Lakeland, Florida; *A Phase II, Randomized, Double-Blind, Placebo Controlled, Parallel Group, Efficacy and Safety Study of Different Doses and Schedules of Administration of NV1FGF in Patients with Severe Peripheral Artery Occlusive Disease*. Sponsor: Aventis Pharma.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 11-15-01
Closed to enrolment; long-term follow-up is ongoing: 12-20-04

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0110-503 (Closed) Gene Therapy/Phase I/Monogenic Disease/Cystic Fibrosis/In Vivo/Nasal Epithelial Cells/Cystic Fibrosis Transmembrane Conductance Regulator cDNA/Polylysine Polyethylene Glycol Complex/Intranasal Administration

Konstan, Michael W., Case Western Reserve University, Cleveland, Ohio; and Wagener, Jeffrey, University of Colorado School of Medicine, Denver, Colorado; *Single Dose Escalation Study to Evaluate Safety of Nasal Administration of CFTR001 Gene Transfer Vector to Subjects with Cystic Fibrosis*.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01
Completed: 11-19-03

0110-504 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor α and β Chain cDNA/Intravenous Infusion

McDonagh, Kevin T., The University of Michigan Health System, Ann Arbor, Michigan; *A Phase I Study of Genetically Modified Autologous Peripheral Blood T-Cells Expressing a Retrovirally Encoded, MART-1 Specific $\alpha\beta$ T-Cell Receptor, With and Without Recombinant Human Interleukin-2, in HLA-A2+*.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

0110-505 (Closed) Gene Therapy/Phase I/Cancer/MUC1 Expressing Carcinoma/Immunotherapy/In Vivo/Naked Plasmid DNA/MUC-1/Intramuscular Injection

Avigan, David E., Beth Israel Deaconess Medical Center, Boston, Massachusetts; *A Phase I Open-Label Study to Assess the Safety and Toxicity of Plasmid DNA MUC1 Vaccine (pMC6.5) in Metastatic Carcinoma*. Sponsor: Centocor, Inc.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 11-12-01
Study never initiated: 4-18-03

0110-506 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/In Vitro/autologous T-Lymphocytes/Immunotherapy/Retrovirus/Interleukin-2 cDNA/Intravenous or Intra-arterial Infusion

Rosenberg, Steven A., National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma Using Lymphocytes Transduced with an Interleukin-2 (IL-2) Gene Following the Administration of a Nonmyeloablative but Lymphocyte Depleting Regimen*.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 11-14-01
Data analysis ongoing: 9-19-07

0110-507 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal Injection

Wolchok, Jedd D., Memorial Sloan-Kettering Cancer Center, New York, New York; *Vaccination of AJCC Stage IIB, IIC, III and IV Melanoma Patients with a Multi-Epitope Peptide Vaccine Using GM-CSF DNA as an Adjuvant: A Pilot Trial to Assess Safety and Immunity*.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

0110-508 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Antisense/Antisense TAR/Antisense tat/rev/In Vitro/CD34+ Cells/Intravenous

Krishnan, Amrita, City of Hope National Medical Center, Duarte, California; *Evaluation of the Safety and Efficacy of ex vivo Modification and Re-Infusion of CD34+ Cells by an Antisense Construct against HIV-1 in a Retrovirus Vector*. Sponsor: Enzo Therapeutics, Inc.

NIH/OBA Receipt Date: 10-10-01. Not Selected for RAC Public Review: 10-30-01

0111-509 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Vector Directed Cell Lysis/In Vivo/Adenovirus/Serotype 5/Repication-Comptetent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen Gene/Intratumoral Injection

Corman, John M., Virginia Mason Medical Center, Seattle Washington; *A Phase I/II Trial of Intraprostatic Injection of CG7060 Followed by Three-Dimensional Conformal Radiation Therapy (3D-CRT) in Patients with Clinically Localized Intermediate or High-Risk Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 11-9-01. Not Selected for RAC Public Review: 12-03-01
Study was never initiated

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0111-510 (Closed) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Prostate Specific Antigen/Intradermal Injection

Vieweg, Johannes, Duke University Medical Center, Durham, North Carolina; *Pilot Study evaluating the Migratory Patterns of Immature and In Vitro Matured Dendritic Cells Transfected with RNA Encoding PSA in Patients with Metastatic Prostate Cancer*.

NIH/OBA Receipt Date: 11-20-01. Not Selected for RAC Public Review: 12-11-01
Closed: 2-02-04

0112-511 (Open) Gene Therapy/Phase I/Cancer Squamous Cell Carcinoma of the Head and Neck (SCCHN)/Immunotherapy/In Vitro/Allogeneic Tumor Cell/Retrovirus/Interleukin-2 cDNA/Intradermal Injection

Johnson, Jonas T., University of Pittsburgh, Pittsburgh, Pennsylvania; *Active Immunization of Patients with Carcinoma of Oral Cavity or Oropharynx with Interleukin-2-Secreting Semi-allogeneic Human Carcinoma Cell Line Transfected with DNA from Autologous Tumor (Phase I Study)*.

NIH/OBA Receipt Date: 12-20-01. Not Selected for RAC Public Review: 1-14-02

0112-512 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Cachexia/In Vivo/Plasmid/Chimeric Transactivator of Progesterone Receptor-Ligand-Binding Domain Fused to the Gal4 DNA Binding Domain/Human Growth Hormone Releasing Hormone (GHRH) cDNA/Intramuscular Injection

Popat, Uday, Baylor College of Medicine, Houston, Texas; *Phase I Study of Human Growth Hormone Releasing Hormone Expressed by a Plasmid DNA Myogenic Vector in Patients with Cancer Cachexia*.

NIH/OBA Receipt Date: 12-21-01. Publicly Reviewed at the March 2002 RAC meeting

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0201-513 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer (NSCLC)/In Vivo/Tumor Suppressor Gene/Cationic Liposome Complex/DOTAP:Cholesterol/Fus 1 cDNA/Intravenous Injection

Lu, Charles, The University of Texas, M.D. Anderson Cancer Center, Houston, Texas; *Phase I Study of Intravenous DOTAP:Cholesterol-Fus 1 Liposome Complex (DOTAP:Chol-Fus 1) in Patients with Advanced Non-Small Cell Lung Cancer (NSCLC) Previously Treated with Chemotherapy*.

NIH/OBA Receipt Date: 01-08-02. Publicly Reviewed at the March 2002 RAC meeting
Closed to accrual: 2-15-06

0201-514 (Open; RAC Reviewed with Recommendations) Gene Marking/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Serotype 2/Human Placental Alkaline Phosphatase (AP or hpAP) cDNA/Nasal and Bronchial Administration

Aitken, Moira L., and Miller, A. Dusty, University of Washington, Seattle, Washington; *Transduction of the Upper and Lower Airway Epithelium in Healthly Subjects by an AAV2 Vector that Encodes Human Placental Alkaline Phosphatase*.

NIH/OBA Receipt Date: 01-09-02. Publicly Reviewed at the March 2002 RAC meeting

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0201-515 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/In Vivo/Herpes Simplex Virus Type 1/HSV Thymidine Kinase (TK), Connexin 43, Tumor Necrosis Factor Alpha (TNF-a), and the Viral Infected Cell Protein Zero (ICP0) Genes/Ganciclovir/Intratumoral (Stereotactic) Injections

Lunsford, L. Dade, and Glorioso, Joseph C., University of Pittsburgh, School of Medicine, Pittsburgh, Pennsylvania; *Gene Therapy of Progressive Glioblastoma Multiforme Using a Replication Defective HSV Multigene Vector NUREL-C2: A Phase I Clinical Trial to Determine the Maximum Tolerable Dose of Vector in Combination with Ganciclovir and Radiosurgery*.

NIH/OBA Receipt Date: 01-09-02. Publicly Reviewed at the March 2002 RAC meeting

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0201-516 (Closed) Gene Therapy/Phase I-II/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/In Vitro/Autologous CD34+ Cells from Peripheral Blood/Retrovirus γcDNA/Intravenous Infusion

Malech, Harry L., National Institutes of Health, Bethesda, Maryland; *Ex Vivo Retroviral Gene Transfer for Treatment of X-Linked Severe Combined Immunodeficiency (XSCID)*.

NIH/OBA Receipt Date: 1-15-02. Not Selected for RAC Public Review: 2-5-02
Long-term follow-up: 2-03-09. Last administration of modified cells: July 2005

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0203-517 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Interferon-beta cDNA/Intratumoral Injection

Dinney, Colin P., University of Texas M.D. Anderson Cancer Center; *An Open Label, Dose-Escalation Study to Determine the Tolerability of Interferon-beta (BG00001) Gene Transfer in the Neoadjuvant Treatment of High-Risk Resectable Prostate Cancer*. Sponsor: Biogen Idec, Inc.

NIH/OBA Receipt Date: 3-5-02. Not Selected for RAC Public Review: 3-25-02

0203-518 (Submission Not Complete) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Fowlpox Virus/Prostate Specific Antigen/Intradermal Injection

Phase II Randomized Study of Fowlpox PSA Vaccine with and without GM-CSF in the Treatment of Advanced Prostate Cancer. Sponsor: Eastern Cooperative Oncology Group

NIH/OBA Receipt Date: 3-11-02.

0203-519 (Closed) Gene Therapy/Phase II/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Laheru, Daniel, John Hopkins University School of Medicine, Baltimore, Maryland; and Nemunaitis, John J., Mary Crowley Medical Research Center, Dallas, Texas; *A Phase II Trial of CG8020 and CG2505 in Patients with Nonresectable or Metastatic Pancreatic Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 3-15-02. Not Selected for RAC Public Review: 4-4-02
Closed to accrual: 3-13-02

0203-520 (Open) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/Pro-Drug/Ganciclovir/In Vitro/Allogeneic T Lymphocytes/Retrovirus/Herpes Simplex Thymidine Kinase cDNA/Graft-Versus-Host Disease/Intravenous Infusion

Orchard, Paul J., University of Minnesota Medical School, Minneapolis, Minnesota; *Transplantation of Unrelated or Mismatched Related Donor T Cells Containing the HSV-TK Suicide Gene to Facilitate Engraftment and Control Graft-Versus-Host Disease in Patients with Fanconi Anemia. A Phase I Trial*.

NIH/OBA Receipt Date: 3-21-02. Not Selected for RAC Public Review: 6-21-02

0204-521 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Peripheral Artery Disease/End Stage Renal Disease/Stenosis Prevention/In Vivo/Adenovirus/Inducible Nitric Oxide Synthase (iNOS) cDNA/Administration at the Arteriovenous (AV) Graft for Hemodialysis Access

Tzeng, Elizabeth, University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *Inducible Nitric Oxide Synthase Gene Therapy for the Prevention of Intimal Hyperplasia in Arteriovenous Grafts used for Hemodialysis Access*.

NIH/OBA Receipt Date: 4-1-02. Publicly Reviewed at the June 2002 RAC meeting

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0204-522 (Open) Gene Therapy/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen cDNA/B7.1 (CD80) cDNA/Intramuscular or Intradermal Injection

Dahut, William, National Institutes of Health, Bethesda, Maryland; *A Pilot Trial of Concurrent Docetaxel and Pox Vector PSA Vaccine Followed by Docetaxel in Metastatic Androgen Independent Prostate Cancer.*

NIH/OBA Receipt Date: 4-9-02. Not Selected for RAC Public Review: 4-29-02

0204-523 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Ovarian/Vector Directed Cell Death/Measles Virus/Carcinoembryonic Antigen (CEA) cDNA/Intraperitoneal Administration

Galanis, Evanthia, Mayo Clinic, Rochester, Minnesota; *Phase I Trial of Intraperitoneal Administration of a CEA Expressing Derivative Manufactured from a Genetically Engineered Strain of Measles Virus in Patients with Recurrent Ovarian Cancer*.

NIH/OBA Receipt Date: 4-11-02. Publicly Reviewed at the June 2002 RAC meeting

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0204-524 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Intramuscular or Intradermal Injection

Arlen, Philip M., National Institutes of Health, Bethesda, Maryland; *A Pilot Study of Sequential Vaccinations with Recombinant Vaccinia-CEA(6D)-TRICOM, and Recombinant Fowlpox-CEA(6D)-TRICOM (B7.1/ICAM-1/LFA-3) with Sargramostim (GM-CSF) , in Conjunction with Standard Adjuvant Chemotherapy in High Risk Breast Cancer Patients Status Post Surgery with 4+ or More Lymph Nodes and CEA Expressing Tumors*

NIH/OBA Receipt Date: 4-16-02. Not Selected for RAC Public Review: 5-6-02

0204-525 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal Administration

Wolf, Judith K., The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Dose Escalation Study of Intraperitoneal tgDCC-E1A and Intravenous Carboplatin for Treatment of Recurrent, Platinum-Sensitive Ovarian Cancer*. Sponsor: Targeted Genetics Corp.

NIH/OBA Receipt Date: 4-24-02. Not Selected for RAC Public Review: 5-14-02
No longer active: 11-22-02

0204-526 (Closed) Gene Therapy/Phase I/Cancer/Colon Carcinoma (Hepatic Metastasis)/Herpes Simplex Virus Type-1/Tumor Lysis/Intrahepatic Artery Administration

Fong, Yuman, Memorial Sloan-Kettering Cancer Center, New York, New York; *A Phase I, Open-Label, Dose-Escalating Study of Safety, Tolerability, and Anti-Tumor Activity of a Single Intrahepatic Arterial Injection of a Genetically Engineered Herpes Simplex Virus, NV1020, in Herpes Simplex Seronegative Subjects with Adenocarcinoma of the Colon with Metastasis to the Liver*. Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 4-23-02. Not Selected for RAC Public Review: 5-13-02
Never initiated: 9-25-06

0204-527 (Closed) Gene Therapy/Phase I/Cancer/Colon Carcinoma (Hepatic Metastasis)/Herpes Simplex Virus Type-1/Tumor Lysis/Intrahepatic Artery Administration

Fong, Yuman, Memorial Sloan-Kettering Cancer Center, New York, New York; *Long-Term Follow-Up of the Safety and Survival of HSV Simplex Seronegative Subjects with Adenocarcinoma of the Colon with Metastasis to the Liver Who Enrolled in a Phase I Dose-Escalating Study Evaluating Genetically Engineered Herpes Simplex Virus, NV1020*. Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 4-23-02. Not Selected for RAC Public Review: 5-13-02
Never initiated: 9-25-06

0204-528 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Disorders/Erectile Dysfunction/In Vivo/Plasmid/Human Maxi-K Channel hSlo cDNA/Intracavernous Injection

Bar-Chama, Natan; Mt. Sinai School of Medicine., New York, New York; *Pilot Study of the Human hslo/maxi-K Gene to Treat Erectile Dysfunction*.

NIH/OBA Receipt Date: 4-23-02. Publicly Reviewed at the June 2002 RAC meeting

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0204-529 (Open; RAC Reviewed with Recommendations) Gene Therapy/Other Disorders/Intractable Pain/In Vivo/Herpes Simplex Virus Type 1/Proenkephalin/Subcutaneous Inoculation

Fink, David, and Glorioso, Joseph, University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *Gene Transfer for Intractable Pain: A Phase I Clinical Trial to Determine the Maximum Tolerable Dose of a Replication Defective HSV Vector Expressing Human Proenkephalin*.

NIH/OBA Receipt Date: 4-24-02. Publicly Reviewed at the June 2002 RAC meeting

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0204-530 (Closed) Gene Therapy/Phase II/Cancer/Pancreatic Cancer/Immunotherapy/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection

Senzer, Neil Nathan, U.S. Oncology, Inc./Mary Crowley Medical Research Center, Dallas Texas; Richards, Donald, Tyler Care Center, Tyler, Texas; Hecht, J. Randolph, UCLA Medical Center, Los Angeles , California; Kenady, Daniel E., University of Kentucky, Lexington, Kentucky; Chung, Theodore D.K., Virginia Commonwealth University, Richmond, Virginia; Vogel, Stephen, The University of Florida, Gainesville, Florida; Reid, Tony, University of California, San Diego Cancer Center, La Jolla, California; Chang, Kenneth J., University of California, Irvine, Orange, California; Javle, Milind, Roswell Park Cancer Institute, Buffalo, New York; Wong, Lucas, Scott & White Memorial Hospital and Clinic, Temple, Texas; Rosemurgy, Alexander, University of South Florida, Tampa, Florida; Posner, Mitchell C., University of Chicago, Chicago, Illinois; Perry, David, Washington Cancer Institute, Washington, D.C. Kallab, Andre, Medical College of Georgia, Augusta, Georgia; Sharma, Anand K., Medical University of South Carolina; Charleston, South Carolina; Brell, Joanna M., Case Western University Hospitals, Cleveland, Ohio; Harris, Hobart, San Francisco General Hospital, San Francisco, California; Wadler, Scott; Weill Medical College, Cornell University, New York, New York; Canto, Marcia, Johns Hopkins Hospital, Baltimore, Maryland; Pinto, Harlen, VA Palo Alto Health Care System, Palo Alto, California; Strasberg, Steven M., Washington University School of Medicine, St. Louis, Missouri; Shirinian, Mihran; Gendale Adventist Medical Center; Glendale, California; Harris, Hobart W.; University of California, San Francisco; San Francisco, California; Ritch, Paul, Froedtert Hospital; Milwaukee, Wisconsin; Strasberg, Steven M.; Washington University School of Medicine; St. Louis, Missouri; Khan, M. Qaseem; Marshfield Clinic Research Foundation, Marshfield, Wisconsin; Talavera, Joyce; Tuft-New England Medical Center; Boston, Massachusetts; Owens, Michael M.; West Hills Gastroenterology Associates; Portland, Oregon; Stephenson, Joe; Cancer Centers of the Carolinas; Greenville, South Carolina; Reid, Tony; Moores University of California San Diego Cancer Center; Marshall, John, Lombardi Cancer Center, Georgetown University; Washington, DC; Fisher, William; Baylor College of Medicine; Houston, Texas; Khong, Hung; University of South Alabama; Mobile, Alabama; Khan, Mohamamad; Marshfield Clinic; Marshfield, Wisconsin; Posner, Mitchell C.; University of Chicago Hospital; Chicago, Illinois; Shirinian, Mihran; Glendale Adventist Medical Center; Glendale, California; Strauss, James; The Presbyterian Hospital of Dallas; Dallas, Texas; Fisher, William E.; Baylor College of Medicine; Houston, Texas; Tokar Margarita; University Medical Center Soroka; Be'er Sheva, Israel; Schnirer, Isac; Wolfson Hospital, Holon, Israel; Epelbaum, Ron; Rambam Medical Center; Haifa, Israel; Chung, Theodore; Virginia Commonwealth University; Richmond, Virginia; VonHoff, Daniel D.; Scottsdale Clinical Research Institute; Phoenix, Arizona; Stephenson, Joe J., Jr.; Cancer Centers of the Carolinas; Greenville, South Carolina; Richards, Donald A.; Tyler Cancer Center; Tyler, Texas; Chang, Kenneth J.; University of California, Irvine; Orange, California; Laheru, Daniel; Johns Hopkins University; Baltimore, Maryland; Strasberg, Steven; Washington University School of Medicine; St. Louis, Missouri; Reid, Tony; University of California at San Diego Cancer Center; San Diego, California; Stemmer, Salomon; Davidoff Center Rabin Medical Center; Petach Tikva, Israel; Aderka, Dan; The Chaim Sheba Medical Center; Tel Hashomer, Israel; Karamlou, Kasra; Pacific Oncology, P.C.; Portland, Oregon; Gurtler, Jayne; East Jefferson General Hospital; Metairie, Louisiana; Iyer, Robert; Roswell Park Cancer Institute; Buffalo, New York; Elahi, Riaz; St. James Hospital and Health Centers; Olympia Fields, Illinois; Holladay, Charles S.; Charleston Cancer Center; Dragovich, Tomislav; Arizona Cancer Center; Burke, James M.; Billings Clinic; Billings, Montana; Stevens, Tyler; The Cleveland Clinic; Cleveland, Ohio; Ayub, Kamran; Virginia Mason Medical Center; Seattle, Washington; Robbins, David Herbert; Beth Israel Medical Center; New York, New York; Theall, Kathy Puyana; Tufts-New England Medical Center; Boston, Massachusetts; Patel, Aalpen; University of Pennsylvania; Philadelphia, Pennsylvania; Klapman, Jason; H. Lee Moffitt Cancer Center & Research Institute; Tampa, Florida; Crocenzi, Todd S.; West Hills Gastroenterology Assoc., P.C.; Portland, Oregon; Khandekar, Janardan Dinkar; Evanston Northwestern Healthcare; Evanston, Illinois; Halline, Allan G.; University of Illinois at Chicago; Chicago, Illinois; Arriaga, Yull; University of Texas Southwestern Medical Center at Dallas; Dallas, Texas; Nguyen, Cuong C.; Mayo Clinic; Scottsdale, Arizona; Jain, Sanjay Ray; Beth Israel Deaconess Medical Center; Boston, Massachusetts; Philip, Philip A.; Karmanos Cancer Institute; Detroit, Michigan; Zervos, Emmanuel E.; The Brody School of Medicine at East Carolina University; Greenville, North Carolina; Abayomi, Alubunmi K.; Virginia Commonwealth University; Richmond, Virginia; Lenz, Heinz-Josef; University of Southern California; Los Angeles, California; Ramanathan, Ramesh K.; Scottsdale Clinical Research Institute; Scottsdale, Arizona; Obel, Jennifer; Evanston Northwestern Healthcare; Evanston, Illinois; Saccaro, Steven J; Christus St. Frances Cabrini Hospital; Alexandria, Louisiana; and Jafri, Syed F.; Kansas City Gastroenterology Hepatology; Kansas City, Missouri; Sung, Max W.; Mount Sinai Medical Center; New York, New York; Kozuch, Peter S.; Beth Israel Medical Center; New York, New York; Milsad, Rebecca; Beth Israel Deaconess Medical Center; Boston, Massachusetts; and Ross, Sharona B.; University of South Florida; Tampa, Florida; *A Randomized, Phase II, Study of TNFerade™ Biologic with 5-FU and Radiation Therapy for First-Line Treatment of Unresectable Locally Advanced Pancreatic Cancer*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 4-24-02. Not Selected for RAC Public Review: 5-14-02
Closed: 5-30-10

0204-531 (Open) Gene Therapy/Cancer/Mesothelioma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interferon-beta/Intrapleural Administration

Sterman, Daniel, University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Phase I Trial of Intrapleural Gene Therapy of Malignant Pleural Disease Using E1-Deleted Adenoviruses Containing the Human Interferon Beta Gene.*

NIH/OBA Receipt Date: 4-24-02. Not Selected for RAC Public Review: 5-14-02

0204-532 (Closed) Gene Therapy/Other Disorders/Peripheral Arterial Occlusive Disease (PAOD)/In Vivo/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intramuscular Injection

Haser, Paul B., St. Michael's Medical Center, Newark, New Jersey; *Double-Blind, Randomized, Placebo-Controlled Study of Ascending Doses on Tolerability of Ad5.1 Mediated Human FGF-4 Gene Transfer Given Intramuscularly on One Day in Patients with Peripheral Arterial Occlusive Disease (PAOD) Fontaine Stage III or Fontaine Stage IV.* Sponsor: Berlex Laboratories.

NIH/OBA Receipt Date: 4-24-02. Not Selected for RAC Public Review: 7-10-02
Closed: 12-16-02

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0204-533 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Prostate/Radiotherapy/In Vivo/Adenovirus/Serotype 5/Human Sodium-Iodide Symporter (NIS) cDNA/Intratumoral Injection

Morris, John C., Mayo Clinic, Rochester, Minnesota; *Phase I Trial of In Situ Gene Therapy for Locally Recurrent Prostate Cancer Following Radiation Therapy Failure Using Sodium/Iodide Symporter and Radioiodine*.

NIH/OBA Receipt Date: 4-25-02. Publicly Reviewed at the June 2002 RAC meeting

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0204-534 (Open; RAC Reviewed with Recommendations) Non-therapeutic (Healthy Volunteers)/Phase I/In Vivo/Adenovirus/Serotype 4/Human Immunodeficiency Virus-1 env Plus rev or gag/Protease Plus rev Inserted Genes/Oral or Intranasal Administration

Connors, Mark, National Institutes of Health, Bethesda, Maryland; *Phase I Study of AD4-ΔE3-HIV$_{env}$ and AD4-ΔE3-HIV$_{gag/pro}$ Recombinant Vaccines in HIV-negative Volunteers*.

NIH/OBA Receipt Date: 4-24-02. Publicly Reviewed at the June 2002 RAC meeting

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0205-535 (Open) Gene Therapy/Phase I/Cancer/Acute Lymphoblastic Leukemia (ALL)/Immunotherapy/In Vitro/Plasmid DNA/Chimeric T Cell Receptor (CD19R) cDNA/Fusion Gene Encoding Hygromycin Phosphotransferase and Herpes Simplex Thymidine Kinase (HyTK)/Intravenous Infusion

Cooper, Laurence J. N., City of Hope Medical Center, Duarte, California; *Phase I Study to Evaluate the Safety of Cellular Immunotherapy for High-Risk CD19+ Acute Lymphoblastic Leukemia after Autologous Hematopoietic Stem Cell Transplantation Using Genetically Modified CD19-redirected Autologous Cytolytic T Cell Clones*.

NIH/OBA Receipt Date: 5-6-02. Not Selected for RAC Public Review: 5-28-02

0205-536 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection

Kaufman, Howard L., Columbia University, New York, New York; Plante, Mark, The University of Vermont, Burlington, Vermont; DiPaola, Robert, Robert Wood Johnson Medical School, New Brunswick, New Jersey; and Israeli, Ronald, Staten Island Urological Research PC, Staten Island, New York; *Phase I Open Label Study to Evaluate the Safety of PROSTVAC-VF-TRICOM in the Treatment of Subjects with Adenocarcinoma of the Prostate*. Sponsor: Therion Biologics Corporation.

NIH/OBA Receipt Date: 5-16-02. Not Selected for RAC Public Review: 6-26-02

0205-537 (Open) Gene Therapy/Phase I-II/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Intramuscular Or Intradermal Injection

Kasten-Sportes, Claude, National Institutes of Health, Bethesda, Maryland; *A Phase I-II Study of Tumor Antigen (CEA) Immunization with Autologous Peripheral Progenitor Cell Transplantation in Patients Previously Untreated for Metastatic Breast Cancer*.

NIH/OBA Receipt Date: 5-24-02. Not Selected for RAC Public Review: 6-14-02

0205-538 (Open) Gene Therapy/Phase I-II/Cancer/Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/p53 cDNA/Intradermal Injection

Antonia, Scott J., University of South Florida, Tampa, Florida; *A Phase I-II Trial Using Dendritic Cells Transduced with an Adenoviral Vector Containing the p53 Gene to Immunize Patients with Extensive Stage Small Cell Lung Cancer after Standard Chemotherapy*.

NIH/OBA Receipt Date: 5-24-02. Not Selected for RAC Public Review: 6-14-02

0206-539 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Other Disorders/Superficial Corneal Opacity/Corneal Scarring/InVivo/Retrovirus/dnG1 Cyclin/Eye Administration (Ophthalmic Instillation)

Song, Jonathan C., Keck School of Medicine, University of Southern California, Los Angeles, California; *Phase I/II Evaluation of Safety and Efficacy of a Matrix-Targeted Retroviral Vector Bearing a Dominant Negative Cyclin G1 Construct (Mx-dnG1) as Adjunctive Intervention for Superficial Corneal Opacity/Corneal Scarring*.

NIH/OBA Receipt Date: 6-5-02. Publicly Reviewed at the September 2002 RAC meeting

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0206-540 (Closed) Gene Therapy/Phase I/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intraperitoneal Administration

Wolf, Judith K., The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Dose Escalation Study of Intraperitoneal tgDCC-E1A and Intravenous Paclitaxel in Women with Platinum-Resistant Ovarian Cancer*. Sponsor: Targeted Genetics Corp.

NIH/OBA Receipt Date: 6-6-02. Not Selected for RAC Public Review: 6-26-02
Never initiated: 11-22-02

0206-541 (Open) Gene Therapy/Phase I-II/Cancer/Breast/Immunotherapy/In Vivo/Naked Plasmid/Gene Encoding NY-ESO-1 Epitope/Intralymphnodal Injection

Waisman, James R., University of Southern California, Los Angeles, California; *A Phase I/II Study of Intranodal Delivery of Synchrovax BPL Vaccine, an Epitope Synchronization Plasmid DNA Vaccine, in Sage IV Breast Carcinoma Patients*. Sponsor: CTL ImmunoTherapies Corporation.

NIH/OBA Receipt Date: 6-13-02. Not Selected for RAC Public Review: 7-18-02

0207-542 (Open) Gene Therapy/Phase I-II/Cancer/Pancreas/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection

Fernandez-del Castillo, Carlo, Massachusetts General Hospital, Boston, Massachusetts; Aguilar-Cordova, Estuardo, Harvard Gene Therapy Initiative, Boston, Massachusetts; Fisher, William, Baylor College of Medicine, Houston, Texas; Bloomston, Mark; The Ohio State University; Columbus, Ohio and Chung, Vincent; City of Hope; Duarte, California; *AdV-tk Gene Therapy in Combination with Chemoradiation for Locally Advanced Pancreatic Cancer*.

NIH/OBA Receipt Date: 7-17-02. Not Selected for RAC Public Review: 8-6-02

0207-543 (Closed) Gene Therapy/Phase I/Cancer/Follicular Lymphoma/Immunotherapy/In Vitro/Plasmid DNA/Chimeric T Cell Receptor (CD19R) cDNA/Fusion Gene Encoding Hygromycin Phosphotransferase and Herpes Simplex Virus Thymidine Kinase

Jensen, Michael, City of Hope National Medical Center, Duarte, California; *Phase I Study to Evaluate the Safety of Cellular Immunotherapy for CD 19+ Follicular Lymphoma Using Autologous T Cell Cytolytic Clones Genetically Modified to be CD19-Specific and Express HyTK*.

NIH/OBA Receipt Date: 7-18-02. Not Selected for RAC Public Review: 8-7-02
Closed: 02/13/2012

0207-544 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Melanoma/Pro-Drug/Valacyclovir/In Vivo/DNA-Liposome Complexes/Herpes Simplex Thymidine Kinase cDNA/Intravenous Injection

Thompson, John A., Seattle Cancer Care Alliance and The University of Washington, Seattle, Washington; Curti, Brendan; Providence Portland Medical Center; Portland, Oregon; and Cranmer, Lee; Arizona Cancer Center; Tucson, Arizona; *A Phase I Study to Evaluate the Safety and Pharmacokinetics of Pro-1, a Liposome-Encapsulated Thymidine Kinase Gene Formulation, in Patients with Stage IV Metastatic Melanoma*. Sponsor: Protiva Biotherapeutics, Inc.

NIH/OBA Receipt Date: 7-24-02. Publicly Reviewed at the September 2002 RAC meeting

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0207-545 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Peripheral Blood Lymphocytes/Plasmid DNA/Immunoglobulin Heavy (H) Chain Gene/Telomerase Reverse Transcriptase (hTERT) Gene/Intravenous Infusion

Zanetti, Maurizio, University of California, San Diego, San Diego, California; *A Phase I, Escalating Dose, Open Label Evaluation of Safety, Feasibility and Tolerability of Transgenic Lymphocyte Immunization Vaccine (TLI) in Subjects with Histologically Proven Prostate Adenocarcinoma*. Sponsor: Cosmo Bioscience, Inc.

NIH/OBA Receipt Date: 7-24-02. Publicly Reviewed at the September 2002 RAC meeting
Closed: 12-6-04

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0207-546 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Peripheral Artery Disease/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection

Powell, Richard J., Dartmouth Medical School, Lebanon, New Hampshire; Daniel, George K., The Care Group, LLC, Indianapolis, Indiana; Davies, Mark G., University of Rochester Medical Center, Rochester, New York; Comerota, Anthony J., Jobst Vascular Center, Toledo, Ohio; Henry, Timothy D., Minneapolis Heart Institute Foundation; Rockson, Stanley, Stanford University School of Medicine, Stanford, California; Young, John J., Linder Clinical Trial Center, Cincinnati, Ohio, Mendelsohn, Farrell, Cardiology, P.C., Birmingham, Alabama; Lopez, John, The University of Chicago, Chicago, Illinois; Chronos, Nicholas, American Cardiovascular Research Institute, Atlanta, Georgia; Conte, Michael, Brigham and Women's Hospital, Boston, Massachusetts; Cooper, Christopher, Medical College of Ohio, Toledo, Ohio; Faries, Peter, New York Presbyterian Hospital, Weill Medical College at Cornell University, New York, New York; Goldman, Corey, Oschner Clinic Foundation, New Orleans, Louisiana; Kwolek, Christopher, Massachusetts General Hospital, Boston, Massachusetts; Saucedo, Jorge, Oklahoma Medical Center, Oklahoma City, Oklahoma; Farber, Alik, Midway Hospital Medical Center, Los Angeles, California; Bandyk, Dennis, University of South Florida, Tampa, Florida; Moursi, Mohammed, Central Arkansas Veterans Healthcare System, Little Rock, Arkansas; Sidawy, Anton; Veteran Affairs Medical Center, Washington, DC; Martinez, Jeffrey; Peripheral Vascular Associates; San Antonio, Texas; Clair, Daniel; The Cleveland Clinic Foundation; Cleveland, Ohio; and Kafie, Fernando; Baptist Clinical Research; Pensacola, Florida; *A Phase I/II Double-Blind, Randomized, Placebo-Controlled Study to Assess the Safety and Efficacy of AMG0001 to Improve Perfusion in Critical Leg Ischemia*. Sponsor: AnGes, Inc.

NIH/OBA Receipt Date: 7-24-02. Publicly Reviewed at the September 2002 RAC meeting
Closed to enrollment; follow-up is ongoing: 01-09-06

0207-547 (Withdrawn from RAC Review) Gene Therapy/Phase I-II/Peripheral Artery Disease/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection

Powell, Richard J., Dartmouth Medical School, Lebanon, New Hampshire; *A Phase I/II Double-Blind, Randomized, Placebo-Controlled Study to Assess the Safety and Efficacy of AMG0001 to Improve Perfusion and Healing After Major Amputation Due to Critical Leg Ischemia*. Sponsor: AnGes, Inc.

NIH/OBA Receipt Date: 7-24-02. Withdrawn from RAC review: 9-6-02.

0207-548 (Closed) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous Infusion

Vieweg, Johannes and Chao, Nelson, Duke University Medical Center, Durham, North Carolina; *Active Immunotherapy with Mature, Tumor RNA-Transfected, Autologous Dendritic Cells with or without the IL2-Diphtheria Toxin Conjugate Denileukin Difitox (Ontak®) in Patients with Metastatic Renal Cell Carcinoma*.

NIH/OBA Receipt Date: 7-29-02. Not Selected for RAC Public Review: 8-16-02
Closed to enrollment, follow-up ongoing: 3-4-05

0208-549 (Closed) Gene Therapy/Phase II/Cancer/Esophagus/Immunotherapy/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection

Senzer, Neil, US Oncology, Dallas, Texas; Swisher, Stephen G., M.D. Anderson Cancer Center, Houston, Texas; Reid, Tony, University of California, San Diego Cancer Center, La Jolla California; Hanna, Nader, University of Kentucky, Lexington, Kentucky; Shabahang, Mohammad-Mohsen, Scott and White Memorial Hospital and Clinic, Temple, Texas; Mauer, Ann, University of Chicago Cancer Research Center, Chicago, Illinois; Chung, Ted, Massey Cancer Center, Virginia Commonwealth University, Richmond, Virginia; and Rosemurgy, Alexander, University of South Florida, Tampa, Florida; Chang, Kenneth J., University of California, Irvine, Orange, California; Kleinberg, Lawrence, Sidney Kimmel Cancer Center, Johns Hopkins, Baltimore, Maryland; Chak, Amitabh; Case Western University Hospitals, Cleveland, Ohio; Pinto, Harlan, VA Palo Alto Health Care System, Palo Alto, California; Visconti, John, St. Louis University, St. Louis, Missouri; and Hoffman, Phillip; University of Chicago; Chicago, Illinois; *A Phase II, Multi-Center, Single Arm Evaluation of Preoperative Chemoradiation Plus TNFerade™ Biologic (Ad_{GV}EGR.TNF.11D) Prior to Esophagectomy for Locally Advanced Esophageal Cancer*. Sponsor: GenVec.

NIH/OBA Receipt Date: 8-1-02. Not Selected for RAC Public Review: 8-21-02
Closed to enrollment: 02-04-10

0208-550 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α-(1,3) galactosyltransferase Gene/Subcutaneous Injection

Morton, Roscoe F., Iowa Methodist Medical Center, Des Moines, Iowa; *A Phase I/II Study of an Antitumor Vaccination Using α(1,3) galactosyltransferase Expressing Allogeneic Tumor Cells in Patients with Relapsed or Refractory Breast Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 8-26-02. Publicly Reviewed at the December 2002 RAC meeting

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0210-551 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/Vaccinia Virus/Tyrosinase cDNA/Intramuscular Injection

Topalian, Suzanne, National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma Using Recombinant Vaccinia and Fowlpox Viruses Encoding the Tyrosinase Antigen in Combination with Interleukin-2*.

NIH/OBA Receipt Date: 10-3-02. Not Selected for RAC Public Review: 10-24-02

0210-552 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1, 3)galactosyltransferase Gene/Subcutaneous Injection

Morris, John C., National Institutes of Health, Bethesda, Maryland; *A Phase I/II Study of an Antitumor Vaccination Using α(1, 3)galactosyltransferase Expressing Allogeneic Tumor Cells in Patients with Refractory or Recurrent Non-Small Cell Lung Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 10-9-02. Publicly Reviewed at the December 2002 RAC meeting
Closed to accrual for the phase I portion: 5-17-06

0210-553 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic B-Leukemia/Immunotherapy/In Vitro/Plasmid DNA/Interleukin-2/CD40 Ligand/Subcutaneous Injections

Brenner, Malcolm, Baylor College of Medicine, Houston, Texas; and Kamble, Rammurti; Center for Cell and Gene Therapy, Baylor College of Medicine; Houston, Texas; *Treatment of Chronic Lymphocytic B-Leukemia (B-CLL) with Human IL-2 and Human CD40 Ligand Plasmid Gene Modified Autologous Tumor Cells*.

NIH/OBA Receipt Date: 10-9-02. Not Selected for RAC Public Review: 10-30-02

0210-554 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Infectious Diseases/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag-Pol-Nef-Env cDNA/Interleukine-2 (IL-2)/Ig Fusion Protein/Bioinjector 2000® Injections

Dolin, Raphael, Harvard Medical School, Boston Massachusetts; Blattner, William, University of Maryland, Baltimore, Maryland; and Hammer, Scott Columbia University/New York Blood Center, New York, New York; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of the HIV-1 DNA Vaccine VRC-HIVDNA009-00-VP (Gag-Pol-Nef-Multiclade Env) with the Plasmid Cytokine Adjuvant VRC-ADJDNA004-IL2-VP (IL-2/Ig)*.

NIH/OBA Receipt Date: 10-9-02. Not Selected for RAC Public Review: 10-30-02

0210-555 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid/Prostate Specific Antigen (PSA)/Intramuscular Injection

Malkowicz, S. Bruce, University of Pennsylvania Health System, Philadelphia, Pennsylvania; *A Phase I Study of a Polynucleotide Anti-Tumor Immunization to Human Prostate Specific Antigen (PSA) in Patients with Hormone-Refractory Prostate Cancer (HRPC)*. Sponsor: Centocor Inc.

NIH/OBA Receipt Date: 10-9-02. Not Selected for RAC Public Review: 10-30-02

0210-556 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Plasmid DNA/Adenovirus/Serotype 5/L523S cDNA/Intramuscular Injection

Nemunaitis, John J., US Oncology, Dallas, Texas; Anthony, Stephen P., Cancer Care Northwest, Spokane, Washington; Richards, Donald, Tyler Cancer Center, Tyler, Texas; Berman, Barry S., Cancer Centers of Florida, Ocoee, Florida; and West, Howard, Swedish Cancer Institute, Seattle, Washington; *Phase I Open-Label, Dose Escalation Trial Evaluating the Safety and Immunogenicity of Sequential Administration of Recombinant DNA and Adenovirus Expressing L523S Protein in Patients with Early Stage Non-Small Cell Lung Cancer*. Sponsor: Corixa Corporation

NIH/OBA Receipt Date: 10-9-02. Publicly Reviewed at the December 2002 RAC meeting
Closed to accrual: 9-30-05

0210-557 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Coronary Artery Disease/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramyocardial Injection

Thompson, Craig; Dartmouth Medical School; Lebanon, New Hampshire; Wang, Andrew; Duke University School of Medicine; Durham, North Carolina; Mendelsohn, Farrell O.; Baptist Health System; Birmingham, Alabama; and Wang, Andrew; Duke University Medical Center; Durham, North Carolina; *An Open Label Dose Escalation Study to Assess the Safety of AMG0001 Administered via Intramyocardial Injection Catheter in Patients with Ischemic Heart Disease (IHD) not Amenable to Coronary Artery Bypass Graft (CABG) or Percutaneous Coronary Intervention (PCI)*. Sponsor: AnGes, Inc.

NIH/OBA Receipt Date: 10-9-02. Publicly Reviewed at the December 2002 RAC meeting

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0212-558 (Open) Gene Therapy/Phase I-II/Cancer/Breast/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Serotype 5/p53 cDNA/Subcutaneous Injection

Reed, Elizabeth C., University of Nebraska Medical Center, Omaha, Nebraska; *Adenovirus p53 Infected DC Vaccine for Breast Cancer*.

NIH/OBA Receipt Date: 12-9-02. Not Selected for RAC Public Review: 1-13-03

0212-559 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection

Kaufman, Howard L., Columbia University, New York, New York; and Safran, Howard, The Miriam Hospital, Providence, Rhode Island; *An Open Label Phase I Study to Evaluate the Safety and Tolerability of rV-CEA(6D)/TRICOM™ Admixed with rV-MUC-1 Followed by rF-CE(6D)/TRICOM™ in Combination with GM-CSF in Subjects with Unresectable Adenocarcinoma of the Pancreas*. Sponsor: Therion Biologics Corporation.

NIH/OBA Receipt Date: 12-10-02. Not Selected for RAC Public Review: 1-06-03

0212-560 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-/GM-CSF/Intramuscular Or Intradermal Injection

Gulley, James L., National Institutes of Health, Bethesda, Maryland; *A Phase I/II Pilot Study of Sequential Vaccinations with rFOWLPOX-PSA (L155)-TRICOM (PROSTAVAC-F/TRICOM) Alone, or in Combination with rVACCINIA-PSA (L155)-TRICOM (PROSTAVAC-V/TRICOM) and the Role of GM-CSF, in Men with Prostate Cancer*. Sponsor: Therion Biologics Corporation.

NIH/OBA Receipt Date: 12-10-02. Not Selected for RAC Public Review: 1-06-03
Closed to accrual: 3-22-06

0212-561 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Shuman, Marc, University of California, San Francisco Cancer Center, San Francisco, California; *Pancreatic GVAX® for Resected Adenocarcinoma of the Pancreas*.

NIH/OBA Receipt Date: 09-04-02. Not Selected for RAC Public Review: 1-03-03

0212-562 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vitro/Allogeneic K562 Cell/Combination With Untransduced Tumor Cells/Plasmid DNA/Electroporation/DMRIE-Cholesterol/Granulocyte-macrophage Colony Stimulating Factor cDNA/CD40 Ligand cDNA/Intradermal Injection

Dessureault, Sophie, University of South Florida, Tampa, Florida*; A Phase I Trial Using a Universal GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) in the Formulation of Autologous Tumor Cell-Based Vaccines for Cancer Patients with Stage IV Disease.*

NIH/OBA Receipt Date: 12-18-02. Not Selected for RAC Public Review: 1-10-03

0212-563 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/GD-2 Specific scFvFc-Zeta T Cell Receptor/Intravenous Injections

Meyers, G. Doug, and Brenner, Malcolm, Baylor College of Medicine, Houston, Texas; *Administration of Peripheral Blood T-Cells and EBV Specific CTLs Transduced to Express GD-2 Specific Chimeric T Cell Receptors to Patients with Neuroblastoma*.

NIH/OBA Receipt Date: 12-24-02. Publicly Reviewed at the March 2003 RAC meeting

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0301-564 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer Adenocarcinoma Expressing Carcinoembryonic Antigen (CEA)/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/Anti-CEA-sFv-Zeta T Cell Receptor-CD28/Intravenous Infusion

Junghans, Richard and Katz, Steven C.; Roger Williams Hospital; Providence, Rhode Island; *Phase Ia/Ib Trial of 2nd Generation Designer T Cells in Adenocarcinoma*.

NIH/OBA Receipt Date: 1-06-03. Publicly Reviewed at the March 2003 RAC meeting

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0301-565 (Closed) Gene Therapy/Phase I/Anaplastic Thyroid Cancer/Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5 p53 cDNA/Intratumoral Injections

Reid, William K., Vanderbilt-Ingram Oncology, Vanderbilt University Medical Center, Franklin, Tennessee; *Study to Evaluate the Overall Response and Safety of Biweekly Intratumoral Administration of RPR/INGN 201 in Anaplastic Thyroid Cancer*.

NIH/OBA Receipt Date: 1-06-03. Not Selected for RAC Public Review

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0301-566 (Open) Gene Therapy/Phase I/Cancer/Hematologic Malignancy Following Allogeneic Bone Marrow Transplantation/Pro-drug/Elimination of Graft Versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/CD34-Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous

DiPersio, John, Washington University School of Medicine, St. Louis, Missouri; *Infusion of Genetically Modified T Cells: Tracking and Toxicity*.

NIH/OBA Receipt Date: 1-07-03. Not Selected for RAC Public Review: 5-13-03

0301-567 (Closed) Gene Therapy/Phase II/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Percutaneous Cardiac Catheterization/Intra-myocardial Injection

Losordo, Douglas W., St. Elizabeth's Medical Center, Tufts University School of Medicine, Boston Massachusetts; Fortuin, F. David, Mayo Clinic Hospital, Scottsdale, Arizona; Schatz, Richard A., Scripps Clinic, LaJolla, California; Henry, Timothy, Abbott Northwestern Hospital, Minneapolis, Minnesota; Brown, Charles, Piedmont Hospital, Atlanta, Georgia; Schaer, Gary L., Rush University Medical Center, Chicago, Illinois; Mendelsohn, Farrell O., Cardiology P.C., Birmingham, Alabama; Reisman, Mark, Swedish Medical Center, Seattle, Washington; Simari, Robert D., Mayo Clinic, Rochester, Minnesota; Perin, Emerson C., St. Luke's Episcopal Hospital, Houston, Texas; Conrad, James A., Lee Memorial Health System, Fort Myers, Florida; Murphy, Allan , Riverside Regional Medical Center, Newport News, Virginia; Waksman, Ron, Washington Hospital Center, Washington, DC; Foster, Malcolm T., Baptist Heart Institute, Knoxville, Tennessee; Dib, Nabil; Arizona Heart Institute; Phoenix, Arizona; Moses, Jeffrey W., Columbia University Medical Center; New York, New York; McKeever, Louis Stephen; Midwest Heart Foundation; Lombard, Illinois; Sanborn, Timothy; Evanston Northwestern Healthcare; Evanston, Illinois; Liberman, Henry L.; Crawford Long Hospital; Atlanta, Georgia; Annex, Brian; Durham VA Medical Center; Durham, North Carolina; Hermiller, James B.; The Care Group, LLC, The Heart Center of Indiana; Indianapolis, Indiana; Chronos, Nicholas; Saint Joseph's Research Institute; Atlanta, Georgia; Anwar, Azam; HeartPlace; Dallas, Texas; Fischell, Tim; Borgess Medical Center; Kalamazoo, Michigan; Bhoopalam, Vishwajeth; Nebraska Heart Institute; Lincoln, Nebraska; Kipperman, Robert M.; Oklahoma Cardiovascular Associates; Oklahoma City, Oklahoma; Wong, S. Chiu; New York Presbyterian Hospital-Weill Medical College of Cornell University; New York, New York; Bajwa, Tanvir; St. Luke's Medical Center; Milwaukee, Wisconsin; Mishkel, Gregory; Prairie Cardiovascular Consultants, Ltd.; Springfield, Illinois; Watkins, Matthew; University of Vermont College of Medicine; Burlington, Vermont; Costa, Marco; University of Florida Health Sciences Center; Jacksonville, Florida; Ling, Frederick S.; Rochester Medical Center; Rochester, New York; Dippel, Eric; Cardiovascular Medicine, P.C.; Davenport, Iowa; Molk, Barry L.; Aurora Denver Cardiology Associates, P.C.; Aurora, Colorado; Meilman, Henry; MidAtlantic Cardiology; Baltimore, Maryland; and Lieberman, Scott M.; Cardiovascular Associates of East Texas; Tyler, Texas; *A Multicenter, Randomized, Double-Blind, Dose Ranging Placebo-Controlled Study Evaluating Defined Doses of Percutaneously Delivered Via Boston Scientific Stiletto™ Endocardial Direct Injection Catheter System pVGI.1 (VEGF2) (Placebo, 20, 200, or 800 μg) in Patients with Class III or IV Angina*. Sponsor: Corautus Genetics, Inc. (formerly Vascular Genetics, Inc.)

NIH/OBA Receipt Date: 1-08-03. Not Selected for RAC Public Review: 1-29-03
Closed to enrollment: 5/23/06

0301-568 (Closed) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/DNA-Liposome Complexes/Poloxamer 188/Del-1 cDNA/Intramuscular Injection

Rajagopalan, Sanjay, University of Michigan, Ann Arbor, Michigan; Rocha-Singh, Krishna, Prairie Education and Research Cooperative, Springfield, Illinois; Kleiman, Neal S., Baylor College of Medicine, The Methodist Hospital, Houston, Texas; Litt, Marc, Jacksonville Heart Center, Jacksonville, Florida; Hermiller, James B., The Care Group, LLC, Indianapolis, Indiana; Henry, Tim, Abbott Northwestern Hospital/Minneapolis Heart Institute Foundation, Minneapolis, Minnesota; Snell, Jeffrey, Rush-Presbyterian St. Luke's Medical Center, Chicago, Illinois; Grossman, P. Michael, University of Michigan Health Systems, Ann Arbor, Michigan; Mendelsohn, Farrell O., Cardiology P.C., Birmingham, Alabama; Kandzari, David, Duke University Medical Center, Durham, North Carolina; Weiss, Robert, Andoscoggin Cardiology Associates, Auburn, Maine; Ramaiah, Venkatesh, Arizona Heart Institute, Phoenix, Arizona; Powell, Richard, Dartmouth-Hitchcock Medical Center, Lebanon, New Hampshire; Chronos, Nicholas, American Cardiovascular Research Institute, Atlanta, Georgia; Gottlieb, Daniel, Burien, Washington; Piana, Robert, Vanderbilt University Medical Center, Nashville, Tennessee; Anderson, R. David, Sarasota Memorial Health Care System, Sarasota, Florida; Saucedo, Jorge, University of Oklahoma Health Sciences Center, Oklahoma City, Oklahoma; and Karlsberg, Ronald P., Access Clinical Trials/Cardiovascular Research Institute, Beverly Hills, California; *A Phase II Multi-Center, Double-Blind, Placebo-Controlled, Trial of VLTS-589 in Subjects with Intermittent Claudication Secondary to Peripheral Arterial Disease*. Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 1-08-03. Not Selected for RAC Public Review: 1-29-03
Closed: 6-9-06

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0301-569 (Closed) Gene Therapy/Phase II/Monogenic Disease/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) cDNA/Aerosol Administration

Moss, Richard, Stanford University School of Medicine, Palo Alto, California; Spencer, L. Terry, University of Florida, Gainesville, Florida; Clancy, John P., University of Alabama at Birmingham, Birmingham, Alabama; Zeitlin, Pamela L., Johns Hopkins University, Baltimore, Maryland; Milla, Carlos E., University of Minnesota, Minneapolis, Minnesota; Colombo, John L., University of Nebraska Medical Center, Omaha, Nebraska; Accurso, Frank, The Children's Hospital Denver, Denver, Colorado; Dorkin, Henry L., Massachusetts General Hospital, Boston, Massachusetts; Waltz, David A., Children's Hospital Boston, Boston, Massachusetts; Pilewski, Joseph M., University of Pittsburgh, Pittsburgh, Pennsylvania; Pian, Mark; Children's Hospital and Health Center, San Diego, California; and Ferkol, Thomas; Washington University School of Medicine, St. Louis, Missouri; *A Multicenter, Double-Blind, Placebo-Controlled, Phase II Study of Aerosolized tgAAVCF for the Treatment of Cystic Fibrosis*. Sponsor: Targeted Genetics Corporation.

NIH/OBA Receipt Date: 1-08-03. Not Selected for RAC Public Review: 1-29-03
Closed: 3-17-05

0301-570 (Open; RAC Reviewed with Recommendations) Non-therapeutic (Healthy Volunteers)/Cholera Vaccine/In Vivo/*Vibrio cholerae*/Oral Administration

Tacket, Carol O., Center for Vaccine Development, University of Maryland, Baltimore, Maryland*; Use of in vivo Expression Technology to Identify Virulence Factors and Protective Antigens of Vibrio cholerae 01*.

NIH/OBA Receipt Date: 1-08-03. Publicly Reviewed at the March 2003 RAC meeting

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0302-571 (Closed) Gene Therapy/Phase II/Immunotherapy/Cancer/Non-Small Cell Lung Carcinoma (NSCLC)/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Ctokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intradermal Injections

Ross, Helen J., Providence Portland Medical Center, Robert W. Franz Cancer Research Center, Earle A. Chiles Research Institute, Portland, Oregon*;* Anthony, Stephen P., Cancer Care Northwest, Spokane, Washington; Kelly, Karen, University of Colorado, Denver, Colorado; Sterman, Daniel, University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; Harper, Harry, Hackensack University Medical Center, Hackensack, New Jersey; Carbone, David, Vanderbilt University Medical Center, Nashville, Tennessee; Figlin, Robert, University of California, Los Angeles, School of Medicine, Los Angeles, California; Jablons, David, University of California, San Francisco Comprehensive Cancer Center, San Francisco, California; and Schiller, Joan H., University of Wisconsin Comprehensive Cancer Center, Madison, Wisconsin; *A Phase II Randomized Study of GM-CSF Gene-Modified Autologous Tumor Vaccine (CG8123) With and Without Low-Dose Cyclophosphamide in Advanced Stage Non-Small Cell Lung Cancer.* Sponsor: Cell Genesys.

NIH/OBA Receipt Date: 2-11-03. Not Selected for RAC Public Review: 3-04-03
Closed: 01-13-06

0302-572 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Adenovirus Type 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intratumoral Injection

Corman, John, Virginia Mason Medical Center, Seattle, Washington; Nemunaitis, John J., Mary Crowley Medical Research Center, Dallas, Texas; Nieva, Jorge; Scripps Cancer Center, San Diego, California; and DeWeese, Theodore; Johns Hopkins University, Baltimore, Maryland; *A Phase I Trial of Intraprostatic Injection of CG7870 Followed by Three-Dimensional Conformal Radiation Therapy (3D-CRT) in Patients with Clinically-Localized Intermediate-Risk Prostate Cancer*. Sponsor: Cell Genesys.

NIH/OBA Receipt Date: 2-24-03. Not Selected for RAC Public Review: 3-14-03
Closed: 3-3-05

0303-573 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Human and Mouse gp100 cDNAs/Intramuscular Injection

Wolchok, Jedd D., Memorial Sloan Kettering Cancer Center, New York, New York; *Injection of AJCC Stage IIB, IIC, III and IV Melanoma Patients with Human and Mouse gp100 DNA: A Phase I Trial to Assess Safety and Immune Response*.

NIH/OBA Receipt Date: 3-24-03. Not Selected for RAC Public Review: 4-11-03

0303-574 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer (NSCLC)/Immunotherapy/In Vitro/AD#100, an Allogeneic Human Lung Adenocarcinoma Cell Line/Plasmid DNA/Human Heat Shock Protein gp96-Ig cDNA/Intradermal Injections

Raez, Luis E., University of Miami School of Medicine, Miami, Florida; *Novel Tumor Vaccine gp96-Ig Fusion Protein in Advanced (Stage III), Relapsed or Metastatic (Stage IV) Non-Small Cell Lung Cancer (NSCLC) Patients Who Have Failed First Line Chemotherapy*.

NIH/OBA Receipt Date: 3-24-03. Not Selected for RAC Public Review: 4-11-03

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0304-575 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Disorders/Retinitis Pigmentosa/In Vitro/Plasmid/Encapsulated Cell-Based Drug Delivery Device/Human Ciliary Neurotrophic Factor (CNTF) cDNA/Intraocular Implantation (Via Sclerotomy)

Sieving, Paul A., National Institutes of Health, Bethesda, Maryland; *A Phase I Study of NT-501, an Implant of Encapsulated Human NTC-201 Cells Releasing Ciliary Neurotrophic Factor (CNTF), in Patients with Retinitis Pigmentosa*. Sponsor: Neurotech USA.

NIH/OBA Receipt Date: 4-10-03. Publicly Reviewed at the June 2003 RAC meeting

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0304-576 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/In Vivo/Plasmid DNA/Epidermal Growth Factor Receptor Antisense/Intratumoral Injection

Grandis, Jennifer Rubin, University of Pittsburgh School of Medicine, Pittsburgh, Pennsylvania; *Phase I Trial of Intratumoral EGFR Antisense DNA in Advanced Head and Neck Squamous Cell Carcinoma*.

NIH/OBA Receipt Date: 4-11-03. Not Selected for RAC Public Review: 5-01-03

0304-577 (Open) Gene Therapy/Cancer/Renal Cell Cancer/Immunotherapy/Vaccinia Virus/5T4 cDNA/Intramuscular Injection

Kaufman, Howard, Columbia University College of Physicians and Surgeons, New York, New York; *A Preliminary Study of the Safety, Immunogenicity and Clinical Efficacy of TroVax Given in Conjunction with Interleukin 2 in the Treatment of Stage IV Renal Cell Cancer*. Sponsor: Oxford BioMedica, plc.

NIH/OBA Receipt Date: 4-23-03. Not Selected for RAC Public Review: 5-12-03

0304-578 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Emens, Leisha A., The Sidney Kimmel Comprehensive Cancer Center, Johns Hopkins University, Baltimore, Maryland; *A Phase I Vaccine Safety and Chemotherapy Dose-Finding Trial of GM-CSF-Secreting Breast Cancer Vaccine Given in a Specifically Timed Sequence with Immunomodulatory Doses of Cyclophosphamide and Doxorubicin*.

NIH/OBA Receipt Date: 4-22-03. Not Selected for RAC Public Review: 5-12-03

0304-579 (Open) Gene Therapy/Phase I/Infectious Disease/Cytomegalovirus (CMV) Disease/In Vitro/EBV and CMV-Specific Cytotoxic T Lymphocytes/Adenovirus/CMV pp65 Gene/Intravenous

Bollard, Catherine; Texas Children's Hospital and Center for Cell and Gene Therapy and Baylor College of Medicine; Houston, Texas; *Virus Specific Cytotoxic T-Lymphocytes for the Prophylaxis of CMV after Allogeneic Stem Cell Transplant: A Dose Finding Trial*.

NIH/OBA Receipt Date: 4-22-03. Not Selected for RAC Public Review: 5-12-03

0304-580 (Open) Gene Therapy/Phase II/Cancer/Melanoma/In Vivo/Tumor Suppressor/Adenovirus/Melanoma Differentiation Associated Protein-7 cDNA/Intratumoral Injection

Kim, Kevin B., The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Phase II Study Examining the Biological Efficacy of Intratumoral INGN 241 (Ad-mda7) Administration in Patients with In Transit Melanoma*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 4-22-03. Not Selected for RAC Public Review: 5-12-03

0304-581 (Open) Gene Therapy/Phase I/Cancer/Bladder/Immunotherapy/In Vivo/Fowlpox Virus/B7.1 (CD80)/ICAM-1/LFA-3/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intravesical Administration

Weiss, Robert; The Cancer Institute of New Jersey, UMDNJ-Robert Wood Johnson Medical School; New Brunswick, New Jersey; *Phase I Study of Intravesical Recombinant Fowlpox-GM-CSF (rF-GM-CSF) and/or Recombinant Fowlpox-TRICOM (rF-TRICOM) in Patients with Bladder Carcinoma Scheduled for Cystectomy*. Sponsor: Therion Biologics Corporation

NIH/OBA Receipt Date: 4-23-03. Not Selected for RAC Public Review: 5-13-03

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0305-582 (Open) Gene Therapy/Phase I-II/Cancer/Ovarian/Oncogene-Regulation/In Vivo/Cationic Liposome Complex/DC-Chol-DOPE/E1A/Intrperitoneal Administration

Ueno, Naoto; The University of Texas M.D. Anderson Cancer Center; Houston, Texas; *A Phase 1/2 Randomized Study of Intraperitoneal tgDCC-E1A and Intravenous Paclitaxel in Women with Platinum-Resistant Ovarian Cancer.*

NIH/OBA Receipt Date: 5-30-03. Not Selected for RAC Public Review: 6-20-03

0306-583 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo Electroporation/Plasmid DNA/Interleukin-12 cDNA/Intratumoral In Vivo Electroporation

Daud, Adil, H. Lee Moffitt Cancer Center, Tampa, Florida; *Phase I Trial of Intratumoral pIL-12 Electroporation in Malignant Melanoma.*

NIH/OBA Receipt Date: 6-19-03. Not Selected for RAC Public Review: 7-10-03

0307-584 (Open) Gene Therapy/Phase II/Immunotherapy/Cancer/Melanoma/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype5/Cyotokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous and Intradermal Injection

Hodi, F. Stephen, Dana-Farber Cancer Institute, Boston, Massachusetts; *A Phase II Trial of Vaccination with Autologous Lethally Irradiated Melanoma Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Granulocyte-Macrophage Colony Stimulating Factor in Stage III and IV Metastatic Melanoma Patients.*

NIH/OBA Receipt Date: 7-16-03. Not Selected for RAC Public Review: 8-05-03

0307-585 (Open) Gene Therapy/Phase I/Cancer/CEA Expressing Carcinomas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection

Carson, William E., III, The Ohio State University, Columbus, Ohio; *A Phase I Study of Sequential Vaccinations with Fowlpox-CEA(6D)-TRICOM (B7.1/ICAM/LFA-3) and Vaccinia-CEA(6D)-TRICOM, in Combination with GM-CSF and Interferon-Alfa-2B in Patients with CEA Expressing Carcinomas.*

NIH/OBA Receipt Date: 7-22-03. Not Selected for RAC Public Review: 8-11-03

0307-586 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection

Kaufman, Howard L., Columbia University, New York, New York; and Marshall, John, Georgetown University Medical Center, Washington, D.C.; *An Open Label Phase I Study to Evaluate the Safety and Tolerability of PANVAC^{TM}-VF in Combination with GM-CSF in Patients with Unresectable Adenocarcinoma of the Pancreas.*

NIH/OBA Receipt Date: 7-21-03. Not Selected for RAC Public Review: 8-08-03

0307-587 (Open) Gene Therapy/Phase II/Cancer/Colorectal/In Vivo/Adenovirus/Type 5/Tumor Necrosis Factor cDNA/Intratumoral Injection

Libutti, Steven K.; and Rosenberg, Steven A.; National Institutes of Health, Bethesda, Maryland; *A Phase II Randomized Trial Concerning TNFerade Biologic with Capecitabine and Radiation Therapy Followed by Surgical Resection Versus Capecitabine and Radiation Therapy Followed by Surgical Resection for the Treatment of Rectal Cancer.*

NIH/OBA Receipt Date: 7-21-03. Not Selected for RAC Public Review: 8-08-03

0307-588 (Closed) Gene Therapy/Phase I/Other Diseases-Disorders/Rheumatoid Arthritis/In Vivo/Adeno-Associated Virus/Serotype 2/Tumor Necrosis Factor Receptor-Fc Immunoglobulin (TNFR:Fc) Fusion Gene cDNA/Intra-Articular Administration

Mease, Philip J., Swedish Hospital Rheumatology Clinical Research Division, Seattle, Washington; Hobbs, Kathryn, Denver Arthritis Clinic, Denver, Colorado; Bingham, Clifton; New York University School of Medicine, New York, New York; and Furst, Daniel E., University of California, Los Angeles, Los Angeles, Califonia; *A Phase I Dose Escalation Study of Intra-Articular Administration of tgAAC94, a Recombinant Adeno-Associated Vector Containing the TNFR:Fc Fusion Gene, in Inflammatory Arthritis.* Sponsor: Targeted Genetics.

NIH/OBA Receipt Date: 7-26-03. Publicly Reviewed at the September 2003 RAC meeting
Long-term follow-up is ongoing: 08-23-07

0307-589 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Glaucoma/In Vivo/Adenovirus/Serotype 5/p21$^{WAF-1/Cip1}$ cDNA/Subconjunctival Injection

Kaufman, Paul L., University of Wisconsin-Madison Medical School, Madison, Wisconsin; and Weinreb, Robert N., University of California, San Diego, La Jolla, California; *A Phase 1 Study in Glaucoma Subjects Receiving SCH 412499 (rAd-p21) Administered as a Single Injection into the Subconjunctival Space Prior to Primary Trabeculectomy.* Sponsor: Schering Corporation.

NIH/OBA Receipt Date: 7-23-03. Publicly Reviewed at the December 2003 RAC meeting

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0307-590 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/Yeast Cytosine Deaminase cDNA/5-Flurocytosine/Herpes Simplex Thymidine Kinase cDNA/Valganciclovir/Adenovirus Death Protein/Intratumoral Injection

Kim, Jae Ho, and Freytag, Svend, Henry Ford Health System, Detroit, Michigan; *Phase I/II Study of Replication-Competent Adenovirus-Mediated Double Suicide Gene Therapy in Combination with Conventional Dose Conformal Radiation Therapy for the Treatment of Intermediate to High-Risk Prostate Cancer*

NIH/OBA Receipt Date: 7-23-03. Not Selected for RAC Public Review: 8-12-03

0307-591 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Tumor Suppressor Gene/In Vivo/Cationic Liposome Complex/DOTAP-DOPE/p53 cDNA/Intravenous Infusion

Marshall, John, Lombardi Cancer Center, Georgetown University Medical Center, Washington, DC; *An Open-Label Safety Study of Escalating Doses of SGT-53 for Systemic Injection in Patients with Advanced Solid Tumor Malignancies.*

NIH/OBA Receipt Date: 7-23-03. Not Selected for RAC Public Review: 8-12-03

0307-592 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Cervical Cancer/Immunotherapy/In Vivo/Listeria monocytogenes/Human Papilloma Virus E7 Gene/Intravenous Injection

Marshall, John, Lombardi Cancer Center, Georgetown University Medical Center, Washington, DC; *A Phase 1 Study to Determine the Safety and Immunogenicity of Vaccination with Listeria monocytogenes Expressing Human Papilloma Virus Type 16 E7 for the Treatment of Progressive, Recurrent and Advanced Squamous Cell Cancer of the Cervix.* Sponsor: Advaxis, Inc.

NIH/OBA Receipt Date: 7-23-03. Publicly Reviewed at the December 2003 RAC meeting

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0307-593 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Parkinson's Disease/In Vivo/Adeno-Associated Virus/Aromatic L-amino Acid Decarboxylase cDNA/Intrastriatal Administration

Aminoff, Michael, University of California, San Francisco, San Francisco, California; *A Phase I Open-Label Safety Study of Intrastriatal Infusion of Adeno-Associated Virus Encoding Human Aromatic L-amino Acid Decarboxylase (AAV-hAAADC-2) in Subjects with Advanced Parkinson's Disease.* Sponsor: Avigen.

NIH/OBA Receipt Date: 7-23-03. Publicly Reviewed at the October 17, 2003 RAC meeting
Closed to enrollment: 2-17-06

0307-594 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Degenerative Arthritis/In Vitro/Allogeneic Human Chrondrocytes/Retrovirus/Transforming Growth Factor-β1 (TGF-β1)/Intra-Articular Administration

Mont, Michael A., Sinai Hospital of Baltimore, Baltimore, Maryland; *A Phase 1 Study to Determine the Safety and Biological Activity of Cell-Mediated Gene Therapy Using TissueGene-C in Patients with Degenerative Joint Disease of the Knee Prior to Total Knee Arthroplasty.* Sponsor: TissueGene, Inc.

NIH/OBA Receipt Date: 7-23-03. Publicly Reviewed at the September 2003 RAC meeting

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0307-595 (Open) Gene Therapy/Phase I-II/Cancer/Cervical/Immunotherapy/In Vivo/Plasmid DNA/HPV 16 E7 cDNA/Intramuscular Injection

Trimble, Cornelia, Johns Hopkins University School of Medicine, Baltimore, Maryland; *A Phase I/II Clinical Trial of pNGVL4a-Sig/E7 (detox)/HSP70 for the Treatment of Patients with HPV16+ Cervical Intraepithelial Neoplasia 2/3 (CIN2/3).*

NIH/OBA Receipt Date: 7-24-03. Not Selected for RAC Public Review: 8-12-03

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0307-596 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/RNAs Transcribed In Vitro from Plasmids Containing cDNAs for MART-1, Tyrosinase, gp100, and MAGE-3/Intra-Nodal Injections

Liao, Xinsheng M., University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Phase I Open Labeled Non-Randomized Study of RNA-DC as Tumor Vaccine in Patients with Melanoma*.

NIH/OBA Receipt Date: 7-25-03. Not Selected for RAC Public Review: 8-12-03

0307-597 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/Yeast Cytosine Deaminase cDNA/5-Flurocytosine/Herpes Simplex Thymidine Kinase cDNA/Valganciclovir/Adenovirus Death Protein/Intratumoral Injection

Kim, Jae Ho, and Freytag, Svend, Henry Ford Health System, Detroit, Michigan; *Phase I/II Study of Replication-Competent Adenovirus-Mediated Double Suicide Gene Therapy in Combination with Salvage Intensity Modulated Radiation Therapy for the Treatment of Locally Recurrent Prostate Cancer.*

NIH/OBA Receipt Date: 7-23-03. Not Selected for RAC Public Review: 8-12-03

0307-598 (Open) Gene Therapy/Phase I/Infectious Disease/Adenovirus Disease/In Vitro/Adenovirus-Specific Cytotoxic T Lymphocytes/Adenovirus/No Transgene/Intravenous

Bollard, Catherine M., and Keuhnle, Ingrid, Baylor College of Medicine, Houston, Texas; *Administration of Virus-Specific Cytotoxic T-Lymphocytes for the Prophylaxis and Therapy of Adenovirus Infection Post Allogeneic Stem Cell Transplant.*

NIH/OBA Receipt Date: 7-30-03. Not Selected for RAC Public Review: 8-19-03

0308-599 (Closed) Gene Therapy/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta Chain cDNAs/Intravenous Infusion

Rosenberg, Steven A., National Institutes of Health, Bethesda, Maryland; *Treatment of Patients with Metastatic Melanoma by Lymphodepleting Conditioning Followed by Infusion of TCR-Gene Engineered Lymphocytes and Subsequent Peptide Immunization.*

NIH/OBA Receipt Date: 8-05-03. Not Selected for RAC Public Review: 8-25-03
Closed to accrual, data analysis is ongoing: 9-7-07

0308-600 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection

Kantoff, Philip, Dana-Farber Cancer Institute, Boston, Massachusetts; Hayward, Malcolm, Highlands Oncology Group, Springdale, Arkansas; Patterson, Thomas, Medical & Surgical Specialists, L.L.C., Galesburg, Illinois; Lugg, James, Wyoming Research Foundation, Inc., Cheyenne, Wyoming; Clark, Randil L., North Idaho Urology, Coeur d´Alene, Idaho; Dineen, Martin K., Atlantic Urological Associates, Daytona Beach, Florida; Karlin, Gary S., Lawrenceville Urology, Lawrenceville, New Jersey; Harper, William M., IV Urology Center of Columbus, Columbus, Georgia; McGee, James L., OSF Saint Francis Medical, Peoria, Illinois; Gittleman, Marc, South Florida Medical Research, Aventura, Florida; Bilhzrtz, David, Urology Associates, Nashville, Tennessee; Godschalk, Michael, VA Medical Center, Richmond, Virginia; Pittman, Walter, Urology Centers of Alabama, P.C., Homewood, Alabama; Zinner, Norman, Western Clinical Research, Inc., Torrance, California; Gange, Steven, Salt Lake Research, Salt Lake City, Utah; Kadesky, Keith, Urology Clinics of North Texas, PA and Urology Specialists & Associates, PA, Dallas, Texas; Plante, Mark K., The University of Vermont, Burlington, Vermont; Tannenbaum, Sigmund I., The Urology Center, Greensboro, North Carolina; Roper, Ronald P., Urology Associates, Marietta, Georgia; Brosman, Stanley A., Pacific Clinical Research, Marina Del Rey, California; Schiff, William, Urology Associates of Central California, Fresno, California; Hamway, Sammy, South Dayton Urological Associates, Kettering, Ohio; Hauke, Ralph, University of Nebraska Medical Center, Omaha, Nebraska; Mega, Anthony, The Miriam Hospital, Providence, Rhode Island; Leventhal, Sheryl L., Nyack Hospital, Nyack, New York; Bubley, Glenn, Beth Israel Deaconess Medical Center, Boston, Massachusetts; Glode, L. Michael, The University of Colorado Health Sciences Center, Denver, Colorado; Roland, Kendrith M., Jr., Carle Clinic Association, Urbana, Illinois; Mobley, William C., Urology Associates, P.C., Davenport, Iowa; Lilly, Michael B., Loma Linda University, Loma Linda, California; Efros, Mitchell, AccuMed Research Associates, Garden City, New York; Amin, Asim**,** Lombardi Cancer Research Center, Georgetown University, Washington, DC; Sharkey, Jerrold, Advanced Research Institute, Inc., New Port Richey, Florida; Mason, Terry, Prairie Medical Associates, Ltd., Chicago, Illinois; Tomera, Kevin M., Alaska Clinical Research Center, Anchorage, Alaska; Kabbinavar, Fairooz, University of California, Los Angeles, Los Angeles, California; Wachs, Barton H., Atlantic Urological Medical Group, Long Beach, California; Snoy, Frederick, Urology Group of New Mexico, Albuquerque, New Mexico; Fisher, Hugh A.G., The Urological Institute of Northeastern New York, Albany, New York; Bailen, James L., Metropolitan Urology, PSC, Jeffersonville, Indiana; McMurtry, James M., Virginia Urology, Richmond, Virginia; Niku, Soheil D., Simi-San Fernando Valley Urology Associates, Granada Hills, California; Feldman, Robert A., Connecticut Clinical Research Center, Waterbury, Connecticut; Sandock, David S., Midwest Research Specialists, LLC, Milwaukee, Wisconsin; Macaluso, Joseph N., Jr., Urologic Institute of New Orleans, Gretna, Louisiana; See, William A., The Medical College of Wisconsin, Milwaukee, Wisconsin; Bidair, Mohamed, Center for Urological Research, La Mesa, California; Kaufman, Howard; Columbia University College of Physicians and Surgeons, New York, New York; DiPaola, Robert; The Cancer Institute of New Jersey, New Brunswick, New Jersey; Lewis, Nancy; Fox Chase Cancer Center, Philadelphia, Pennsylvania; Perry, David J.; Washington Cancer Institute/Washington Hospital Center, Washington, DC; Waterfield, William; Franklin Square Hospital Center, Baltimore, Maryland; Chatta, Gurkamal S.; University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania; Nemunaitis, John; Mary Crowley Medical Research Center, Dallas, Texas; DiStefano, Alfred; Arlington Cancer Center, Arlington, Texas; Holladay, Charles S., Charleston Cancer Center, Charleston, South Carolina; Bennett, James, Midtown Urology, Atlanta, Georgia; Page, Ray D., Texas Cancer Care, Fort Worth, Texas; Lee, Christopher; New York University; New York, New York; and Ferrari, Anna; New York University Medical Center; New York, New York; *A Phase II Randomized, Double Blind, Controlled Study to Evaluate the Safety and Efficacy of PROSTVAC®-VF/TRICOM™ in Combination with GM-CSF in Patients with Androgen-Independent Adenocarcinoma of the Prostate*.

NIH/OBA Receipt Date: 8-18-03. Not Selected for RAC Public Review: 9-08-03
Long-term follow-up: 5-17-07

0309-601 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection

Hall, Simon J., Mount Sinai School of Medicine, New York, New York; *Phase I Trial of Adenovirus-Mediated IL-12 Gene Transduction in Patients with Radiorecurrent Prostate Cancer.*

NIH/OBA Receipt Date: 9-10-03. Not Selected for RAC Public Review: 9-30-03

0309-602 (Closed) Gene Therapy/Cancer/Chronic Myelogenous Leukemia/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Levitsky, Hyam I., Johns Hopkins University School of Medicine, Baltimore, Maryland; *K562/GM-CSF Vaccination in Combination with Imatinib Mesylate for Chronic Myeloid Leukemia*.

NIH/OBA Receipt Date: 9-23-03. Not Selected for RAC Public Review: 10-14-03
Study is closed to new accrual as of 8-24-05.

0309-603 (Open) Gene Therapy/Phase I/Cancer/Prostate/In Vivo/Adenovirus/Serotype 5/Tumor Necrosis Factor-Related Apoptosis-Inducing Ligand (TRAIL) cDNA/Intratumoral Injection

Konety, Badrinath, University of Iowa, Iowa City, Iowa; *A Phase I Study of Ad5-TRAIL in Men with Clinically Organ Confined Prostate Cancer Undergoing Radical Prostatectomy*.

NIH/OBA Receipt Date: 9-25-03. Not Selected for RAC Public Review: 10-16-03

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0309-604 (Closed) Gene Therapy/Phase IIb-III/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration

Sanz, Mark Louis, International Heart Institute of Montana, Missoula, Montana; Bajwa, Tanvir, Heart Car Associates, LLC, Milwaukee, Wisconsin; Carter, Andrew, Providence Heart Institute, Portland, Oregon; Fischell, Tim, Borgess Medical Center, Kalamazoo, Michigan; Antolin, Rosa Ana Hernandez, Hospital Clinico Unmiversitario San Carlos, Madrid, Spain; Beatt, Kevin J., Hammersmith Hospital, London, United Kingdom; Coghlan, John G., Royal Free Hospital, London, United Kingdom; de Belder, Adam, Royal Sussex County Hospital, Brighton, United Kingdom; Dubiel, Jacek S., Zaklad Hermodynamiki, Krakow, Poland; Duque, Marco Antonio Pena, Instituto Nacional de Cardiologia "Ignacio Chavez"; Gil, Robert, Klinik Kardiologii Inwazyjnej, Warszawa, Poland; Hulkuri, Heikki, Oulu University Hospital, Oulu, Finland; Janessens, Stefan, University Hospital Gasthuisberg, Leuven Belgium; Mautner, Branco, Institute of Cardiology and Cardiovascular Surgery of the Favaloro Foundation, Buenos Aires, Argentina; Naslund, Ulf, Norrlans Universitetssjukhs, Umea, Sweden; Naveri, Hannu, Jorvi Hospital, Espoo, Finland; O'Brien, Timothy, University College Hospital, Galway, Ireland; Redwood, Simon R., St. Thomas' Hospital, London, United Kingdom; Rothman, Martin T., The London Chest Hospital, London, United Kingdom; Ruzyllo, Witold, Instytut Kardiologii, Warszawa, Poland; Siegel, Robert M., Advanced Cardiac Specialists, Gilbert, Arizona; Sylven, Christer, Huddinge Universitetssjukhs, Stockholm, Sweden; Thomas, Martyn R., King's College Hospital, London, United Kingdom; Uren, Neal G., Royal Infirmary of Edinburgh, Edinburgh, United Kingdom; Yia-Hertuala, Seppo, University of Kuopio, Kuopio, Finland; Archer, Stephen; University of Alberta, Edmonton, Alberta, Canada; Barbeau, Gerald; Hospital Laval, Ste-Foy Quebec, Canada; Bilodeau, Luc; Montreal Heart Institute, Montreal, Quebec, Canada; Curtis, Michael; Foothills Medical Center, Calgary, Alberta, Canada; Ducas, John; Health Sciences Centre, Winnipeg, Manitoba, Canada; Goulet, Gilles; Saint-Luc Hospital, Montreal, Quebec, Canada; Hilton, J. David; Victoria Heart Institute Foundation, Victoria, BC, Canada; Lemire, Francois; Hotel-Dieu Hospital, Montreal, Quebec, Canada; Marquis, Jean-Francis; University of Ottawa Heart Institute, Ottawa, Ontario, Canada; Niemela, D. Karl; Tampere University Hospital, Tampere, Finland; Schechtmann, Norberto; Holmes Regional Medical Center, Melbourne, Florida; Stewart, Duncan; St. Michael's Hospital, Toronto, Ontario, Canada; and Reeves, Frances; CHUM Hospital Notre Dame, Montreal, Quebec, Canada; *A Multinational Multicenter, Randomized, Double-Blind, Placebo Controlled Study to Evaluate the Efficacy and Safety of Ad5FGF-4 in Patients with Stable Angina*. Sponsor: Cardium Therapeutics

NIH/OBA Receipt Date: 9-25-03. Not Selected for RAC Public Review: 10-16-03
Closed to enrollment: 5-14-04

0309-605 (Closed) Gene Therapy/Cancer/Chronic Myelogenous Leukemia, Acute Myelogenous Leukemia, Myelodysplasia/Elimination of Graft-vs-Host Disease/In Vitro/Allogeneic T Lymphocytes/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous

Kornblau, Steven, M.D. Anderson Cancer Center, Houston, Texas; *A Phase I Study Evaluating Thymidine Kinase Transduced Lymphocytes in Conjunction with Allogeneic Transplant for the Control of Graft-vs-Host Disease and the Induction of Graft-vs-Host Malignancy for Patients with High Risk Chronic Myelogenous Leukemia, Acute Myelogenous Leukemia and Myelodysplasia*.

NIH/OBA Receipt Date: 9-26-03. Not Selected for RAC Public Review: 10-17-03
Closed: 12-2-10; study never activated

0310-606 (Open) Gene Therapy/Cancer/Breast or Ovarian/Immunotherapy/In Vivo/Plasmid DNA/HER2 cDNA/Intradermal Injection

Disis, Mary, University of Washington, Seattle, Washington; *A Phase I Safety and Efficacy of a DNA Plasmid Based Vaccine Encoding the HER2/neu (HER2) Intracellular Domain in Subjects with HER2 Overexpressing Tumors*.

NIH/OBA Receipt Date: 10-2-03. Not Selected for RAC Public Review: 10-24-03

0310-607 (Open) Gene Therapy/Cancer/Renal Cell Carcinoma, Melanoma, or Hematologic Malignancy/Immunotherapy/In Vitro/Allogeneic Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous

Keogh, George, and Vieweg, Johannes, Duke University Medical Center, Durham, North Carolina; *A Pilot Study of Mature, Total-Tumor-RNA-Transfected, Donor-Derived Dendritic Cell Therapy in Patients Who Have Undergone Nonmyeloablative Allogeneic Stem Cell Transplantation for Metastatic Renal Cell Carcinoma, Metastatic Melanoma, or Hematologic Malignancy*.

NIH/OBA Receipt Date: 10-2-03. Not Selected for RAC Public Review: 10-24-03

0310-608 (Closed) Gene Therapy/Phase I/Cancer/EBV-Positive Hodgkin's or Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/LMP2A-Specific Cytotoxic T Lymphocytes (CTL)/Adenovirus/LMP2A cDNA/Intravenous Administration

Bollard, Catherine M., Heslop, Helen, and Rooney, Cliona, Baylor College of Medicine, Houston, Texas; *Administration of LMP2A-Specific Cytotoxic T-Lymphocytes Following CD45 Antibody to Patients with Relapsed EBV-Positive Hodgkin's or Non-Hodgkin's Lymphoma*.

NIH/OBA Receipt Date: 10-6-03. Not Selected for RAC Public Review: 10-27-03
Closed to new enrollment: 9-7-07

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0310-609 (Closed) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Plasmid/NY-ESO-1 cDNA/Particle-Mediated Delivery to Skin

Altorki, Nasser, New York Presbyterian Hospital and Joan and Sanford I. Weill Medical College of Cornell University, New York, New York; and Sharma, Padmanee; University of Texas M.D. Anderson Cancer Center; Houston, Texas; *Safety and Immunological Evaluation of NY-ESO-1 Plasmid DNA (pPJV7611) Cancer Vaccine Given by Particle-Mediated Epidermal Delivery (PMED) in Patients with Tumor Type Known to Express NY-ESO- 1 or LAGE- 1 Antigen A Phase I Trial*.

NIH/OBA Receipt Date: 10-7-03. Not Selected for RAC Public Review: 10-28-03
Study completed in 2007

0310-610 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Immunotherapy/In Vitro/Autologous Bone Marrow-Derived Stromal Cells (BMSC)/Plasmid DNA/Interleukin-12 cDNA/Electroporation/Stereotactic Injection or Via Intracranial Catheter

Mikkelsen, Tom, Henry Ford Hospital, Detroit, Michigan; *A Phase I/II Study of the Treatment of Recurrent or Progressive Malignant Glioma Using Autologous Bone Marrow-Derived Stromal Cells (BMSC) Non-Virally Transduced to Express IL-12*. Sponsor: Cognate Therapeutics.

NIH/OBA Receipt Date: 10-7-03. Publicly Reviewed at the December 2003 RAC meeting

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0310-611 (Closed) Gene Therapy/Cardiovascular Diseases/Peripheral Artery Disease/In Vivo/Skeletal Myofibers/Plasmid DNA/VOP32E VEGF-A Transcription Factor cDNA/Intramuscular Injection

Lederman, Robert, National Institutes of Health, Bethesda, Maryland; and Mendelshohn, Farrell O.; Cardiology Associates, PC; Birmingham, Alabama; *Modulation of Vascular Endothelial Growth Factor (VEGF) Using an Engineered Zinc-Finger Transcription Factor to Treat Lower Limb Intermittent Claudication*.

NIH/OBA Receipt Date: 10-7-03. Not Selected for RAC Public Review: 10-28-03
Closed to new enrollment: 5-20-08; trial complete as of 12-14-10

0310-612 (Closed) Gene Therapy/Phase II/Immunotherapy/In Vitro/Cancer/Non-Small Cell Lung Carcinoma (NSCLC)/Bronchioloalveolar Carcinoma (BAC)/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intradermal Injections

Davies, Angela M., University of California, Davis, Sacramento, California; Mudad, Raja; Tulane University; New Orleans, Louisiana; Taylor, Sarah A., The University of Kansas Medical Center, Kansas City, Kansas; Khurshid, Anwar, Arlington Cancer Center, Arlington, Texas; Budd, G. Thomas, Cleveland Clinic Foundation, Cleveland, Ohio; Rivkin, Saul; Swedish Cancer Institute, Swedish Medical Center; Seattle, Washington; Raftopoulos, Harlambos; Columbia University Medical Center; New York, New York; Kelly, Karen; University of Colorado Cancer Center; Aurora, Colorado; Wozniak, Antoinette J.; Wayne State University; Detroit, Michigan; and Kraut, Michael; St. John Providence Cancer Institute; Southfield, Michigan; *Phase II Trial of CG8123, an Autologous Cancer Vaccine (GVAX), in Patients with Selected Stage IIIB and IV Bronchioloalveolar Carcinoma (BAC)*. Sponsor: Southwest Oncology Group.

NIH/OBA Receipt Date: 10-24-03. Not Selected for RAC Public Review: 11-14-03
Closed to accrual: 08-19-05

0311-613 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Sarcoma or Neuroblastoma/In Vivo/Herpes Simplex Virus Type 1/Vector-Directed Tumor Cell Lysis/Rat Prodrug Enzyme CYP2B1 cDNA/Intratumoral Injection

Cripe, Timothy P., Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio; *A Phase I Dose Escalation Study of Intratumoral Herpes Simplex Virus-1 Mutant in Patients with Refractory Sarcoma or Neuroblastoma*.

NIH/OBA Receipt Date: 11-6-03. Publicly Reviewed at the June 2005 RAC meeting

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0311-614 (Open; RAC Reviewed with Recommendations) Non-therapeutic (Healthy Volunteers)/Dental Caries Prevention/In Vivo/Streptococcus mutans/Oral Administration

Stone, Constance E., University of Florida, Gainesville, Florida; *First Time in Human Safety Study of Streptococcus mutans Lactic Acid-Deficient Effector Strain (A2JM) Administered in Conjunction with Twice Daily Dose of D-alanine Mouthwash in Healthy Adult Male Subjects for Replacement Therapy as an Aid in the Protection against Dental Caries*. Sponsor: Oragenics, Inc.

NIH/OBA Receipt Date: 11-20-03. Publicly Reviewed at the March 2004 RAC meeting

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0311-615 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/gp100 Melanoma Antigen/Intramuscular Administration of a Nonmyeloablative Lymphocyte Depleting Regimen

Rosenberg, Steven A., National Institutes of Health, Bethesda, Maryland; *Phase II Study in Metastatic Melanoma Using Lymphocytes Reactive with the GP100 Antigen and Immunization Using a Recombinant rF-gp100P209 Virus Encoding a gp100 Peptide Following a Nonmyeloablative Lymphocyte Depleting Regimen*.

NIH/OBA Receipt Date: 11-25-03. Not Selected for RAC Public Review: 12-16-03

0312-616 (Open) Gene Therapy/Phase I-II/Cancer/Colon Carcinoma (Hepatic Metastasis)/Herpes Simplex Virus Type-1/Vector-Directed Cell Lysis/Intrahepatic Artery Administration

Kemeny, Nancy; Memorial Sloan-Kettering Cancer Center, New York, New York; Nemunaitis, John J.; Mary Crowley Medical Research Center, Dallas, Texas; Reid, Tony, University of California, San Diego, San Diego, California; Chari, Ravi; Vanderbilt University; Nashville, Tennessee; Geller, David A.; UPMC Presbyterian; Pittsburg, Pennsylvania; Galanis, Evanthia; Mayo Clinic; Rochester, Minnesota; and Tanabe, Kenneth; Massachusetts General Hospital; Boston, Massachusetts; *A Phase I/II, Open-Label Study (with a Sequential Dose Escalation Stage Followed by an Expansion of a Selected Dose Cohort), to Evaluate the Safety and Anti-Tumor Effects of NV1020, Administered Repeatedly Via Hepatic Artery Infusion Prior to Second-Line Chemotherapy, in Patients with Colorectal Adenocarcinoma Metastatic to the Liver*. Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 12-2-03. Not Selected for RAC Public Review: 2-3-04

0312-617 (Open) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vivo/Plasmid DNA/Human and Mouse Prostate Specific Membrane Antigen cDNAs/Intramuscular Injection

Slovin, Susan F., Memorial Sloan-Kettering Cancer Center, New York, New York; *Injection of Renal Cell Carcinoma Patients with Human and Mouse Prostate Specific Membrane Antigen (PSMA) DNA: A Phase I Trial to Assess Safety and Immune Response*.

NIH/OBA Receipt Date: 12-9-03. Not Selected for RAC Public Review: 12-30-03

0312-618 (Open) Gene Therapy/Phase I/Cancer/CEA Expressing Cancers/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection

Marshall, John, Georgetown University Medical Center, Washington, D.C.; *Randomized Single Institution Pilot Study of Vaccinia-CEA(6D0-TRICOM with GM-CSF in Combination with Docetaxel in Patients with CEA-Bearing Cancers*.

NIH/OBA Receipt Date: 12-18-03. Not Selected for RAC Public Review: 2-11-04

0312-619 (Open) Gene Therapy/Monogenic Disease/ Late Infantile Neuronal Ceroid Lipofuscinosis/In Vivo/Brain/Adeno-Associated Virus 2/CLN2 (Tripeptidyl Peptidase) cDNA/Intraparenchymal Injection

Crystal, Ronald, Weill Medical College of Cornell University, New York, New York; *Administration of a Replication Deficient Adeno-Associated Virus Gene Transfer Vector Expressing the Human CLN2 cDNA to the Brain of Children with Late Infantile Neuronal Ceroid Lipofuscinosis*.

NIH/OBA Receipt Date: 12-29-03. Not Selected for RAC Public Review: 1-20-04

0312-620 (Open) Gene Therapy/Phase I/Cancer/Squamous Cell Carcinoma of the Head and Neck/Radiotherapy/In Vivo/Adenovirus/Serotype 5/Human Sodium-Iodide Symporter (NIS) cDNA/Intratumoral Injection

Trask, Douglas K. and Domann, Frederick E., Carver College of Medicine, The University of Iowa, Iowa City, Iowa; *A Phase I Study of Genetically Targeted Radiotherapy Using Intratumoral Administration of Adenovirus Expressing the Sodium-Iodine Symporter (Ad-NIS) Plus Systemic ^{131}I in Subjects with Refractory Squamous Cell Carcinoma of the Head and Neck*.

NIH/OBA Receipt Date: 12-30-03. Not Selected for RAC Public Review: 1-21-04

0401-621 (Open) Gene Therapy/Phase I/Cancer/Advanced Myelodysplasia (MDS) or Refractory Acute Myeloid Leukemia (AML)/Immunotherapy/In Vitro/Autologous Myeloblasts/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous Injection

Ho, Vincent T., Dana-Farber Cancer Institute and Harvard Medical School, Boston, Massachusetts; *GM-CSF Secreting Leukemia Cell Vaccinations after Allogeneic Non-Myeloablative Peripheral Blood Stem Cell Transplantation in Patients with Advanced Myelodysplastic Syndrome or Refractory Acute Myeloid Leukemia***.**

NIH/OBA Receipt Date: 1-6-04. Not Selected for RAC Public Review: 1-27-04

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0401-622 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Other Diseases-Disorders/Congestive Heart Failure (CHF)/In Vivo/Adenovirus/Serotype 5/Adenylyl Cyclase Type VI (AC_{VI}) cDNA/Intracoronary Injection

Penny, William F.; University of California, San Diego; San Diego, California; and Henry, Timothy; Minneapolis Heart Institute Foundation; Minneapolis, Minnesota; *Adenylyl Cyclase VI Gene Transfer for CHF*.

NIH/OBA Receipt Date: 1-9-04. Publicly Reviewed at the March 2004 RAC meeting

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0401-623 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Other Diseases-Disorders/Alzheimer's Disease/In Vivo/Adeno-Associated Virus/Serotype 2/Nerve Growth Factor cDNA/Intracranial Injection

Bennett, David A., Rush University Medical Center, Chicago, Illinois; and Fleisher, Adam; University of California San Diego; San Diego, California; *A Phase I/II, Dose-Escalating, Randomized and Controlled Study to Assess the Safety, Tolerability, and Efficacy of CERE-110 [Adeno-Associated Virus (AAV)-based, Vector-Mediated Delivery of Beta-Nerve Growth Factor (β-NGF)] in Subjects with Mild to Moderate Alzheimer's Disease*. Sponsor: Ceregene, Inc.

NIH/OBA Receipt Date: 1-12-04. Publicly Reviewed at the March 2004 RAC meeting
Closed to new enrollment: 6-05

0401-624 (Open) Gene Therapy/Phase I-II/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Adenovirus/Serotype 5/Replication-Competent Virus/RGD Motif/Intratumoral Injection

Lang, Frederick F. and Conrad, Charles A., The University of Texas M.D. Anderson Cancer Center, Houston, Texas; *Phase I Trial of Conditionally Replication-Competent Adenovirus (Delta-24-RGD) for Recurrent Malignant Gliomas*.

NIH/OBA Receipt Date: 1-12-04. Publicly Reviewed at the March 2004 RAC meeting

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0401-625 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Ovarian/Vector-Directed Cell Lysis/In Vivo/Adenovirus/Serotype 5/Replication –Competent Virus/RGD Motif/Intraperitoneal Injection

Alvarez, Ronald D., Barnes, Mack N., and Curiel, David T., University of Alabama at Birmingham, Birmingham, Alabama; *A Phase I Study of a Tropism Modified Conditionally Replicative Vector (Ad5-Δ24RGD) for Intraperitoneal Delivery in Ovarian and Extraovarian Cancer Patients*.

NIH/OBA Receipt Date: 1-13-04. Publicly Reviewed at the March 2004 RAC meeting

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0401-626 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vivo/Plasmid in Poly (DL-lactide-coglycolide) (PLG) Microparticles/Cytochrome P450 Isoenzyme 1B1 (CYP1B1) cDNA/Subcutaneous Injection

Gribben, John G., Dana-Farber Cancer Institute and Harvard Medical School, Boston, Massachusetts; *A Phase I Study to Determine the Feasibility and Safety of Vaccinating Cancer Patients with ZYC300 in Combination with GM-CSF and Imiquimod with or without Cyclophosphamide Pre-Dosing*.

NIH/OBA Receipt Date: 1-13-04. Not Selected for RAC Public Review: 2-3-04

0401-627 (Open) Gene Therapy/Phase I-II/Cancer/Epstein-Barr Virus Lymphoma/Pro-Drug/Elimination of Graft Versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous

O'Reilly, Richard J., Memorial Sloan-Kettering Cancer Center, New York, New York; *A Phase I-II Trial of EBV-Sensitized Donor T-Cells Transduced with a Dicistronic Retroviral Vector, Termed NIT, Encoding a Mutant Human Nerve Growth Factor Receptor (mLNGFR) and Herpes Simplex Virus Thymidine Kinase (HSV-TK) for the Treatment of EBV Lymphoma in Marrow and Organ Allograft Recipients, and in Patients with Genetic Immunodeficiencies*.

NIH/OBA Receipt Date: 1-13-04. Not Selected for RAC Public Review: 2-3-04

0401-628 (Closed) Gene Therapy/Other Diseases-Disorders/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Percutaneous Cardiac Catheterization/Intra-Myocardial Injection

Losordo, Douglas W., St. Elizabeth's Medical Center, Boston, Massachusetts; *An Evaluation of a 800 μg dose of pVGI.1 (VEGF2) Percutaneously Delivered Via Boston Scientific Corporation Stiletto™ Endocardial Injection Catheter in up to Seven Angina Patients Who Previously Received a Placebo Dose within Study Protocol VEGF2-CAD-CL-005*. Sponsor: Corautus Genetics, Inc.

NIH/OBA Receipt Date: 1-13-04. Not Selected for RAC Public Review: 2-3-04
Never initiated: 5-23-06

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0401-629 (Open) Gene Therapy/Phase I/Cancer/Superficial Injectable Tumors/Vaccinia Virus/Vector-Directed Tumor Lysis/Pro-Drug/Cytosine Deaminase cDNA/5-FC/Somatostatin Receptor cDNA/Intratumoral Injection

Zeh, Herbert J, III, University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *A Phase I Dose-Escalation Trial of vvDD-CDSR (Double-Deleted Vaccinia Virus Plus CD/SMR) Administered by Intratumoral Injection in Patients with Superficial Injectable Tumors*.

NIH/OBA Receipt Date: 1-13-04. Publicly Reviewed at the March 2004 RAC meeting

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0401-630 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Human Telomerase Reverse Transcriptase (hTERT) cDNA/Intradermal Injections

Dahm, Philipp and Vieweg, Johannes, Duke University Medical Center, Durham, North Carolina; *Active Immunotherapy with Mature, Human Telomerase Reverse Transcriptase RNA-Transfected, Autologous Dendritic Cells with or without the IL-2 Diphtheria Toxin Conjugate Denileukin Difitox (ONTAK^{R}) in Subjects with Metastatic Prostate Cancer*.

NIH/OBA Receipt Date: 1-16-04. Not Selected for RAC Public Review: 2-6-04

0401-631 (Open) Gene Therapy/Phase I/Cancer/CEA Expressing Cancers/Liver Metastasis/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/Subcutaneous Injection

Gulley, James, National Institutes of Health, Bethesda, Maryland and Hoffmeister, Karen, National Naval Medical Center, Bethesda, Maryland*; A Pilot Trial of a CEA-TRICOM Based Vaccine and Radiation to Liver Metastasis in Adults with CEA Positive Solid Tumors*.

NIH/OBA Receipt Date: 1-21-04. Not Selected for RAC Public Review: 2-10-04

0403-632 (Open) Gene Therapy/Phase I-II/Diabetic Peripheral Neuropathy/In Vivo/Plasmid/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Losordo, Douglas W., St. Elizabeth's Medical Center, Boston, Massachusetts; and Gooch, Clifton; Columbia University; New York, New York; *pVGI.1 (VEGF-2) Gene Transfer for Diabetic Neuropathy*.

NIH/OBA Receipt Date: 3-18-04. Not Selected for RAC Public Review: 4-7-04

0403-633 (Closed; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Autoimmune Disease--Multiple Sclerosis (MS)/Immunotherapy/In Vivo/Plasmid DNA/Myelin Basic Protein (hMBP) cDNA/Intramuscular Injection

Vollmer, Timothy L.; Barrow Neurological Institute, St. Joseph's Hospital and Medical Center; Phoenix, Arizona; and Weiner, Leslie P.; University of Southern California; Los Angeles, California; *Phase I Trial of Immunotherapy with BHT-3009 Alone or Combined with Atorvastatin in Patients with Multiple Sclerosis*. Sponsor: Bayhill Therapeutics

NIH/OBA Receipt Date: 3-22-04. Publicly Reviewed at the June2004 RAC meeting
Closed to accrual: 6-16-06

0403-634 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Fowlpox Virus/B7.1 (CD80)/ICAM-1/LFA-3/Intratumoral Injection

Gajewski, Thomas F., University of Chicago Medical Center, Chicago, Illinois**;** *Phase II Study of Intratumoral Injection of rF-TRICOM^{TM} in Patients with Metastatic Melanoma Who have Detectable Circulating Melanoma-Specific T Cells***.**

NIH/OBA Receipt Date: 3-9-04. Not Selected for RAC Public Review: 3-29-04

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0403-635 (Closed) Gene Therapy/Phase III/Cancer/Pancreas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection

Marshall, John, Lombardi Cancer Research Center, Georgetown University Medical Center, Washington, DC; DiStefano, Alfred, Arlington Cancer Center, Arlington, Texas; Morse, Michael; Duke University Medical Center, Durham, North Carolina; Gurtler, Jayne; Gurtler and Brinz, APMC, Metairie, Louisiana; Nemunaitis, John; Mary Crowley Medical Research Center, Dallas, Texas; Rowland, Kendrith; Carle Cancer Center, Urbana, Illinois; Geils, George F. Jr.; Charleston Hematology-Oncology, PA, Charleston, South Carolina; Hagan, M. Kelly; Virginia Cancer Institute, Richmond, Virginia; Langdon, Robert M.; Nebraska Methodist Hospital, Omaha, Nebraska; Lenz, Heinz-Josef; USC/Norris Comprehensive Cancer, Los Angeles, California; Eisenberg, Peter; California Cancer Care, Greenbrae, California; Holladay, Charles S.; Charleston Cancer Center, Charleston, South Carolina; Ervin, Thomas J., Maine Center for Cancer Medicine and Blood Disorders, Scarborough, Maine; Waterfield, William, Franklin Square Hospital Center, Baltimore, Maryland; Gold, Philip, Swedish Cancer Institute, Seattle, Washington; Belt, Robert, Kansas City Cancer Center, Kansas City, Missouri; Melin, Susan, Wake Forest University, Winston-Salem, North Carolina; Page, Ray D., Texas Cancer Care, Fort Worth, Texas; Brammer, Melissa, Tulane Cancer Center, New Orleans, Louisiana; Tchekmedyian, Nerses Simon, Pacific Shores Medical Group, Long Beach, California; Hainsworth, John D., The Sarah Cannon Cancer Center, Nashville, Tennessee; Gabrail, Nashat Y., Gabrail Cancer Center, Canton, Ohio; Perry, David J., Washington Hospital Center, Washington Cancer Institute, Washington, DC; Stone, Michael D., Greeley Medical Clinic, Greeley, Colorado; Antonia, Scott, H. Lee Moffitt Cancer Center & Research Institute, Tampa, Florida; Dreisbach, Luke, Desert Hematology Oncology Medical Group, Inc., Rancho Mirage, California; Kaufman, Howard, Columbia University, New York, New York; Robles, Carlos, Miami VA Medical Center, Miami, Florida; Ozer, Howard; University of Oklahoma Health Sciences Center; Oklahoma City, Oklahoma; Wisenfeld, Martin; Oncology Associates; Cedar Rapids, Iowa; Reich, Elizabeth; Jupiter Medical Center; Jupiter, Florida; Poplin, Elizabeth; Cancer Institute of New Jersey, New Brunswick, New Jersey; Treisman, Jonathan; Medical Consultants, Ltd.; Milwaukee, Wisconsin; Walters, Theodore; St. Luke's Mountain States Tumor Institute; Boise, Idaho; DeSimone, Philip A.; University of Kentucky, Markey Cancer Center, Lexington, Kentucky; Anthony, Stephen P.; Cancer Care Northwest; Spokane, Washington; Damjanov, Nevena; Temple University; Philadelphia, Pennsylvania; Reid, Tony; University of California, San Diego Cancer Center; LaJolla, California; Wollner, Ira; Henry Ford Health System; Detroit, Michigan; Talavera, Joyce; Tufts New England Medical Center; Boston, Massachusetts; Frank, Richard; Norwalk Hospital; Norwalk, Connecticut; Picus, Joel; Washington University School of Medicine; St. Louis, Missouri; Raju, Robert N.; Dayton Oncology & Hematology, P.A.; Kettering, Ohio; Richards, Donald; Tyler Cancer Center; Tyler, Texas; Berman, Barry; Cancer Centers of Florida, P.A.; Ocoee, Florida; Arsenau, James; New York Oncology Hematology, PC; Albany, New York; Stephenson, Joe, Jr.; Cancer Centers of the Carolinas; Greenville, South Carolina; Tezcan, Haluk; North Idaho Cancer Center; Coeur d'Alene, Idaho; Benedetto, Pasquale; University of Miami School of Medicine; Miami, Florida; Kemeny, Margaret; Queens Hospital Center; Queens, New York; Hecht, J. Randolph; University of California, Los Angeles; Los Angeles, California; Kane, Madeline; University of Colorado Health Science Center; Aurora, Colorado; Pipas, J. Marc; Norris-Cotton Cancer Center, Dartmouth-Hitchcock Medical Center; Lebanon, New Hampshire; Castine, Michael J., III; Medical Oncology, LLC; Baton Rouge, Louisiana; Khong, Hung; University of South Alabama Cancer Research Institute; Mobile, Alabama; Zhang, Paul; Hematology Oncology Life Center, Alexandria, Louisiana; Miller, William; Scripps Cancer Center; San Diego, California; Hindenburg, Alexander, Winthrop University Hospital; Mineola, New York; Kauh, John; Winship Cancer Institute, Emory University School of Medicine; Atlanta, Georgia; Yadav, Sanjav; Hematology Oncology Consultants, Inc.; Columbus, Ohio; Fisher, William E.; Elkins Pancreas Center, Baylor College of Medicine; Houston, Texas; Warr, Thomas A.; Great Falls Clinic, LLP; Great Falls, Montana; Visconti, John; St. Louis University Cancer Center; St. Louis, Missouri; Conkling, Paul; Virginia Oncology Associates; Norfolk, Virginia; Reid, Tony; University of California, San Diego; San Diego, California; Karimi, Misagh; University of California, Irvine; Orange, California; Schlossman, David; Missouri Cancer Associates; Columbia, Missouri; Cohn, Allan; Rocky Mountain Cancer Centers; Denver, Colorado; Javle, Milind; Roswell Park Cancer Institute; Buffalo, New York; Smith, David A.; Northwest Cancer Specialists, P.C.; Vancouver, Washington; Cartwright, Thomas H.; Ocala Oncology Center; Ocala, Florida; Preti, Alejandro; H. Alejandro Preti; Houston, Texas; McKenney, Scott; Texas Oncology; Beaumont, Texas; Flynn, Patrick J.; Minnesota Oncology Hematology, P.A.; Minneapolis, Minnesota; Hellerstedt, Beth; Texas Oncology, PA; Austin, Texas; and Kindler, Hedy Lee; University of Chicago; Chicago, Illinois; *A Phase III Randomized, Controlled Study to Evaluate the Safety and Efficacy of PANVAC^{TM}-VF in Combination with GM-CSF Versus Best Supportive Care of Palliative Chemotherapy in Patients with Metastatic (Stage IV) Adenocarcinoma of the Pancreas Who Have Failed a Gemcitabine-Containing Chemotherapy Regimen*. Sponsor: Therion Biologics Corporation

NIH/OBA Receipt Date: 3-8-04. Not Selected for RAC Public Review: 3-29-04
Closed: 5-17-07

0403-636 (Closed) Gene Therapy/Phase I/Cancer/Colorectal Adenocarcinoma/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection

Gulley, James L., National Institutes of Health, Bethesda, Maryland; *An Open Label Pilot Study to Evaluate the Safety and Tolerability of PANVAC^{TM}-V and PANVAC^{TM}-F in Combination with Sargramostim in Patients with Metastatic Carcinoma*.

NIH/OBA Receipt Date: 3-26-04. Not Selected for RAC Public Review: 4-15-04
Closed to accrual: 08-03-12

0403-637 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Vector-Directed Cell Lysis/In Vivo/Adenovirus Type 5/Replication-Competent Virus/Promoter and Enhancer Elements of the Prostate Specific Antigen/Intravenous Injection

Small, Eric, UCSF Comprehensive Cancer Center, San Francisco, California; and Senzer, Neil; Mary Crowley Medical Research Center, Dallas, Texas; *A Phase 1/2a Dose-Escalation Trial of Intravenous CG7870 in Combination with Docetaxel in Chemotherapy-Naïve Patients with Metastatic Hormone-Refractory Prostate Cancer*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 3-29-04. Not Selected for RAC Public Review: 4-16-04
Closed: 6-29-05

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0404-638 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Disease/Alpha-1 Antitrypsin Deficiency/In Vivo/Adeno-Associated Virus/Serotype 1/Alpha-1 Antitrypsin cDNA/Intramuscular

Flotte, Terence R., University of Massachusetts Medical School, Worcester, Massachusetts; *Phase I Trial of Intramuscular Injection of a Recombinant Adeno-Associated Virus 1-Alpha 1-Antitrypsin (rAAV-CB-hAAT) Gene Vector to AAT-Deficient Adults***.**

NIH/OBA Receipt Date: 4-6-04. Publicly Reviewed at the June 2004 RAC meeting

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0404-639 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3) galactosyltransferase Gene/Subcutaneous Injection

Hemstreet, George P., University of Nebraska Medical Center, Omaha, Nebraska; *A Phase I/II Study of an Antitumor Vaccination Using α(1,3) galactosyltransferase Expressing Allogeneic Tumor Cells in Patients with Hormone Refractory Prostate Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

0404-640 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3) galactosyltransferase Gene/Intradermal Injection

Morris, John C., National Institutes of Health, Bethesda, Maryland; and Riker, Adam; H. Lee Moffitt Cancer Center; Tampa, Florida; *A Phase I/II Study of an Antitumor Vaccination Using α(1,3) galactosyltransferase [α(1,3) GT] Expressing Allogeneic Tumor Cells in Patients with Recurrent or Refractory Malignant Melanoma*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

0404-641 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Colorectal, Pancreatic, and Non-Small Cell Lung Cancers/Immunotherapy/In Vivo/*Saccharomyces cerevisiae*/Mutated Ras Oncoproteins/Subcutaneous Injections

O'Neil, Bert, University of North Carolina School of Medicine, Chapel Hill, North Carolina; Cohn, Allen, Rock Mountain Cancer Centers, Denver, Colorado; Morse, Michael A., Duke University Medical Center, Durham, North Carolina; Back, Anthony; Seattle Cancer Care Alliance; Seattle, Washington; and Kelly, Karen; University of Colorado Health Sciences Center; Aurora, Colorado; *A Phase I Open Label, Non-Randomized, Dose-Escalation, Multi-Center, Therapeutic Trial of the Safety, Immunogenicity and Efficacy of GI-4000, an Inactivated Recombinant Saccharomyces cerevisiae Immunotherapeutic Expressing Three Different Mutations of the Ras Oncoprotein, in Patients with Solid Tumors Expressing Mutations in Ras*. Sponsor: GlobeImmune, Inc.

NIH/OBA Receipt Date: 4-13-04. Publicly Reviewed at the June 2004 RAC meeting

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0404-642 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Heart Failure/In Vivo/Adeno-Associated Virus/Serotype 6/Sarcoplasmic Reticulum Calcium ATPase 2a (SERCA2a) cDNA/Intramyocardial Injection

London, Barry, University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania; *Phase I Trial of Gene Transfer During VD Support with SERCA2a*.

NIH/OBA Receipt Date: 4-13-04. Publicly Reviewed at the June 2004 RAC meeting

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0404-643 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Bladder/Vector-Directed Cell Lysis/In Vivo/Adenovirus Serotype 5/Conditionally Replication Competent Virus/GM-CSF/Intravesical Administration

Nemunaitis, John, US Oncology, Dallas, Texas; Corman, John M.; Virginia Mason Medical Center; Seattle, Washington; Lamm, Donald; BCG Oncology, Phoenix, Arizona; Stephenson, Joe, Jr.; Cancer Centers of the Carolinas; Greenville, North Carolina; Arseneau, James C.; Albany Regional Cancer Center; Albany, New York; McKiernan, James M.; Columbia University; New York; New York; Purcell, William T.; Billings Clinical Research Center; Billings, Montana; Meng, Maxwell; UCSF Comprehensive Cancer Center; San Francisco, California; Lerner, Seth P.; Baylor College of Medicine; Houston, Texas; and Garbo, Lawrence E.; New York Oncology Hematology, P.C.; Albany, New York; *A Phase I/II Dose-Escalation Trial of Intravesical CG0070 for Superficial Transitional Cell Carcinoma of the Bladder after Bacillus Calmette-Guerin Failure*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 4-13-04. Publicly Reviewed at the June 2004 RAC meeting

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0404-644 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Adenovirus Serotype 5/Fusion Protein of MUC-1 and CD40 Ligand (CD40L) cDNA/Subcutaneous Injection

Deisseroth, Albert B., Sidney Kimmel Cancer Center, San Diego, California; *A Single Arm Open-Label Phase I Study of an Injectable Replication-Incompetent Adenoviral Vector Vaccine with Protein Boost Used to Produce an Immune Response for MUC-1 Positive Epithelial Cancer Cells in Advanced Breast Cancer Patients*.

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0404-645 (Open) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/Plasmid/Fibroblast Growth Factor-1 (FGF-1) cDNA/Intramuscular Injections

Henry, Tim, Minneapolis Hear Institute Foundation, Minneapolis, Minnesota; *Double-Blind, Randomized, Placebo-Controlled, Parallel Group and Dose-Finding, Multicentric, Safety and Efficacy Study with Intramuscular Injections of NV1FGF in Subjects with Intermittent Claudication*. Sponsor: Gencell

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

0404-646 (Open) Gene Therapy/Phase I-II/Infectious Diseases/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Lentivirus/HIV-1/Antisense env/Intravenous

Stein, David, Albert Einstein College of Medicine, Bronx, New York; Steinhart, Corklin R.; Steinhart Medical Associates; Miami, Florida; Zolopa, Andrew; Stanford University Medical Center; Stanford, California; and Blick, Gary; CIRCLE Medical, LLC; Norwalk, Connecticut; *A Phase I/II, Open-Label, Multicenter Study to Evaluate the Safety, Tolerability, and Biological Activity of Repeated Doses of Autologous T Cells Transduced with VRX496 in HIV-Positive Patients*. Sponsor: VIRxSYS

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-10-04

0404-647 (Open) Gene Therapy/Phase II/Cancer/Superficial Injectable Tumors/Vaccinia Virus/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) and Humanized Escherichia coli β galactosidase cDNAs/Intratumoral Injection

Nemunaitis, John, US Oncology, Dallas, Texas; Stephenson, Joe J., Jr.; The Cancer Centers of the Carolinas; Greenville, South Carolina; and Ribas, Antonia; University of California, Los Angeles; Los Angeles, California; *A Phase I/II Dose-Escalation Trial of JX-594 (Thymidine Kinase-Deleted Vaccinia Virus Plus GM-CSF) Administered by Intratumoral Injection in Patients with Superficial Injectable Tumors*. Sponsor: Jennerex Biotherapeutics, Inc.

NIH/OBA Receipt Date: 4-13-04. Not Selected for RAC Public Review: 5-3-04

0404-648 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous and Intraprostatic Injections

Gulley, James L., National Institutes of Health, Bethesda, Maryland; *A Phase I Feasibility Study of an Intraprostatic PSA-Based Vaccine in Prostate Cancer Patients with Local Failure Following Radiotherapy or Clinical Progression on Androgen Deprivation Therapy in the Absence of Local Definitive Therapy*.

NIH/OBA Receipt Date: 4-19-04. Not Selected for RAC Public Review: 5-7-04

0404-649 (Open) Gene Therapy/Phase II/Cancer/Hepatic Metastasis of Colorectal Adenocarcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injections

Morse, Michael, Duke University Medical Center, Durham, North Carolina; *A Phase II Study of Active Immunotherapy with PANCAC™ or Autologous Cultured Dendritic Cells Infected with PANVAC™ After Complete Resection of Hepatic Metastasis of Colorectal Carcinoma*.

NIH/OBA Receipt Date: 4-27-04. Not Selected for RAC Public Review: 5-17-04

0405-650 (Closed) Gene Therapy/Phase II/Cancer/Immunotherapy/In Vivo/Vaccinia/Fowlpox virus/NY-ESO-1 cDNA/Subcutaneous Injection

Odunsi, Kunle O.; Roswell Park Cancer Institute; Buffalo, Buffalo, New York; *Phase II Study of Recombinant Vaccinia-NY-ESO-1 (rV-NY-ESO-1) and Recombinant Fowlpox-Ny-ESO-1 (rF-NY-ESO-1) in Patients with Epithelial Ovarian, Fallopian Tube or Primary Peritoneal Carcinoma Whose Tumors Express NY-ESO-1 or LAGE-1 Antigen*.

NIH/OBA Receipt Date: 5-6-04. Not Selected for RAC Public Review: 6-4-04
Closed, long-term follow-up continues: 4-19-10

0405-651 (Closed) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD 80)/ICAM-1/LFA-3/Subcutaneous Injection

Arlen, Philip M., National Institutes of Health, Bethesda, Maryland; *A Pilot Trial of a CEA/TRICOM-Based Vaccine in Combination with Combined Chemotherapy/Radiotherapy in Patients with Unresectable Stage III Non-Small Cell Lung Cancer (NSCLC)*.

NIH/OBA Receipt Date: 5-13-04. Not Selected for RAC Public Review: 6-4-04
No individuals enrolled: 1-9-06

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0405-652 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vivo/Plasmid DNA/HPV16 E7 cDNA/Intramuscular Injection

Forastiere, Arlene; Johns Hopkins Comprehensive Cancer Center; Baltimore, Maryland; *An Open-Label Phase One Study of the Safety and Immunogenicity of Repeated Vaccination with NGVL4a-HPV16Sig/E7(detox)/HSP70 in Patients with Stage III or IV HPV 16-Positive Head and Neck Squamous Cell Carcinoma (HNSCC)*.

NIH/OBA Receipt Date: 5-18-04. Not Selected for RAC Public Review: 6-8-04

0405-653 (Closed) Gene Therapy/Phase III/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Curti, Brendan D.; Providence Portland Medical Center; Portland, Oregon; Ernstoff, Marc; Dartmouth-Hitchcock Medical Center; Hanover, New Hampshire; Hauke, Ralph; University of Nebraska Medical Center; Omaha, Nebraska; Decker, David A.; William Beaumont Hospital; Royal Oak, Michigan; Siegel, Leonard; Bienes Comprehensive Cancer Center/Holy Cross Hospital; Fort Lauderdale, Florida; Corman, John; Virginia Mason Medical Center; Seattle, Washington; Nemunaitis, John; Mary Crowley Medical Research Center; Dallas, Texas; Richards, Donald; Tyler Cancer Center; Tyler, Texas; Poiesz**,** Bernard J.; SUNY Upstate Medical University; Syracuse, New York; Geils, George F., Jr.; Charleston Hematology Oncology, PA; Charleston, South Carolina; Macapinlac, Manuel, Jr.; Jacoby Medical Center; Bronx, New York; Assikis, Vasily, Winship Cancer Institute, Emory University; Atlanta, Georgia; Nieva, Jorge J.; Scripps Cancer Center, San Diego, California; Govindarajan, Rangaswamy; University of Arkansas for Medical Sciences; Little Rock, Arkansas; Hogan, Thomas F.; Mayo Clinic Scottsdale; Scottsdale, Arizona; Bidair, Mohamed; Center for Urological Research; La Mesa, California; Chatta, Gurkamal; University of Pittsburgh Cancer Institute; Pittsburgh, Pennsylvania; Drake, Charles; Sidney Kimmel Cancer Center at Johns Hopkins; Baltimore, Maryland; Whitlock, Norris; The Urology Group; Spartansburgh, South Carolina; Culkin, Daniel; University of Oklahoma; Oklahoma City, Oklahoma; Dreicer, Robert; Cleveland Clinic Foundation; Cleveland, Ohio; Khong, Hung T.; University of South Alabama Cancer Research Institute; Mobile, Alabama; Baron, Ari; Pacific Hematology Oncology Associates, California Pacific Medical Center; San Francisco, California; Tannock, Ian; Princess Margaret Hospital; Ontario, Canada; Higano, Celestia; Seattle Cancer Care Alliance; Seattle, Washington; Purcell, William T.; Deaconess Billings Clinic; Billings, Montana; Peace, David; University of Illinois at Chicago; Chicago, Illinois; Preti, Alejandro; Discovery Alliance, Inc.; Houston, Texas; Leibowitz, Leslie; Heamotology-Oncology Associates of Northern New Jersey; Morristown, New Jersey; Zehngebot, Lee M.; Hematology and Oncology Consultants, PA; Orlando, Florida; Tirona, Maria R. B. Tria; Marshall University; Huntington, West Virginia; Fruehauf, John P.; University of California, Irvine Medical Center; Orange, California; Glode, Michael; University of Colorado Denver and Health Sciences Center; Aurora, Colorado; Petrylak, Dan; Columbia University; New York, New York; Berman, Barry; Cancer Centers of Florida, PA; Ocoee, Florida; Papish, Steven W.; Hematology-Oncology Associates of Northern New Jersey, Carol G. Simon Cancer Center; Morristown, New Jersey; Harris, Wayne B.; Veterans Affairs Medical Center; Decatur, Georgia; Cohen, Seth; St. Luke's-Roosevelt Hospital Center; New York, New York; Donnell, Robert F.; Medical College of Wisconsin; Milwaukee, Wisconsin; Elrafei, Tarek; Jacobi Medical Center; Bronx, New York; and Alemany, Carlos; Cancer Centers of Florida; Ocoee, Florida; *A Phase III Randomized, Open-Label Study of CG1940 and CG8711 Versus Docetaxel and Prednisone in Patients with Metastatic Hormone-Refractory Prostate Cancer Who are Chemotherapy-Naïve*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 5-20-04. Not Selected for RAC Public Review: 6-10-04
Closed: 10-16-08

0406-654 (Open) Gene Therapy/Cancer/Mantle Cell Lymphoma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/Electroporation/DMRIE-Cholesterol/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/CD40 Ligand cDNA/Intradermal Injection

Dessureault, Sophie, The Moffitt Cancer Center and Research Institute, University of South Florida, Tampa, Florida; *A Phase II Trial Using a Universal GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) in the Formulation of Autologous Tumor Cell-Based Vaccines for Patients with Mantle Cell Lymphoma*.

NIH/OBA Receipt Date: 61-04. Not Selected for RAC Public Review: 6-22-04

0406-655 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Human Telomerase Reverse Transcriptase (hTERT)/Intradermal Injections

Dahm, Philipp, Duke University Medical Center, Durham, North Carolina; *Active Immunotherapy with Human LAMP Telomerase mRNA-Transfected Immature, Autologous Dendritic Cells Following In-Situ Priming with the Immunostimulant Imiquimod (ALDARA^{TM}) in Patients with Metastatic Prostate Cancer*.

NIH/OBA Receipt Date: 6-9-04. Not Selected for RAC Public Review: 6-29-04

0407-656 (Open) Gene Therapy/Phase I-II/Other/Diabetic Peripheral Neuropathy/In Vivo/Skeletal Muscle Myocytes/Plasmid DNA/VOP32E VEGF-A Transcription Factor cDNA/Intramuscular Injection

Cornblath, David, Johns Hopkins Hospital, Baltimore, Maryland; *A Phase I/II, Dose-Escalation Clinical Trial of SB509 in Subjects with Diabetic Neuropathy*. Sponsor: Sangamo BioSciences, Inc.

NIH/OBA Receipt Date: 7-6-04. Not Selected for RAC Public Review: 7-27-04

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0407-657 (Open) Gene Therapy/Phase II/Cancer/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/Electroporation/DMRIE-Cholesterol/Granulocyte-Macrophage Colony Stimulating Factor cDNA/CD40 Ligand cDNA/Intradermal Injection

Dessureault, Sophie, H. Lee Moffitt Cancer Center & Research Institute, University of South Florida, Tampa, Florida; *A Phase II Trial Using a Universal GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) in the Formulation of Autologous Tumor Cell-Based Vaccines for Patients with Malignant Melanoma*.

NIH/OBA Receipt Date: 7-12-04. Not Selected for RAC Public Review: 8-2-04

0407-658 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid DNA/Prostatic Acid Phosphatase (PAP) cDNA/Intradermal Injection

McNeel, Douglas G., University of Wisconsin Medical School, Madison, Wisconsin; *A Phase I Study of a DNA-Based Vaccine Targeting Acid Phosphatase (PAP) in Patients with Stage D0 Prostate Cancer*.

NIH/OBA Receipt Date: 7-23-04. Not Selected for RAC Public Review: 8-12-04

0407-659 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Squamous Cell Carcinoma/Immunotherapy/In Vitro/Allogeneic Fibroblasts/DNA-Liposome Complexes/Tumor Cell DNA Fragments/Intradermal Injection

Cohen, Edward P.; University of Illinois at Chicago; Chicago, Illinois; *Active Immunization of Patients with Stage III/IV Carcinoma of Oral Cavity or Oropharynx with Semi-Allogeneic Non Malignant Human Fibroblasts Transfected with DNA from Autologous Tumor (Phase I Study)*.

NIH/OBA Receipt Date: 7-26-04. Not Selected for RAC Public Review: 8-16-04

0407-660 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Fusion Protein of MUC-1 and CD40 Ligand (CD40L) cDNA/Subcutaneous Injection

Deisseroth, Albert; Sidney Kimmel Cancer Center; San Diego, California; *A Single Arm Open-Label Phase I Study of an Injectable Replication-Incompetent Adenoviral Vector Vaccine with Protein Boost used to Produce an Immune Response for MUC-1 Positive Epithelial Cancer Cells in Prostate Cancer Patients*.

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

0407-661 (Open) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1/VP16 cDNA/Intramuscular Injection

Olin, Jeffrey W.; Mount Sinai School of Medicine; New York, New York; Mendelsohn, Farrel; Cardiology, PC, Baptist Medical Center; Birmingham, Alabama; Chronos, Nicolas; American Cardiology Research Institute; Atlanta, Georgia; Powell, Richard; Dartmouth-Hitchcock Medical Center; Lebanon, New Hampshire; Henry, Timothy; Minneapolis Heart Institute Foundation; Minneapolis, Minnesota; Lard, John; Washington Hospital Center; Washington, DC; Comerota, Anthony; Jobst Vascular Center; Toledo, Ohio; Moen, Elaine; The Care Group at the Heart Center, Indianapolis, Indiana; Wittgen, Catherine; St. Louis University Medical Center; St. Louis, Missouri; Schainfeld, Robert; Caritas St. Elizabeth's Medical Center; Boston, Massachusetts; Dalman, Ronald; VA Palo Alto Health Care System; Palo Alto, California; Kandzari, David; Duke University Medical Center, Durham, North Carolina; Cooke, John; Stanford University School of Medicine; Stanford, California; Olin, Jeffery, Mt. Sinai Medical Center; New York, New York; Sheehan, Peter, New York University School of Medicine; New York, New York; Neal, Ryan; Baylor College of Medicine; Houston, Texas; Illig, Karl; Rochester Medical Center; Rochester, New York; Bartholomew, John; Cleveland Clinic Foundation; Cleveland, Ohio; Miller, Julie; The Johns Hopkins Hospital; Baltimore, Maryland; Saucedo, Jorge; University of Oklahoma Health Science Center; Oklahoma City, Oklahoma; Jaff, Michael; Massachusetts General Hospital; Boston, Massachusetts; Goldman, Corey; Oschner Clinic Foundation Hospital; New Orleans, Louisiana; Moneta, Gregory; Oregon Health & Science University; Portland, Oregon; Bashir, Riyaz; Medical University of Ohio; Toledo, Ohio; Snell, R. Jeffrey; Schaer, Gary; Rush University Medical Center; Chicago, Illinois; Lee, Jason T.; VA Palo Alto Healthcare Systems; Palo Alto, California; Cardenas, Gustavo A.; Palm Beach Heart Research Institute; Atlantis, Florida; Dawson, David Lee; University of California Davis Medical Center; Sacramento, California; Mitchell, Robert; Duke University Medical Center; Durham, North Carolina; Marston, William A.; The University of North Carolina at Chapel Hill; Chapel Hill, North Carolina; Martinez, Jeffrey M.; San Antonio, Texas; Yonehiro, Layne R.; Baptist Hospital, Inc.; Pensacola, Florida; Schairer, John R.; Henry Ford Hospital; Detroit, Michigan; Bandyk, Dennis; University of South Florida; Tampa, Florida; Coyler, William; University of Toledo, Toledo, Ohio; Farber, Alik; Boston Medical; Boston Massachusetts; Zhou, Wei; VA Palo Alto Heath Care System; Palo Alto, California; and Nambi, Vijay; Baylor College of Medicine; Houston, Texas; *A Phase 2, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, Multicenter, Dose-Selection Study of Ad2/Hypoxia Inducible Factor (HIF)-1 α/VP16 in Patients with Intermittent Claudication*. Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0407-662 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag cDNA/Interleukine-15 cDNA/Intramuscular Injections

Baden, Lindsey Robert and Dolin, Raphael; Harvard Medical School and Brigham and Women's Hospital; Boston, Massachusetts; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of HIV-1 Gag DNA alone or with IL-15 DNA, Boosted with Homologous Vaccine, HIV CTL Multi-Epitope (MEP) Vaccine, or Gag + IL-12 Plasmid Vaccine in Healthy, HIV-1 Uninfected Adult Participants*.

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

0407-663 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag cDNA/Interleukine-12 cDNA/Intramuscular Injections

Wright, Peter Farnum and Spearman, Paul W.; Vanderbilt University School of Medicine; Nashville, Tennessee; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of an HIV-1 Gag DNA Vaccine with or without IL-12 DNA Adjuvant, Boosted with Homologous Plasmids or with HIV CTL Multiepitope Vaccine / RC529-SE Plus GM-CSF, in Healthy, HIV-1 Uninfected Adult Participants*.

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

0407-664 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag cDNA/Interleukine-12 cDNA/Intramuscular Injections

Wright, Peter Farnum and Spearman, Paul W.; Vanderbilt University School of Medicine; Nashville, Tennessee; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of HIV-1 Gag DNA Vaccine and IL-12 DNA Adjuvant or HIV CTL Multiepitope Peptide Vaccine / RC529-SE with or without GM-CSF Given to Participants Who have Received the CTL MEP / RC529-SE Vaccine with or without GM-CSF as a Priming Regimen.*

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

0407-665 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Herpes Simplex Virus Type-1/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intratumoral Injection

Nemunaitis, John; Mary Crowley Medical Research Center; Dallas, Texas; Amatruda, Thomas T.; Hubert H. Humphrey Cancer Center; Robbinsdale, Minnesota; Anderson, Clay; University of Missouri Health Care; Columbia, Missouri; Kaufman, Howard; Columbia University; New York, New York; Whitman, Eric; Mountainside Hospital; Montclair, New Jersey; Glaspy, John A.; UCLA Medical Center; Los Angeles, California; Gonzalez, Rene; University of Colorado Cancer Center; Aurora, Colorado; and Reid, Tony; University of California, San Diego; San Diego, California; *Phase II Study of the Efficacy, Safety and Immunogenicity of ONCOVEX$^{GM\text{-}CSF}$ in Patients with Inoperable Malignant Melanoma*. Sponsor: BioVex, Limited

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

0407-666 (Open) Gene Therapy/Phase I-II/Cancer/Colorectal Carcinoma with Liver Metastasis/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interferon-beta/Intravenous Administration

Reid, Tony; University of California, San Diego; San Diego, California; and Nemunaitis, John; Mary Crowley Medical Research Center; Dallas, Texas; *A Phase I/II Study of Interferon-beta Gene Transfer (Ad.hIFN-β) in the Treatment of Refractory Colorectal Carcinoma with Liver Metastasis*. Sponsor: Biogen Idec Inc.

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

0407-667 (Open) Gene Therapy/Phase I-II/Infectious Diseases/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Lentivirus/HIV-1/Antisense env/Intravenous

June, Carl and MacGregor, Rob Roy; University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; *A Phase I/II, Open-Label, Single Center Study to Evaluate the Tolerability, Trafficking and Therapeutic Effects of Repeated Doses of Autologous T Cells Transduced with VRX496 in HIV-Infected Subjects*. Sponsor: VIRxSYS Corporation

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04

0407-668 (Closed) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/p501 cDNA/Intratumoral Injection

Higano, Celestia S.; University of Washington, Seattle Cancer Care Alliance; Seattle, Washington; *Immunotherapy Consisting of Adenovirus Expressing P501 Protein Followed by CPC-P501 Protein Plus AS15 Adjuvant: A Phase I/II Trial of a Therapeutic Vaccine Regimen in Patients with Prostate Cancer who have PSA $\geq$ 0.4 ng/ml after Radical Prostatectomy*. Sponsor: Corixa Corporation

NIH/OBA Receipt Date: 7-27-04. Not Selected for RAC Public Review: 8-16-04
No participants were enrolled: 09-22-05

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0407-669 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Epilepsy/In Vivo/Adeno-Associated Virus/Pseudotype 1 /2/Human Neuropeptide Y (NPY) cDNA/Intracranial Injection

During, Matthew, J.; Weill Medical College of Cornell University, New York, New York; and Fried, Itzhak and Stern, John M.; University of Los Angeles, California School of Medicine; Los Angeles, California; *Hippocampal NPY Gene Transfer in Subjects with Intractable Temporal Lobe Epilepsy.*

NIH/OBA Receipt Date: 7-30-04. Publicly Reviewed at the September 2004 RAC meeting

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0408-670 (Open) Gene Therapy/Cancer/Chronic Myelogenous Leukemia (CML)/Immunotherapy/In Vitro/Allogeneic K562 Cells Cationic Liposome Complexes/GM-CSF cDNA/Intradermal and Subcutaneous Injection

Wadleigh, Martha; Harvard Medical School and Dana-Farber Cancer Institute; Boston, Massachusetts; *GM-K562 Vaccination for CML Patients with Persistent Disease on Imatinib Mesylate*.

NIH/OBA Receipt Date: 8-23-04. Not Selected for RAC Public Review: 9-13-04

0408-671 (Open) Gene Therapy/Peripheral Artery Disease/In Vivo/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Losordo, Douglas W., Caritas St. Elizabeth's Medical Center, Boston, Massachusetts; *A Randomized Double-Blind, Placebo-Controlled, Dose-Escalating Study of Intramuscular Vascular Endothelial Growth Factor 2 Gene Transfer in Patients with Moderate or High-Risk Critical Limb Ischemia*.

NIH/OBA Receipt Date: 8-24-04. Not Selected for RAC Public Review: 9-14-04

0409-672 (Open) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/B7 (CD80), HLA-A1, HLA-A2 cDNAs/Intradermal Injection

Raez, Luis E., University of Miami School of Medicine, Miami, Florida; *A Phase II Clinical Trial of Adjuvant Immunotherapy with an Allogeneic B7.1/HLA-A Transfected Tumor Cell Vaccine in Patients with Stage I-II Non-Small Cell Lung Cancer*.

NIH/OBA Receipt Date: 9-17-04. Not Selected for RAC Public Review: 3-25-05

0409-673 (Closed) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/DNA-Liposome Complexes/Poloxamer 188/Del-1 cDNA/Intramuscular Injection

Grossman, P. Michael, University of Michigan Health Systems, Ann Arbor, Michigan; *A Phase II Multicenter, Randomized, Open-Label, Dose Ranging, Multiple-Dose Trial of VLTS-585 in Subjects with Intermittent Claudication Secondary to Peripheral Disease*. Sponsor: Valentis, Inc.

NIH/OBA Receipt Date: 9-20-04. Not Selected for RAC Public Review: 10-8-04
No participants enrolled: 6-9-06

0410-674 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection

Chiocca, E. Antonio, The Ohio State University Medical Center, Columbus, Ohio, Agular-Cordova, Estuardo, Advantagene, Inc., Waban, Massachusetts; New, Pamela Z.; The Methodist Hospital; Houston, Texas; Portnow, Jana; City of Hope Medical Center; Duarte, California; and Lesniak, Maciej S.; The University of Chicago; Chicago, Illinois; *A Phase Ib Study of ADV-TK + Valacyclovir Gene Therapy in Combination with Standard Radiation Therapy for Malignant Gliomas*. Sponsor: Advantagene, Inc.

NIH/OBA Receipt Date: 10-12-04. Not Selected for RAC Public Review: 11-1-04

0410-675 (Open, RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Urba, Walter J. and Fox, Bernard A., Providence Portland Medical Center, Earle A. Chiles Research Institute, Robert W. Franz Cancer Center, Portland, Oregon; *Development of Effective Immunotherapy for Prostate Cancer Patients: Phase I/II Study of Human GM-CSF Gene Transduced Irradiated Prostate Allogeneic Cancer Cell Vaccines (Allogeneic Prostate GVAX®) in Advanced Prostate Cancer Patients made Lymphopenic by Chemotherapy and Infused with Autologous Peripheral Blood Mononuclear Cells*.

NIH/OBA Receipt Date: 10-12-04. Publicly Reviewed at the December 2004 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0410-676 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid/Electroporation/Interleukin-2 cDNA/Intratumoral Injection

Gonzalez, Rene, University of Colorado Health Sciences Center, Aurora, Colorado; Richards, Jon M.; Lutheran General Hospital; Park Ridge, Illinois; Schwarzenberger, Paul O.; Clinical Oncology Research Associates; Mobile, Alabama; Whitman, Eric D.; Mountainside Hospital; Montclair, New Jersey; and Amatruda, Thomas T.; Hubert H. Humphrey Cancer Center; Robbinsdale, Minnesota; *A Phase I, Multi-Center, Open-Label, Dose-Escalation Trial to Evaluate the Safety of Intratumoral VCL-IM01 Followed by Electroporation in Metastatic Melanoma*. Sponsor: Vical, Inc.

NIH/OBA Receipt Date: 10-18-04. Not Selected for RAC Public Review: 11-5-04

0410-677 (Open; RAC Reviewed with Recommendations) Gene Therapy/Monogenic Diseases/Eye (Retinal) Disease Due to RPE65 Mutations/In Vivo/Adeno-Associated Virus/Serotype 2/RPE65 cDNA/Subretinal Injection

Jacobson, Samuel G., University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania; and Byrne, Barry J.; University of Florida; Gainesville, Florida; *Phase I Trial of Ocular Subretinal Injection of a Recombinant Adeno-Associated Virus (rAAV-RPE65) Gene Vector in Patients with Retinal Disease Due to RPE65 Mutations*.

NIH/OBA Receipt Date: 10-20-04. Publicly Reviewed at the June 2005 RAC meeting

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0410-678 (Open) Gene Therapy/Phase I-II/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3)galactosyltransferase Gene/Intradermal Injection

Talamonti, Mark, Northwestern Medical Faculty Foundation, Inc., Chicago, Illinois; *A Phase I/II Study of an Antitumor Vaccination Using α(1,3)galactosyltransferase Expressing Allogeneic Tumor Cells in Patients with Pancreatic Cancer*. Sponsor: NewLink Genetics

NIH/OBA Receipt Date: 10-20-04. Not Selected for RAC Public Review: 11-9-04

0410-679 (Open, RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Diseases/Duchenne Muscular Dystrophy/In Vivo/Adeno-Associated Virus/Serotype 2/Mini-Dystrophin Gene/Intramuscular Injection

Mendell, Jerry R., Columbus Children's Research Institute, Columbus, Ohio; *Phase I Trial of rAAV2.5-CMV-Mini-Dystrophin Gene Vector in Duchenne Muscular Dystrophy*.

NIH/OBA Receipt Date: 10-20-04. Publicly Reviewed at the December 2004 RAC meeting

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0411-680 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vitro/Autologous Dendritic Cells/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/GM-CSF/Subcutaneous Injection

Morse, Michael; Duke University Medical Center; Durham, North Carolina; *A Phase I Study of Regulatory T Cell Depletion with Denileukin Diftitox Followed by Active Immunotherapy with Autologous Dendritic Cells Infected with CEA-6D Expressing Fowlpox-TRICOM in Patients with Advanced or Metastatic Malignancies Expressing CEA*.

NIH/OBA Receipt Date: 11-16-04. Not Selected for RAC Public Review: 12-6-04

0411-681 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/anti-PSMA-sFv-Zeta T Cell Receptor-CD 28/Intravenous Infusion

Junghans, Richard Paul; Roger Williams Medical Center; Providence, Rhode Island*; Phase Ia/Ib Trial of Anti-PSMA Designer T Cells in Advanced Prostate Cancer after Non-Myeloablative Conditioning*.

NIH/OBA Receipt Date: 11-22-04. Publicly Reviewed at the March 2005 RAC meeting

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0412-682 (Open) Gene Therapy/Phase I/Cardiovascular Diseases/Peripheral Artery Disease/In Vivo/Skeletal Myofibers/Plasmid DNA/VOP32E VEGF-A Transcription Factor cDNA/Intramuscular Injection

Annex, Brian H.; Duke University, Durham VA Medical Center; Durham, North Carolina; *Treatment of Lower Extremity Critical Limb Ischemia via Modulation of VEGF-A Using an Engineered Zinc-finger Transcription Factor (EW-A-401) to Evaluate Safety and the Effects on Progenitor Cells*.

NIH/OBA Receipt Date: 12-3-04. Not Selected for RAC Public Review: 12-27-04

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0412-683 (Closed) Gene Therapy/Cancer/Malignant Glioma/Vector-directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection

Markert, James M.; University of Alabama at Birmingham; Birmingham, Alabama; *A Staged Phase I Study of the Treatment of Malignant Glioma with G207, a Genetically Engineered HSV-1, Followed by Radiation Therapy*. Sponsor: MediGene, Inc.

NIH/OBA Receipt Date: 12-6-04. Not Selected for RAC Public Review: 12-27-04
Closed: 05-25-12

0412-684 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Plasmid DNA/Rat HER2-neu ECD cDNA/Intramuscular Injection

Gilewsik, Theresa; Memorial Sloan-Kettering Cancer Center; New York, New York; *Xenogeneic HER2/neu DNA Immunization for Patients with Metastatic and High Risk Breast Cancer: A Phase I Study to Assess Safety and Immunogenicity*.

NIH/OBA Receipt Date: 12-20-04. Not Selected for RAC Public Review: 1-11-05

0415-685 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Mouse TYPR2 cDNA/Intramuscular Injection

Wolchok, Jedd D.; Memorial Sloan-Kettering Cancer Center; New York, New York; *Injection of AJCC Stage IIB, IIC, III and IV Melanoma Patients with Mouse TYRP2 cDNA: A Phase I Trial to Assess Safety and Immune Response*.

NIH/OBA Receipt Date: 12-22-04. Not Selected for RAC Public Review: 1-13-05

0501-686 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer (NSCLC)/Immunotherapy/In Vitro/AD#100 (Allogeneic Human Lung Adenocarcinoma Cell Line)/Plasmid DNA/Human Heat Shock Protein gp96-Ig cDNA/Intradermal Injections

Raez, Luis E.; University of Miami School of Medicine; Miami, Florida; *Novel Tumor Vaccine gp96-Ig Fusion Protein in Advanced (Stage IIIB), Relapsed or Metastatic (Stage IV) Non-Small Cell Lung Cancer (NSCLC) Patients Who have Failed First Line Chemotherapy*.

NIH/OBA Receipt Date: 1-7-05. Not Selected for RAC Public Review: 4-14-05

0501-687 (Closed) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection

Kandzari, David Edward; Duke University Medical Center; Durham, North Carolina; *A Phase II Open-Label Study to Assess the Angiogenic Response of AMG0001 by Measuring Improvement of Lower Extremity Perfusion in Subjects with Peripheral Arterial Disease as Determined by MRI*. Sponsor: Anges, Inc.

NIH/OBA Receipt Date: 1-14-05. Not Selected for RAC Public Review: 2-16-05
Study never initiated: 09-19-05

0501-688 (Open) Gene Therapy/Phase I/Cancer/Bladder/Immunotherapy/In Vivo/Adenovirus Serotype 5/Interferon alpha-2b cDNA/Intravesical Administration

Dinney, Colin P.; The University of Texas, M.D. Anderson Cancer Center; Houston, Texas; *A Phase I Study of the Safety and Tolerability of Intravesical Administration of SCH 721015 (rAd-IFN) in Patients with Transitional Cell Carcinoma of the Bladder*. Sponsor: Schering Corporation

NIH/OBA Receipt Date: 1-14-05. Not Selected for RAC Public Review: 2-8-05

0501-689 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases Disorders/Parkinson's Disease/In Vivo/Adeno-Associated Virus/Neurturin [NTN] cDNA/Intrastriatal Administration

Marks, William J., Jr.; University of California, San Francisco; San Francisco, California; and Verhagen, Leo; Rush University Medical Center; Chicago, Illinois; *A Phase I, Open-Label Study of CERE-120 (Adeno-Associated Virus Serotype 2 [AAV2]-Neurturin [NTN]) to Assess the Safety and Tolerability of Intrastriatal Delivery to Subjects with Idiopathic Parkinson's Disease*. Sponsor: Ceregene, Inc.

NIH/OBA Receipt Date: 1-18-05. Publicly Reviewed at the March 2005 RAC meeting

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0501-690 (Open) Gene Therapy/Phase I/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Adenovirus/Serotype 5/Human Sodium Iodide Symporter (hNIS) cDNA/Intratumoral Injection

Movsas, Benjamin; Henry Ford Health System; Detroit, Michigan; *Phase I Study of Intraprostatic Administration of a Replication-Competent Adenovirus Expressing the Human Sodium Iodide Symporter for Dynamic Non-Invasive Imaging of Adenoviral Gene Therapy Vectors*.

NIH/OBA Receipt Date: 1-18-05. Not Selected for RAC Public Review: 2-8-05

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0501-691 (Open; RAC Reviewed with Recommendations)) Gene Therapy/Phase I/Cancer/Multiple Myeloma/Vector-Directed Cell Lysis/In Vivo/Measles Virus/Sodium Iodide Symporter (NIS) cDNA/Intravenous Administration

Gertz, Morie A.; and Dispenzieri, Angela; Mayo Clinic; Rochester, Minnesota; *Phase I Trial of Systemic Administration of Edmonston Strain of Measles Virus, Genetically Engineered to Express NIS, with or without Cyclophosphamide, in Patients with Recurrent or Refractory Multiple Myeloma***.**

NIH/OBA Receipt Date: 1-18-05. Publicly Reviewed at the March 2005 RAC meeting

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0501-692 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vivo/DNA Complex with PEG-PEI-Cholesterol/Interleukin-12 cDNA/Intraperitoneal Injection

Alvarez, Ronald D.; University of Alabama at Birmingham; Birmingham, Alabama; *A Phase I, Open Label, Dose Escalation Study of the Safety, Tolerability and Preliminary Efficacy of Intraperitoneal EGEN-001 in Patients with Recurrent Epithelial Ovarian Cancer*. Sponsor: Expression Genetics, Inc.

NIH/OBA Receipt Date: 1-18-05. Not Selected for RAC Public Review: 2-8-05

0501-693 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD 80)/ICAM-1/LFA-3/Subcutaneous Injection

DiPaola, Robert S.; UMDNJ-RWJ Medical School, The Cancer Institute of New Jersey; New Brunswick, New Jersey; Bubley, Glenn; Beth Israel Deaconess Medical Center; Boston, Massachusetts; Roscoe, Morton; Ottumwa Regional Health Center; Ottumwa, Iowa; McVicar, Gary; Northwestern University Medical Center; Chicago, Illinois; and Hahn, Noah M.; Indiana Cancer Center; Indianapolis, Indiana; *A Phase II Study of PROSTVAC-V (Vaccinia)/TRICOM and PROSTVAC-F (Fowlpox)/TRICOM with GM-CSF in Patients with PSA Progression after Local Therapy for Prostate Cancer*. Sponsor: Eastern Cooperative Oncology Group

NIH/OBA Receipt Date: 1-25-05. Not Selected for RAC Public Review: 5-11-05

0501-694 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD 80)/ICAM-1/LFA-3/Subcutaneous and Intraprostatic Injections

Gulley, James L.; National Institutes of Health; Bethesda, Maryland; *Phase I/II Trial of a PSA Based Vaccine and an Anti-CTLA-4 Antibody in Patients with Metastatic Androgen Independent Prostate Cancer*.

NIH/OBA Receipt Date: 1-25-05. Not Selected for RAC Public Review: 2-18-05

0501-695 (Open) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Antisense/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid DNA/Electroporation/TGF-β Antisense cDNA/Subcutaneous Injection

Nemunaitis, John J.; Mary Crowley Medical Research Center; Dallas, Texas; *Exploratory Study Comparing Circulation of Epithelial Cells to Outcome in Patients with Stage IIIB-IV Non-Small Cell Lung Cancer Receiving TGF-β Antisense Gene Modified Allogeneic Tumor Cell Vaccine*.

NIH/OBA Receipt Date: 1-26-05. Not Selected for RAC Public Review: 2-24-05

0502-696 (Open) Gene Therapy/Phase I/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Human Telomerase Reverse Transcriptase (hTERT)/Intradermal Injections

Vieweg, Johannes; Duke University Medical Center; Durham, North Carolina; *A Pilot Study of Active Immunotherapy using Telomerase RNA-Transfected Dendritic Cells Following Depletion of Immature Myeloid Cells using the Differentiation Agent Tretinoin (Vesanoid®) in Subjects with Advanced or Metastatic Renal Cell Carcinoma*.

NIH/OBA Receipt Date: 2-10-05. Not Selected for RAC Public Review: 3-3-05

0502-697 (Open) Gene Therapy/Phase I-II/Cancer/Pancreatic Adenocarcinoma/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection

Fisher, William E.; Baylor College of Medicine; Houston, Texas; *A Phase I/II Study of ADV-TK Gene Therapy in Combination with Chemoradiation for Patients with Pancreatic Adenocarcinoma*.

NIH/OBA Receipt Date: 2-24-05. Not Selected for RAC Public Review: 3-16-05

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0502-698 (Open) Gene Therapy/Phase I/Cancer/Hodgkin's Lymphoma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Ambinder, Richard; Johns Hopkins School of Medicine; Baltimore, Maryland; *A Phase I/II Study of Rituximab, High Dose Cyclophosphamide, and GM-CSF Based Immunotherapy for Relapsed Hodgkin's Lymphoma*.

NIH/OBA Receipt Date: 2-25-05. Not Selected for RAC Public Review: 3-17-05

0502-699 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Glioma/Chemoprotection/In Vitro/Peripheral Blood CD34+ Cells/Retrovirus/O^6-Methylguanine DNA Methyltransferase cDNA/Intravenous Infusion

Wagner, Lars Martin; Cincinnati Children's Hospital Medical Center, University of Cincinnati College of Medicine; Cincinnati, Ohio; and Cavaliere, Robert; The Ohio State University; Columbus, Ohio; *A Pilot Study of Temozolomide and O^6-Benzylguanine for Treatment of High-Grade Glioma, using Autologous Peripheral Blood Stem Cells Genetically Modified for Chemoprotection*.

NIH/OBA Receipt Date: 2-25-05. Publicly Reviewed at the June 2005 RAC meeting

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0503-700 (Closed) Gene Therapy/Phase I/Cancer/Pancreas/Dominant Negative Mutation/In Vivo/Retrovirus/dnG1 Cyclin/Intravenous Infusion

Galanis, Evanthia; Mayo Clinic; Rochester, Minnesota; *MC044C-Phase I Evaluation of Safety of Intravenous Infusion of a Pathotropic Retroviral Vector Bearing a Cytocidal Cyclin G1 Construct (Rexin-G) as Intervention for Locally Advanced and Metastatic Pancreatic Cancer Refractory to Standard Chemotherapy*. Sponsor: Epeius Biotechnologies.

NIH/OBA Receipt Date: 3-2-05. Not Selected for RAC Public Review: 3-24-05
Trial is completed: 08-14-09

0503-701 (Open) Gene Therapy/Phase I/Cancer/Colorectal and Pancreatic Carcinoma/Hepatic Metastasis/Dominant Negative Mutation/In Vivo/Retrovirus/dnG1 Cyclin/Hepatic Arterial Infusion

Bruckner, Howard W.; Lutheran Medical Center; Brooklyn, New York; *Tumor Site Specific Phase I Evaluation of Safety of Hepatic Arterial Infusion of a Matrix-Targeted Retroviral Vector Bearing a Dominant Negative Cyclin G1 Construct as Intervention for Colorectal and Pancreatic Carcinoma Metastatic to Liver*. Sponsor: Epeius Biotechnologies.

NIH/OBA Receipt Date: 3-16-05. Not Selected for RAC Public Review: 4-13-05

0504-702 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Tumor Suppressor Gene/In Vivo/Cationic Liposome Complex/DOTAP-DOPE/Retinoblastoma cDNA/Intravenous Infusion

Siefker-Radtke, Arlene; University of Texas M.D. Anderson Cancer Center; Houston, Texas; *A Phase I Study Open-Label Safety Study of Escalating Doses of SGT-RB94 Alone, and in Combination with Gemcitabine for Injection in Patients with Advanced Solid Tumor Malignancies*. Sponsor: SynerGene Therapeutics, Inc.

NIH/OBA Receipt Date: 4-20-05. Not Selected for RAC Public Review: 5-10-05

0504-703 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Peripheral Artery Disease/In Vitro/Autologous Smooth Muscle and Endothelial Cells/Retrovirus/Angiopoietin-1 (Ang-1) cDNA and Endothelial Growth Factor ($VEGF_{165}$) cDNA/Intra-Arterial Infusion

Grossman, P. Michael; University of Michigan and VA Ann Arbor Health Systems; Ann Arbor, Michigan; and Mohler, Emile; University of Pennsylvania; Philadelphia, Pennsylvania; *A Phase I Safety, Dose Escalating Study of MultiGeneAngio in Patients with Peripheral Arterial Disease*. Sponsor: MultiGene Vascular Systems, Ltd.

NIH/OBA Receipt Date: 4-20-05. Publicly Reviewed at the June 2005 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0504-704 (Open) Gene Therapy/Phase III/Cancer/Melanoma/Immunotherapy/In Vivo/Cationic Liposome Complex/DMRIE-DOPE/Alllovectin-7 (Vical VCL-1005)/HLA-B7/Beta-2 Microglobulin cDNA/Intratumoral Injection

Gonzalez, Rene; University of Colorado Cancer Center; Aurora, Colorado; Richards, Jon; Oncology Specialists, S.C.; Park Ridge, Illinois; Atkins, James, N.; Southeastern Medical Oncology Center; Goldsboro, North Carolina; Patel, Ravindranath; Comprehensive Blood and Cancer Center; Bakersfield, California; Dudek, Arkadiusz; University of Minnesota Medical Center; Minneapolis, Minnesota; Alexandrescu, Doru; Washington Hospital Center; Washington, D.C.; Belt, Robert J.; Kansas City Cancer Center; Kansas City Missouri; Schwarzenberger, Paul O.; Sacred Heart Medical Oncology Group; Mobile, Alabama; Khojasteh, Ali; Capital Comprehensive Cancer Care Clinic; Jefferson City, Missouri; Hutchins, Laura F.; University of Arkansas for Medical Sciences; Little Rock, Arkansas; Whitman, Eric D.; Mountainside Hospital and Morristown Memorial Hospital; Montclair and Morristown, New Jersey; Reintgen Douglas S.; Lakeland Regional Cancer Center; Lakeland, Florida; Agarwala, Sanjiv; St. Luke's Hospital & Health Network; Bethlehem, Pennsylvania; Pennock, Gregory; M.D. Anderson Cancer Center Orlando; Orlando, Florida; Gross, Howard M.; Hematology & Oncology of Dayton, Inc.; Dayton, Ohio; Daniels, Gregory; Moores USCD Cancer Center; LaJolla, California; Andtbacka, Robert; Huntsman Cancer Institute; Salt Lake City, Utah; Richart, John; St. Louis University Cancer Center; St. Louis, Missouri; Nemunaitis, John J.; Mary Crowley Medical Research Center; Dallas, Texas; Barve, Minal; Texas Oncology, P.A., Presbyterian; Dallas, Texas; Noyes, R. Dirk; LDS Hospital; Salt Lake City, Utah; Reich, Elizabeth; Jupiter Hematology & Oncology Associates; Jupiter, Florida; Weber, Robert; Saint Francis Memorial Hospital; San Francisco; Warso, Michael A.; University of Illinois at Chicago Medical Center; Chicago, Illinois; Ozer, Howard; University of Oklahoma Health Sciences Center; Oklahoma City, Oklahoma; Markovic, Svetomir; Mayo Clinic; Rochester, Minnesota; Weresch, Joseph M.; Pacific Medical Centers; Seattle, Washington; Daniels, Gregory; Veteran's Administration Hospital; San Diego, California; Bearden, James D., III; Spartanburg Regional Medical Center; Spartanburg, South Carolina; McMasters, Kelly M.; University of Louisville; Louisville, Kentucky; Sardi, Armando; Mercy Medical Center; Baltimore, Maryland; Alter, Robert S.; Hackensack University Medical Center; Hackensack, New Jersey; Kim, Julian A.; University Hospitals/Case Medical Center; Cleveland, Ohio; Faries, Mark B.; John Wayne Cancer Institute; Santa Monica, California; Kaufman, Howard L.; Mount Sinai Medical Center; New York, New York; Cranmer; Lee D., III; Arizona Cancer Center; Tucson, Arizona; Perry, David J.; Washington Hospital Center; Washington, DC; Leming, Phillip D.; The Christ Hospital Cancer Center; Cincinnati, Ohio; Vetto, John; Oregon Health & Science University; Portland, Oregon; Christensen, Scott; University of California, Davis Medical Center; Sacramento, California; Albertini, Mark R.; University of Wisconsin; Madison, Wisconsin; Verschraegen, Claire; University of New Mexico; Albuquerque, New Mexico; Levin, Robert D.; Midwestern Regional Medical Center; Zion, Illinois; George, Jeffrey; Southern Cancer Center; Mobile, Alabama; Colvin, Gerald; Rhode Island Hospital; Providence, Rhode Island; and Royal, Richard E.; University of Texas MD Anderson Cancer Center; Houston, Texas; *A Phase 3 Clinical Trial to Evaluate the Safety and Efficacy of Treatment with 2 mg Intralesional Allovectin-7® Compared to Dacarbazine (DTIC) or Temozolomide (TMZ) in Subjects with Recurrent Metastatic Melanoma*. Sponsor: Vical Inc.

NIH/OBA Receipt Date: 4-20-05. Not Selected for RAC Public Review: 5-10-05

0504-705 (Open) Gene Therapy/Phase I/Other Diseases-Disorders/Rheumatoid Arthritis/In Vivo/Adeno-Associated Virus/Serotype 2/Tumor Necrosis Factor Receptor-Fc Immunoglobulin (TNFR:Fc) Fusion Gene cDNA/Intra-Articular Administration

Mease, Philip; Seattle Rheumatology Associates, Swedish Hospital; Seattle, Washington; Hobbs, Kathryn; Denver Arthritis Clinic; Denver, Colorado; Wei, Nathan; Arthritis and Osteoporosis Research Center of Maryland; Frederick, Maryland; Kivitz, Alan; Altoona Center for Clinical Research; Altoona, Pennsylvania; Schechtman, Joy; SunValley Arthritis Center; Glendale, Arizona; Fudman, Edward; Austin Rheumatology Research; Austin, Texas; Forstot, Joseph Z.; RASF-Clinical Research Center; Boca Raton, Florida; Willis, Larry G.; Bone and Joint Hospital; Oklahoma City, Oklahoma; Cush, John J.; Presbyterian Hospital of Dallas; Dallas, Texas; Wisenhutter, Craig; Coeur d' Alene Arthritis Clinic; Coeur d' Alene, Idaho; Ruderman, Eric M.; Northwestern University, Chicago, Illinois; Greenwald, Maria; Desert Medical Advances; Palm Desert, California; Graham, Galen; United Medical Associates; Johnson City, New York; Maricic, Michael J.; Catalina Pointe Clinical Research Inc.; Tucson, Arizona; Bookbinder, Stephen A.; Ocala Rheumatology Research Center; Ocala, Florida; Pritchard, Charles H.; Rheumatic Disease Associates, Ltd.; Willow Grove, Pennsylvania; Hou, Anthony; Boling Clinical Trials; Upland, California; Fleischmann, Roy M.; Radiant Research-Dallas TX; Dallas, Texas; Fiske, Darrell; Radiant Research-Stuart FL; Stuart, Florida; Burch, Francis X.; Radiant Research-San Antonio TX, San Antonio, Texas; Trapp, Robert G.; The Arthritis Center; Springfield, Illinois; and Dao, Kathryn H.; Arthritis Consultation Center; Dallas, Texas; *A Phase I Dose Escalation Study of Repeat Intra-Articular Administration of tgAAAC94, a Recombinant Adeno-Associated Vector Containing the TNFR:Fc Fusion Gene, in Inflammatory Arthritis Subjects with and without Concurrent TNF- α Antagonists*. Sponsor: Targeted Genetics Corporation

NIH/OBA Receipt Date: 4-20-05. Not Selected for RAC Public Review: 5-10-05

0504-706 (Open) Gene Therapy/PhaseI-II/Cancer/Immunotherapy/In Vivo/Plasmid in Poly (DL-lactide-coglyxolide) (PLG) Microparticles/Cytochrome P450 Isoenzyme 1B1 (CYP1B1) cDNA/Subcutaneous Injection

Haining, W. Nicholas; Dana-Farber Cancer Institute, Harvard Medical School; Boston, Massachusetts; *A Phase 1/2 Clinical Study of ZYC300 to Determined the Optimum Dosing Administration Conditions for Eliciting an Immune Response in Patients with Metastatic Cancer*.

NIH/OBA Receipt Date: 4-20-05. Not Selected for RAC Public Review: 5-10-05

0504-707 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Polio Virus/Intratumoral Injection

Bigner, Darrell D.; Duke University Medical Center; Durham, North Carolina; *Dose-Finding and Safety Study of an Oncolytic Polio/Rhinovirus Recombinant Against Malignant Glioma*.

NIH/OBA Receipt Date: 4-20-05. Publicly Reviewed at the June 2005 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0504-708 (Closed) Gene Therapy/Phase III/Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal Injection

Drake, Charles G.; Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins; Baltimore, Maryland; Gabrail, Nashat Y.; Gabrail Cancer Center; Canton, Ohio; Richards, Donald A.; Tyler Cancer Center; Tyler, Texas; Nemunaitis, John J.; Mary Crowley Medical Research Center; Purcell, William T.; Deaconess Billings Clinic, Billings, Montana; Macapinlac, Manuel; Jacobi Medical Center; Bronx, New York; Stephenson, Joe; Cancer Center of the Carolinas; Greenville, South Carolina; Curti, Brendan; Providence Portland Medical Center; Portland, Oregon; Berry, William R.; Raleigh Hematology Oncology Associates; Cary, North Carolina; Lechner, James J.; Lacey, Washington; Ervin, Thomas; Maine Center for Cancer Medicine and Blood Disorders; Scarborough, Maine; Siegel, Leonard; Michael and Dianne Bienes Comprehensive Cancer Center; Fort Lauderdale, Florida; Small, Eric; University of California San Francisco Comprehensive Cancer Center; San Francisco, California; Polikoff, Jonathan; Kaiser Permanente Medical Group; San Diego, California; Chen, Chien-Shing; Loma Linda University Cancer Institute; Loma Linda, California; Higano, Celestia; Seattle Cancer Care Alliance; Seattle, Washington; Smith, David C.; University of Michigan Comprehensive Cancer Center; Ann Arbor, Michigan; Pantuck, Allan J.; University of California, Los Angeles; Los Angeles, California; Geils, George F., Jr.; Charleston Hematology Oncology, PA; Charleston, South Carolina; Baron, Ari; Pacific Hematology Oncology Associates; San Francisco, California; Harris, Wayne; Winship Cancer Institute; Atlanta, Georgia; Tannock, Ian; Princess Margaret Hospital; Toronto, Canada; Tan, Winston; Mayo Clinic Jacksonville; Jacksonville, Florida; Shapiro, Henry J. Jupiter Hematology & Oncology Associates; Jupiter, Florida; Dreisbach, Duke; Desert Hematology; Rancho Mirage, California; Elrafei, Tarek; Jacobi Medical Center; Bronx, New York; Harris, Wayne Benard; Emory University/The Atlanta Veterans Affairs; Decatur, Georgia; Gressot, Laurent; Northwest Cancer Center; Houston, Texas; Wong, Bryan Y.; Nevada Cancer Institute Medical Group; Las Vegas, Nevada; Hauke, Ralph; University of Nebraska Medical Center; Omaha, Nebraska; and Laber, Damian; University of Louisville; Louisville, Kentucky; *A Phase 3 Randomized, Open-Label Study of Docetaxel in Combination with CG1940 and CG8711 Versus Docetaxel and Prednisone in Taxane-Naïve Patients with Metastatic Hormone-Refractory Prostate Cancer with Pain*. Sponsor: Cell Genesys, Inc.

NIH/OBA Receipt Date: 4-20-05. Not Selected for RAC Public Review: 5-10-05
Closed: 8-28-08

0505-709 (Open) Gene Therapy/Phase II/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection

Gulley, James L.; National Institutes of Health; Bethesda, Maryland; *A Randomized Pilot Phase II Study of Docetaxel alone or in Combination with PANVAC^{TM}-V (Vaccinia) and PANVAC^{TM}-F (Fowlpox) in Patients with Metastatic Breast Cancer.*

NIH/OBA Receipt Date: 5-5-05. Not Selected for RAC Public Review: 5-25-05

0505-710 (Open) Gene Therapy/Phase I-II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogenic Tumor Cells/Lethally Irradiated/Cationic Liposome Complex/B7 (CD80), HLA-A1, HLA-A2 cDNAs/Intradermal Injection

Raez, Luis E.; University of Miami School of Medicine; Miami, Florida; *Phase I/II Clinical Trial of Immunotherapy with an Allogeneic B7.1/HLA-A1 Transfected Tumor Cell Vaccine in Patients with States IIIB/IV Non-Small Cell Lung Cancer That Have Completed First Line Chemotherapy*.

NIH/OBA Receipt Date: 5-6-05. Not Selected for RAC Public Review: 5-26-05

0505-711 (Open) Gene Therapy/Phase I /Cancer/Melanoma/Clear Cell Sarcoma/ Translocation Associated Renal Cell Carcinoma/Alveolar Soft Part Sarcoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous and Intradermal Injection

Hodi, F. Stephen; Dana-Farber Cancer Institute; Boston, Massachusetts; *A Phase I Trial of Vaccination with Autologous, Lethally Irradiated Tumor Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Granulocyte-Macrophage Colony Stimulating Factor in Pediatric and Adult Patients: GVAX for Advanced Clear Cell Sarcoma, Translocation Associated Renal Cell Carcinoma, Alveolar Soft Part Sarcoma and for Children with Stage IV Melanoma*.

NIH/OBA Receipt Date: 5-18-05. Not Selected for RAC Public Review: 6-8-05

0505-712 (Open) Phase I/Cancer/Hematologic Malignancies/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Human Telomerase Reverse Transcriptase (hTERT)/Intradermal Injections

Rizzieri, David; Dahm, Philipp; and Misra, Debashish; Duke University Medical Center; Durham, North Carolina; *Active Immunotherapy with Mature, Human Telomerase Reverse Transcriptase RNA-Transfected, Autologous Dendritic Cells with or without the IL-2 Diphtheria Toxin Conjugate Denileukin Difitox (ONTAK®) in Subjects with Hematologic Malignancies*.

NIH/OBA Receipt Date: 5-19-05. Not Selected for RAC Public Review: 6-9-05

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0506-713 (Open) Gene Therapy/Phase I/Cancer/Lymphoma/In Vitro/Vector-Directed Tumor Lysis/Vaccinia Virus/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) and Humanized *Escherichia coli* β Galactosidase cDNAs/Intravenous Injection

Negrin, Robert S.; Stanford University School of Medicine; Stanford, California; *A Phase I Dose-Escalation Trial of JX-594 (GM-CSF Recombinant Vaccinia Virus) using Cytokine-Induced Killer (CIK) Cells as a Carrier Vehicle, Administered by Intravenous Injection in Patients with Refractory Lymphoma*.

NIH/OBA Receipt Date: 6-14-05. Not Selected for RAC Public Review: 7-14-05

0506-714 (Open) Gene Therapy/Phase I/Cardiovascular Diseases/Congestive Heart Failure (CHF)/In Vivo/Plasmid DNA/Vascular Endothelial Growth Factor cDNA/Intramyocardial Injection

Losordo, Douglas W.; Caritas St. Elizabeth's Medical Center; Boston, Massachusetts; *phVEGF$_{165}$ Gene Transfer to Promote Angiogenesis in Patients with Ischemic Heart Failure*.

NIH/OBA Receipt Date: 6-14-05. Not Selected for RAC Public Review: 7-5-05

0506-715 (Closed) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection

Powell, Richard J.; Dartmouth Medical School; Lebanon, New Hampshire; Moen, Elaine K.; The Care Group; Indianapolis, Indiana; Henry, Tim; Minneapolis Heart Institute Foundation; Minneapolis, Minnesota; Mendelsohn, Farrell; Cardiology, P.C.; Birmingham, Alabama; Clair, Daniel; The Cleveland Clinic Foundation; Cleveland, Ohio; Yonehiro, Layne; Baptist Clinical Research; Pensacola, Florida; Davies, Mark G.; University of Rochester Medical Center; Rochester, New York; Daniel, George; Florida Cardiovascular Research; Atlantis, Florida; Farber, Alik, Boston Medical Center; Boston, Massachusetts; Darling, Ralph Clement, III; The Institute for Vascular Health and Disease; Albany, New York; and Comerota, Anthony James; Jobst Vascular Center; Toledo, Ohio; *A Phase II Double-Blind, Randomized, Placebo-Controlled Study to Assess the Safety and Efficacy of AMG0001 to Improve Perfusion in Critical Leg Ischemia in Subjects Who Have Peripheral Ischemic Ulcers*. Sponsor: AnGes, Inc.

NIH/OBA Receipt Date: 6-23-05. Not Selected for RAC Public Review: 7-14-05
Closed to enrollment: 8-16-07

0506-716 (Open) Gene Therapy/Phase I/Cancers Expressing HER-2 and/or CEA/Immunotherapy/In Vitro/Plasmid DNA/CEA cDNA/HER2 cDNA/DNA Electroporation/Intramuscular Injection

Chiappori, Alberto A.; H. Lee Moffitt Cancer Center and Research Institute, University of South Florida; Tampa, Florida; *A Phase I Study to Evaluate the Safety/Tolerability and Immunogenicity of V-930 in Patients with Cancers Expressing HER-2 and/or CEA*. Sponsor: Merck & Co., Inc.

NIH/OBA Receipt Date: 6-30-05. Not Selected for RAC Public Review: 7-27-05

0507-717 (Open) Gene Therapy/Phase I/Cancer Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous Leukemia Cells/Adenovirus/Serotype 5/Interleukin-2 cDNA/CD40 Ligand cDNA/Subcutaneous Injection

Carrum, George; Baylor College of Medicine; Houston, Texas; *Treatment of Chronic Lymphocytic B-Leukemia (B-CLL) with Human IL-2 Gene Modified and Human CD40 Ligand-Expressing Autologous Tumor Cells after Depletion of Regulatory T Cells*.

NIH/OBA Receipt Date: 7-14-05. Not Selected for RAC Public Review: 8-3-05

0507-718 (Open) Gene Therapy/Phase II/Cancer/Renal Cell Cancer/Immunotherapy/In Vitro/Vaccinia Virus/5T4 cDNA/Intramuscular Injection

Amato, Robert J.; The Methodist Hospital, Houston, Texas; *Safety, Immunology and Biological Activity Evaluation of TroVax® in Treatment of Patients with Locally Advanced or Metastatic Renal Carcinoma*. Sponsor: Oxford BioMedica plc.

NIH/OBA Receipt Date: 7-18-05. Not Selected for RAC Public Review: 8-8-05

0507-719 (Open) Gene Therapy/Cardiovascular Disease/Peripheral Artery Disease/Buerger's Disease/In Vivo/Endothelial Cells/Plasmid DNA/Vascular Endothelial Growth Factor (VEGF) cDNA/Intramuscular Injection

Annex, Brian H.; Duke University School of Medicine, Durham VA Medical Center; Durham, North Carolina; *A Randomized, Multi-Center, Placebo-Controlled, Double Blind (with Optional 2nd Treatment or Cross-Over to Opposite Treatment Arm) Study of Intramuscular Gene Therapy, with pVGI.1 (VEGF2) [Plasmid DNA Coding for Vascular Endothelial Growth Factor 2] in Patients with Thromboangiitis Obliterans (Buerger's Disease)*. Sponsor: Corautus Genetics Inc.

NIH/OBA Receipt Date: 7-21-05. Not Selected for RAC Public Review: 8-10-05

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0507-720 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/Retrovirus/Antisense/Antisense TAR/Antisense *tat/rev*/In Vitro/CD34+ Cells/Intravenous

Cowan, Morton J.; University of California, San Francisco; San Francisco, California; *Evaluation of the Safety and Effects of Ex-Vivo Modification and Re-Infusion of CD34+ Cells by an Antisense Construct Against HIV-1 in a Retrovirus Vector*. Sponsor: Enzo Therapeutics, Inc.

NIH/OBA Receipt Date: 7-22-05. Not Selected for RAC Public Review: 8-12-05

0507-721 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Brentjens, Renier; Memorial Sloan-Kettering Cancer Center; New York, New York; *A Phase I Trial for the Treatment of Purine Analog-Refractory Chronic Lymphocytic Leukemia using Autologous T Cells Genetically Targeted to the B Cell Specific Antigen CD19*.

NIH/OBA Receipt Date: 7-26-05. Not Selected for RAC Public Review: 8-15-05

0507-722 (Open) Gene Therapy/Phase I/Cancer/Primary or Secondary Liver Cancer/In Vivo/Herpes Simplex Virus Type 1/Vector-Directed Tumor Cell Lysis/Rat Prodrug Enzyme CYP2B1 cDNA/Hepatic Artery Injection

Tanabe, Kenneth K.; Massachusetts General Hospital; Boston, Massachusetts; *Phase I Clinical Trial of rRp450 for Subjects with Liver Tumors*.

NIH/OBA Receipt Date: 7-27-05. Not Selected for RAC Public Review: 8-15-05

0507-723 (Open) Gene Therapy/Phase IIb/Other Diseases-Disorders/Autoimmune Disease/Multiple Sclerosis (MS)/Immunotherapy/In Vivo/Plasmid DNA/Myelin Basic Protein (hMBP) cDNA/Intramuscular Injection

Vollmer, Timothy L.; Barrow Neurological Institute, St. Joseph's Hospital and Medical Center; Phoenix, Arizona; *BHT-3009 Immunotherapy in Relapsing Remitting Multiple Sclerosis*. Sponsor: Bayhill Therapeutics, Inc.

NIH/OBA Receipt Date: 7-27-05. Not Selected for RAC Public Review: 8-24-05

0507-724 (Open) Gene Therapy/Phase I/Cancer/EBV-Positive Lymphoma/In Vitro/LMP2A-Specific Cytotoxic T Cells/Autologous Dendritic Cells/Adenovirus/LMP2A cDNA/Retrovirus/Dominant Negative TGFβ Receptor II (DNRII) cDNA/Intravenous Administration

Bollard, Catherine; Heslop, Helen; and Rooney, Cliona; Baylor College of Medicine; Houston, Texas; *Administration of TGF-beta Resistant LMP2A-Specific Cytotoxic T-Lymphocytes to Patients with Relapsed EBV-Positive Lymphoma*.

NIH/OBA Receipt Date: 7-29-05. Not Selected for RAC Public Review: 8-15-05

0508-725 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/Autologous CD34+ Cells/Lentivirus/RNAi Targeted at HIV tat and rev/RNA Decoy for TAR/Ribozyme Targeted at CCR5 Cytokine Receptor (CCR5RZ)/Intravenous

Krishnan, Amrita; City of Hope National Medical Center; Duarte, California; *A Phase I Pilot Study of Safety and Feasibility of Stem Cell Therapy for AIDS Lymphoma using Stem Cells Treated with a Lentivirus Vector Encoding Multiple Anti-HIV RNAs*.

NIH/OBA Receipt Date: 8-01-05. Publicly Reviewed at the September 2005 RAC meeting

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0508-726 (Open) Gene Therapy/Phase I/Immunotherapy/Cancer/Breast/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous and Intradermal Injection

Dranoff, Glenn; and Overmoyer, Beth; Dana-Farber Cancer Institute; Boston, Massachusetts; *A Phase I Trial of Vaccination with Autologous, Lethally Irradiated Breast Cancer Cells Engineered by Adenoviral Mediated Gene Transfer to Secrete Granulocyte-Macrophage Colony Stimulating Factor in Metastatic Breast Cancer Patients*.

NIH/OBA Receipt Date: 8-11-05. Not Selected for RAC Public Review: 8-31-05

0509-727 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Plasmid/Mammaglobin-A cDNA/Intramuscular Injection

Gillanders, William E.; Washington University School of Medicine; St. Louis, Missouri; *Clinical Translation of a Mammaglobin-A DNA Vaccine for Breast Cancer Prevention and Therapy*.

NIH/OBA Receipt Date: 9-13-05. Publicly Reviewed at the December 2005 RAC meeting

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0510-728 (Open) Gene Therapy/Phase II/Cancer/Pancreatic/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Laheru, Daniel; Johns Hopkins University School of Medicine; Baltimore, Maryland; *A Safety and Efficacy Trial of Lethally Irradiated Allogeneic Pancreatic Tumor Cells Transfected with the GM-CSF Gene in Combination with Erbitux (Cetuximab) for the Treatment of Advanced Pancreatic Adenocarcinomas*.

NIH/OBA Receipt Date: 10-03-05. Not Selected for RAC Public Review: 10-25-05

0510-729 (Open) Gene Therapy/Phase I/Cancer/Mesothelioma or Pleural Malignancies/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interfereon-beta/Intrepleural Administration

Sterman, Daniel; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; *A Phase I Clinical Trial of Repeated Dose Intrapleural Adenoviral-Mediated Interferon-β Gene Transfer for Pleural Malignancies*.

NIH/OBA Receipt Date: 10-03-05. Not Selected for RAC Public Review: 10-25-05

0510-730 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Tumor Necrosis Factor cDNA/Intratumoral Injection

Sondak, Vernon; University of South Florida College of Medicine and H. Lee Moffitt Cancer Center and Research Institute; Tampa, Florida; Senzer, Neal Nathan; Mary Crowley Medical Research Center; Dallas, Texas; McDermott, David F.; Beth Israel Deaconess Medical Center, Boston, Massachusetts; Richards, Donald A.; Tyler Cancer Center; Tyler, Texas; and Stephenson, Joe; Cancer Centers of the Carolinas; Greenville, South Carolina; *A Phase II, Open Label, Single Arm, "Proof of Concept" Study of TNFerade Plus Radiation in Patients with Metastatic Melanoma*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 10-06-05. Not Selected for RAC Public Review: 10-27-05

0510-731 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Parotid Salivary Hypofunction/In Vivo/Adenovirus/Serotype 5/Aquaporin-1 (hAQP1) cDNA/Oral Administration

Alevizos, Ilias; National Institutes of Health; Bethesda, Maryland; *Open-Label, Dose-Escalation Study Evaluating the Safety of a Single Administration of an Adenoviral Vector Encoding Human Aquaporin-1 to One Parotid Salivary Gland in Individuals with Irradiation-Induced Parotid Salivary Hypofunction*.

NIH/OBA Receipt Date: 10-17-05. Publicly Reviewed at the December 2005 RAC meeting

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0510-732 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I-II/Cancer/Solid Tumors/Vector-Directed Cell Lysis/In Vivo/Adenovirus Serotype 5/Conditionally Replication Competent Virus/Intratumoral Injection

Nemunaitis, John; Mary Crowley Medical Research Center; Dallas, Texas; *A Phase I/IIA Dose Escalation Trial of Intratumoral Injection with Oncolytic Adenovirus Vector INGN 007 (VRX-007) in Patients with Advanced Solid Tumors*. Sponsor: Introgen Therapeutics, Inc.

NIH/OBA Receipt Date: 10-17-05. Publicly Reviewed at the December 2005 RAC meeting

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0510-733 (Open) Gene Therapy/Phase I-II/Cancer/Hepatocellular Carcinoma/Immunotherapy/In Vivo/Plasmid/Adenovirus/Serotype 5/Alpha fetoprotein (AFP) cDNA/GM-CSF cDNA/Intradermal Injections

Pingpank, James Francis; University of Pittsburgh; Pittsburgh, Pennsylvania; *A Phase I/II Trial Testing Immunization with AFP+ GM-CSF Plasmid Prime and AFP Adenoviral Vector Boost in Patients with Hepatocellular Carcinoma*.

NIH/OBA Receipt Date: 10-17-05. Not Selected for RAC Public Review: 11-04-05

0510-734 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/In Vivo/Adenovirus/Dominant Negative EGF Receptor Mutant cDNA/Intracranial Administration

Broaddus, William C.; and Chung, Theodore D.; Virginia Commonwealth University Medical Center; Richmond, Virginia; *An Open-Label, Phase I, Dose-Escalation Study of D-EGFR-CD533 and Surgery for Patients with Resectable Recurrent High Grade Glioma*.

NIH/OBA Receipt Date: 10-17-05. Publicly Reviewed at the December 2005 RAC meeting

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0510-735 (Open) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/Muscle Cells/Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1 α/VP16 cDNA/Intramuscular Injection

Olin, Jeffrey W.; Mount Sinai School of Medicine; New York, New York; *A Phase 2 Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, Multi-Center Study of Ad2/Hypoxia Inducible Factor (HIF)-1 α/Vp16 Administered by Intramuscular Injection to Patients with No or Poor Option Chronic Critical Limb Ischemia*. Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 10-18-05. Not Selected for RAC Public Review: 11-07-05

0510-736 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Serotype 5/CD40 cDNA/Intradermal Injection

Sonpavde, Guru; Baylor College of Medicine; Houston, Texas; *A Phase I/II, Non-Randomized, Multiple-Dose, Dose-Escalation Study of the Safety, Pharmacokinetics, Pharmacodynamics and Efficacy of Therapeutic Vaccine, BP-GMAX-CD1, Plus Activating Agent, AP1903, in Patients with Castrate Resistant Prostate Cancer*. Sponsor: Bellicum Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 10-18-05. Not Selected for RAC Public Review: 11-07-05

0510-737 (Closed) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Replication Inhibition/In Vitro/Autologous T Lymphocytes/Lentivirus/RNAi Targeted at HIV tat and rev/RNA Decoy for TAR/Ribozyme Targeted at CCR5 Cytokine Receptor (CCR5RZ)/Intravenous

Zaia, John A.; Beckman Research Institute of City of Hope; Duarte, California; *A Pilot Study of Safety and Feasibility of T Cell Immunotherapy Using Lentivirus Vector-Expressed RNAi in Autologous T Cells of HIV-1 Infected Patients Who have Failed Anti-Retroviral Therapy*.

NIH/OBA Receipt Date: 10-19-05. Not Selected for RAC Public Review: 11-07-05
Closed, no subjects enrolled: 06-27-11

0510-738 (Open) Gene Therapy/Phase I/Cancer/Sarcoma or Neuroblastoma/In Vivo/Herpes Simplex Virus Type 1/Vector-Directed Tumor Cell Lysis/No Transgene/Intratumoral Injection

Geller, James; Cincinnati Children's Hospital Medical Center; Cincinnati, Ohio; *A Phase I Dose Escalation Study of Intratumoral Herpes Simplex Virus-1 Mutant HSV1716 in Patients with Refractory Sarcoma or Neuroblastoma*. Sponsor: Crusade Laboratories

NIH/OBA Receipt Date: 10-18-05. Not Selected for RAC Public Review: 11-07-05

0510-739 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Diseases/Cystic Fibrosis/In Vivo/Adeno-Associated Virus/Serotype 6/Human Placental Alkaline Phosphatase (AP or hpAP) cDNA/Nasal Administration

Aitken, Moira L.; University of Washington; Seattle, Washington; *Transduction of the Upper Airway Epithelium in Humans with Cystic Fibrosis by an AAV6 Vector that Encodes Human Placental Alkaline Phosphatase*.

NIH/OBA Receipt Date: 10-19-05. Publicly Reviewed at the December 2005 RAC meeting

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0510-740 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Monogenic Diseases/Retinal Disease Due to RPE65 Mutations/In Vivo/Adeno-Associated Virus/Serotype 2/RPE65 cDNA/Subretinal Injection

Maguire, Albert M.; University of Pennsylvania Health System; Philadelphia, Pennsylvania; and Simonelli, Francesca; University Hospital of the Second University of Naples; Naples, Italy; *A Phase I Safety Study in Subjects with Leber Congenital Amaurosis (LCA) Using Adeno-Associated Viral to Deliver the Gene for Human RPE65 into the Retinal Pigment Epithelium (RPE)*.

NIH/OBA Receipt Date: 10-19-05. Publicly Reviewed at the December 2005 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0510-741 (Open) Gene Therapy/Phase II/Cancer/Pancreas/Immunotherapy/In Vivo/*Saccharomyces cerevisiae*/Mutated Ras Oncoprotein cDNAs/Subcutaneous Injections

Whiting, Samuel; University of Washington and Seattle Cancer Care Alliance, Fred Hutchinson Cancer Research Center; Seattle, Washington; Wollner, Ira Steven; Henry Ford Health System; Detroit, Michigan; Garbo, Lawrence Eli; New York Oncology Hematology, PC; Albany, New York; Stephenson, Joe, Jr.; Oncology and Hematology Associates of South Carolina, LLC; Greenville, South Carolina; Cox, John V.; The Texas Cancer Center Dallas Southwest; Dallas, Texas; Berman, Barry; Cancer Center of Florida; Orlando, Florida; Roberts, Michael; Hematology Oncology Associates; Phoenix, Arizona; Richards, Donald; Tyler Cancer Center; Tyler, Texas; Schlossman, David M.; Missouri Cancer Associates; Columbia, Missouri; McIntyre, Kristi J.; Texas Oncology, PA; Dallas, Texas; Rangineni, Rajagopal; St. Joseph Oncology, Inc.; St. Joseph, Missouri; Spira, Alexander I.; Fairfax-Northern Virginia Hematology-Oncology PC; Fairfax, Virginia; Cohn, Allen L., Rocky Mountain Cancer Center; Denver, Colorado; Flynn, Patrick; Minnesota Oncology Hematology; Minneapolis, Minnesota; Picus, Joel; Washington University in St. Louis; St. Louis, Missouri; Demeure, Michael; University of Arizona; Tucson, Arizona; Jiang, Yixing; Penn State Milton S. Hershey Medical Center; Hershey, Pennsylvania; Jaiyesimi, Ishmael; William Beaumont Hospital, Royal Oak, Michigan; Marshall, John; Georgetown University Medical Center; Washington, D.C.; Garon, Edward; University of California, Los Angeles; Los Angeles, California; Okazaki, Ian; Straub Clinic and Hospital; Honolulu, Hawaii; Ritch, Paul; Medical College of Wisconsin; Milwaukee, Wisconsin; Karpeh, Martin S.; Beth Israel Medical Center; New York, New York; Arnoletti, J. Pablo; University of Alabama at Birmingham; Birmingham, Alabama; O'Neill, Bert; University of North Carolina at Chapel Hill; Chapel Hill, North Carolina; Govindarajan, Rangaswamy; University of Arkansas for Medical Sciences; Little Rock, Arkansas; Rosen, Peter Julian; Tower Cancer Research Foundation; Beverly Hills, Califonia; Reid, Tony; Moores UCSD Cancer Center; LaJolla, Califonia; Muscarella, Peter, II; Ohio State University; Columbus, Ohio; Fisher, William E.; Baylor College of Medicine; Houston, Texas; Leslie, William; Rush University Medical Center; Chicago, Illinois; Zervos, Emmanuel E.; East Carolina University; Greenville, North Carolina; Kudelka, Andrzej P.; Stony Brook University Cancer Center; Stony Brook, New York; Nakeeb, Attila; Indiana University School of Medicine; Indianapolis, Indiana; McCarter, Martin; University of Colorado Health Science; Denver, Colorado; Henderson, Charles Arthur; Piedmont Hospital; Atlanta, Georgia; Shen, Perry; Wake Forest University School of Medicine; Winston-Salem, North Carolina; Imagawa, David K.; University of California, Irvine; Orange, California; Rosemurgy, Alexander S.; University of South Florida; Tampa, Florida; Dragovich, Tomislav; Arizona Cancer Center; Tucson, Arizona; Smith, Lon Shelby; South Texas Hematology, P.A.; San Antonio, Texas; Lee, Peter P. N.; Tower Cancer Research Foundation; Beverly Hills, California; Ross, Sharona B.; University of South Florida; Tampa, Florida; Hamburg, Solomon I.; Tower Hematology Oncology Medical Group; Beverly Hills, California; and Messersimith, Wells; University of Colorado Hospital CRS; Aurora, Colorado; *A Phase 2 Double Blind, Placebo Controlled, Multi-Center Adjuvant Trial of the Efficacy, Immunogenicity, and Safety of GI-4000; an Inactivated Recombinant Saccharomyces cerevisiae Expressing Mutant Ras Protein Combined with a Gemcitabine Regimen Versus a Gemcitabine Regimen with Placebo, in Patients with Post-Resection R0/R1 Pancreatic Cancer with Tumor Sequence Confirmation on Ras Mutations*. Sponsor: GlobeImmune, Inc.

NIH/OBA Receipt Date: 10-19-05. Not Selected for RAC Public Review: 11-07-05

0510-742 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vivo/Plasmid in Poly(DL-Lactide-Coglycolide) (PLG) Microparticles/Cytochrome P450 Isoenzyme 1B1 (CYP1B1) cDNA/Subcutaneous Injection

Haning, W. Nicholas; Dana-Farber Cancer Institute, Harvard Medical School; Boston, Massachusetts; Kurzrock, Razelle; The University of Texas, M.D. Anderson Cancer Center; Houston, Texas; and Nadler, Lee; Dana-Farber Cancer Institute; Boston, Massachusetts; *A Phase I Open-Label Study of the Safety and Feasibility of ZYC300 Administered with Cyclophosphamide Pre-Dosing*. Sponsor: MGI PHARMA Biologics, Inc.

NIH/OBA Receipt Date: 10-19-05. Not Selected for RAC Public Review: 11-07-05

0510-743 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/Human and Mouse gp100 cDNAs/Intramuscular Injection and Particle-Mediated Delivery

Wolchok, Jedd; Memorial Sloan-Kettering Cancer Center; New York, New York; *Injection of AJCC Stage IIB, IIC, III and IV Melanoma Patients with Mouse gp100 DNA: A Phase I Trial to Compare Intramuscular Injection with Particle-Mediated Delivery*.

NIH/OBA Receipt Date: 10-31-05. Not Selected for RAC Public Review: 11-18-05

0511-744 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Flinn, Ian W.; Johns Hopkins School of Medicine; Baltimore, Maryland; *Randomized Trial of Early Versus Late Vaccination with a GM-CSF Secreting Allogeneic Tumor Cell-Based Vaccine, KGEL/GM-CSF/LMP-2, in Patients with High Risk Chronic Lymphocytic Leukemia*.

NIH/OBA Receipt Date: 11-9-05. Not Selected for RAC Public Review: 12-1-05

0511-745 (Open) Gene Therapy/Phase II/Other Disorders/Macular Degeneration/In Vitro/Plasmid/Encapsulated Cell-Based Drug Delivery Device/Human Ciliary Neurotrophic Growth Factor (CNTF) cDNA/Intraocular Implantation (Via Sclerotomy)

Sieving, Paul A.; National Institutes of Health; Bethesda, Maryland; Birch, David G.; Retina Foundation of the Southwest; Dallas, Texas; Brown, David M.; The Methodist Hospital Research Institute; Houston, Texas; Halperin, Lawrence S.; Retina Group of Florida; Fort Lauderdale, Florida; Williams, George A.; Royal Oak, Michigan; Heier, Jeffrey S.; Boston, Massachusetts; Zhang, Kang; University of Utah; Salt Lake City, Utah; *A Phase II Study of Implants of Encapsulated Human NTC-201 Cells Releasing Ciliary Neurotrophic Factor (CNTF), in Participants with Visual Acuity Impairment Associated with Atrophic Macular Degeneration*.

NIH/OBA Receipt Date: 11-14-05. Not Selected for RAC Public Review: 12-5-05

0511-746 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid/Electroporation/Interleukin-2 cDNA/Intratumoral Injection

Richards, Jon M.; Lutheran General Hospital; Park Ridge, Illinois; *A Multi-Center, Open-Label, Extension Trial for the Intratumoral Injection of VCL-IM01 Followed by Electroporation in Metastatic Melanoma*. Sponsor: Vical, Inc.

NIH/OBA Receipt Date: 11-25-05. Not Selected for RAC Public Review: 12-16-05

0512-747 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Herpes Simplex Virus Type-1/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Intratumoral Injection

Senzer, Neil Nathan; Mary Crowley Medical Research Center; Dallas, Texas; Richards, Donald A.; Tyler Medical Center; Tyler, Texas; Binmoeller, Kenneth F.; California Pacific Medical Center; San Francisco, California; Chang, Kenneth; University of California, Irvine; Orange, California; *Targeted Delivery of OncoVEX^{GM-CSF} by Endoscopic Ultrasound (EUS)-Guided Fine Needle Injection (FNI) in Patients with Irresectable Pancreatic Cancer: A Pilot Experiment on Safety and Proof of Concept*. Sponsor: BioVex, Ltd.

NIH/OBA Receipt Date: 12-1-05. Not Selected for RAC Public Review: 12-27-05

0512-748 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma Cytomegalovirus (CMV) Infection/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/CMV pp65-LAMP mRNA/Intradermal Injections

Sampson, John H.; Duke University Medical Center; Durham, North Carolina; *Anti-Tumor Immunotherapy Targeted Against Cytomegalovirus in Patients with Newly Diagnosed Glioblastoma Multiforme during Recovery from Therapeutic Temozolomide-Induced Lymphopenia*.

NIH/OBA Receipt Date: 12-12-05. Not Selected for RAC Public Review: 1-3-06

0512-749 (Open) Gene Therapy/Phase I/Cancer/Head and Neck Cancer/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Tumor Necrosis Factor (TNF) cDNA/Intratumoral Injection

Vokes, Everett; and Seiwert, Tanguy; University of Chicago Medical Center; Chicago, Illinois; *A Phase I/II Safety, Tolerability and 'Proof of Concept' Study of Radiotherapy and Intratumoral Injections of TNFerade^{TM} (AD_{GV}EGR.TNF.11D) for Elderly or Frail Patients with Head and Neck Cancer*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 12-21-05. Not Selected for RAC Public Review: 4-27-06

0512-750 (Closed) Gene Therapy/Phase I/Cancer/Head and Neck Cancer/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Tumor Necrosis Factor (TNF) cDNA/Intratumoral Injection

Vokes, Everett; and Seiwert, Tanguy; University of Chicago Medical Center; Chicago, Illinois; *A Phase I/II Safety, Tolerability, and 'Proof of Concept' Study of TNFerade^{TM} in Combination with Concomitant Radiotherapy, Fluorouracil, and Hydroxyurea (TNF-FHX) for Patients with Unresectable Recurrent Head and Neck Cancer*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 12-21-05. Not Selected for RAC Public Review: 4-27-06
Closed to new enrollment: 02-23-10

0512-751 (Open) Gene Therapy/Phase III/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3Subcutaneous Injection

DiPaola, Robert; The Cancer Institute of New Jersey; New Brunswick, New Jersey; ***PARADIGM****: A Phase III Study of a* ***P****SA Vaccine in* ***A****ndrogen Ablation* ***R****efractory Prostate Cancer with* ***A****bsence of Metastatic* ***Di****sease and* ***GM****-CSF*. Sponsor: Eastern Cooperative Oncology Group (ECOG)

NIH/OBA Receipt Date: 12-23-05. Not Selected for RAC Public Review: 1-23-06

0512-752 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I/Cancer/B-Cell Lymphoma/Immunotherapy/In Vivo/Plasmid DNA/Mouse CD20 cDNA/Intramuscular Injection (Using Biojector ® 2000 Injection Device)

Palomba, M. Lia; Memorial Sloan-Kettering Cancer Center; New York, New York; *Phase I Trial to Assess Safety and Immunogenicity of Xenogeneic CD20 DNA Vaccination in Patients with B-Cell Lymphoma*.

NIH/OBA Receipt Date: 12-27-05. Publicly Reviewed at the March 2006 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0601-753 (Closed) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic K562 Cell Line/Plasmids/HLA-A*0201, DC80, and CD83 cDNAs/Intravenous Infusion

Butler, Marcus Otho; Dana-Farber Cancer Institute; Boston, Massachusetts; *A Pilot Study of the Adoptive Transfer of MART1/Melan-A CTL for Malignant Melanoma*.

NIH/OBA Receipt Date: 1-17-06. Not Selected for RAC Public Review: 2-7-06
Closed: 01/27/2012

0601-754 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/Yeast Cytosine Deaminase cDNA/5-Fluorocytosine/Herpes Simplex Thymidine Kinase cDNA/Valganciclovir/Adenovirus Death Protein/Intratumoral Injection

Ajlouni, Munther; Henry Ford Hospital; Detroit, Michigan; *Phase I Study Combining Replication-Competent Adenovirus-Mediated Suicide Gene Therapy with Neoadjuvant Chemoradiotherapy in the Treatment of Potentially Resectable Pancreatic Adenocarcinoma*.

NIH/OBA Receipt Date: 1-17-06. Not Selected for RAC Public Review: 2-7-06

0601-755 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Vector-Directed Cell Lysis/Replication-Competent Virus/In Vivo/Adenovirus/hTERT Promoter/Intratumoral Injection

Nemunaitis, John; Mary Crowley Medical Research Center; Dallas, Texas; and Burke, James; Billings Clinic; Billings, Montana; *A Phase I Injection Study of Intratumoral Injection with Telomerase-Specific Replication-Competent Oncolytic Adenovirus, Telomelysin (OBP-301) for Various Solid Tumors*. Sponsor: Oncolys BioPharma, Inc.

NIH/OBA Receipt Date: 1-17-06. Not Selected for RAC Public Review: 2-7-06

0601-756 (Open) Gene Therapy/Phase I/Cardiovascular Diseases/Peripheral Artery Disease/Retrovirus/VEGF cDNA/Fibulin-5 cDNA/In Vitro/Autologous Endothelial Cells/MultiGeneGraft (a Biosynthetic Vascular Graft)

Grossman, P. Michael; University of Michigan School of Medicine; Ann Arbor, Michigan; and Velazquez, Omaida C.; University of Pennsylvania Health System; Philadelphia, Pennsylvania; *A Phase I, Single Dose, Safety Study of MultiGeneGraft in Patients with Peripheral Arterial Disease Undergoing Femoro-Popliteal Bypass Surgery*. Sponsor: MultiGene Vascular Systems, Ltd.

NIH/OBA Receipt Date: 1-17-06. Not Selected for RAC Public Review: 2-7-06

0601-757 (Open) Gene Therapy/Cancer/Chronic Lymphocytic Leukemia (CLL)/Immunotherapy/In Vitro/Autologous B Cells/Adenovirus/Serotype 5/CD154 cDNA/Intravenous Infusion

Wierda, William; University of Texas M.D. Anderson Cancer Center; Houston, Texas; *A Phase I Trial of Autologous CLL B-Cells Transduced to Express Chimeric CD154 (ISF35)*. Sponsor: Memgen, LLC

NIH/OBA Receipt Date: 1-31-06. Not Selected for RAC Public Review: 2-24-06

0602-758 (Open; RAC Reviewed with Recommendations) Gene Therapy/Phase I /Monogenic Disease/Mucopolysaccharidosis Type VII (MPS VII)/In Vitro/Autologous CD34+ Cells/Lentivirus/ β-Glucuronidase cDNA/Intravenous

Sands, Mark S.; Washington University School of Medicine; St. Louis, Missouri; *Lentiviral-Mediated, Hematopoietic-Directed Gene Therapy for MPS VII*.

NIH/OBA Receipt Date: 2-10-06. Publicly Reviewed at the June 2006 RAC meeting

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0602-759 (Open) Gene Therapy/Phase II/Cancer/p53 Overexpression/Immunotherapy/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/Anti-p53 TCR Gene/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Cancer that Overexpresses p53 Using Lymphodepleting Conditioning Followed by Infusion of Anti-p53 TCR-Gene Engineered Lymphocytes*.

NIH/OBA Receipt Date: 2-21-06. Not Selected for RAC Public Review: 3-13-06

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0602-760 (Open) Gene Therapy/Phase I/Cancer/Myelodysplastic Syndrome/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Smith, B. Douglas; Johns Hopkins University School of Medicine; Baltimore, Maryland; *K562/GM-CSF Vaccination in Patients with Myelodysplastic Syndrome*.

NIH/OBA Receipt Date: 2-22-06. Not Selected for RAC Public Review: 3-14-06

0602-761 (Open) Gene Therapy/Phase I/Cancer/Chronic Myelogenous Leukemia/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Smith, B. Douglas; Johns Hopkins University School of Medicine; Baltimore, Maryland; *K562/GM-CSF Vaccination in Combination with Imatinib Mesylate as Booster for Chronic Myeloid Leukemia Patients Previously Vaccinated on Protocol J0345* [NIH OBA 0309-602].

NIH/OBA Receipt Date: 2-27-06. Not Selected for RAC Public Review: 3-20-06

0603-762 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/5T4 cDNA/Intramuscular Injection

Amato, Robert J.; The Methodist Hospital; Houston, Texas; *A Phase II Trial to Assess the Activity of TroVax® Alone vs. TroVax® Plus Granulocyte Macrophage-Colony Stimulating Factor (GM-CSF) in Patients with Progressive Hormone Refractory Prostate Cancer*.

NIH/OBA Receipt Date: 3-1-06. Not Selected for RAC Public Review: 3-22-06

0603-763 (Open) Gene Therapy/Phase I/Cancer/Chronic Myelogenous Leukemia/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Smith, B. Douglas; Johns Hopkins University School of Medicine; Baltimore, Maryland; *A Randomized Phase II Trial of Interferon + GM-CSF Versus K562/GM-CSF Vaccination in CML Patients Achieving a Complete Cytogenetic Response to Imatinib*.

NIH/OBA Receipt Date: 3-2-06. Not Selected for RAC Public Review: 3-22-06

0603-764(Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/ Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD 80)/ICAM-1/LFA-3/GM-CSF/Subcutaneous Injection

Madan, Ravi A.; National Institutes of Health; Bethesda, Maryland; *A Randomized Phase II Trial Combining Vaccine Therapy with PROSTVAC/TRICOM and Flutamide vs. Flutamide Alone in Patients with Androgen Insensitive, Non-Metastatic (D0.5) Prostate Cancer*.

NIH/OBA Receipt Date: 3-3-06. Not Selected for RAC Public Review: 3-23-06

0603-765 (Open) Gene Therapy/Phase II/Cancer/Renal Cell Cancer/Immunotherapy/In Vivo/Vaccinia Virus/5T4 cDNA/Intramuscular Injection

Amato, Robert J.; The Methodist Hospital; Houston, Texas; *A Phase II Trial to Assess the Activity of TroVax® Alone vs. TroVax® Plus Interferon Alfa (IFN-α) in Patients with Advanced or Metastatic Renal Cell Cancer*.

NIH/OBA Receipt Date: 3-14-06. Not Selected for RAC Public Review: 4-3-06

0604-766 (Closed) Gene Therapy/Phase I-II/Cancer/Hematological Malignancies/Pro-Drug/Elimination of Graft-Versus-Host Disease/In Vitro/Allogeneic T Lymphocytes/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Intravenous

Kornblau, Steven M.; The University of Texas M.D. Anderson Cancer Center; Houston, Texas; *A Study: Infusion of Donor Lymphocytes Transduced with the Suicide Gene HSV-TK, After Transplantation of Allogeneic T-Depleted Stem Cells from a Haplo-Identical Donor in Patients with Hematological Malignancies 2005-0084*.

NIH/OBA Receipt Date: 4-11-06. Not Selected for RAC Public Review: 5-2-06
Closed: study never activated, 11/05/09

0604-767 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Retinoblastoma/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Valganciclovir/Intratumoral Injection (Intraocular Tumor)

Hurwitz, Richard L.; Baylor College of Medicine; Houston, Texas; *AdV/RSV-TK Followed by Valganciclovir for Treatment of Patients with Retinoblastoma Complicated by Vitreous Seeds*.

NIH/OBA Receipt Date: 4-20-06. Publicly Reviewed at the June 2006 RAC meeting

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0604-768 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Retinoblastoma/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Valganciclovir/Intratumoral Injection (Intraocular Tumor)

Hurwitz, Richard L.; Baylor College of Medicine; Houston, Texas; *Pediatric Phase I Study of AdV/RSV-TK Followed by Valganciclovir for Treatment of Patients with Retinoblastoma*.

NIH/OBA Receipt Date: 4-20-06. Publicly Reviewed at the June 2006 RAC meeting

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0604-769 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other Disorders/Autoimmune Disorder/Type I Diabetes Mellitus/Immunotherapy/In Vivo/Plasmid/Human Proinsulin Protein (h-proIns)/Intramuscular Injection

Gottlieb, Peter A.; University of Colorado Health Sciences Center; Aurora, Colorado; Kipnes, Mark S.; Diabetes and Glandular Disease Research Associates, PA; San Antonio, Texas; Ratner, Robert; Medstar Research Institute; Washington, DC; Rendell, Marc; Creighton University School of Medicine; Omaha, Nebraska; Greenbaum, Carla, J.; Benaroya Research Institute; Hays, Richard; Wellington, Florida; Norwood, Paul C., Jr.; Valley Research; Fresno, California; Sklyer, Jay S.; Diabetes Research Institute; Miami, Florida; Zemel, Leonard R.; Creekside Endocrine Associates, PC; Denver, Colorado; Colman, Peter G.; Royal Melbourne Hospital; Victoria, Australia; Davis, T. M. E.; Fremantle Hospital and Health Care; Fremantle Western Australia; Gerstman, Murray; Eastern Health; Victoria, Australia; Ovalle, Fernando; University of Alabama at Birmingham; Birmingham, Alabama; Heazelwood, Vernon; Princess Alexandra Hospital; Queensland, Australia; Baker, John Richard; Middlemore Hospital; Auckland, New Zealand; Dunn, Peter; Waikato Hospital; Hamilton, New Zealand; Krebs, Jeremy David; Wellington Hospital; Wellington, New Zealand; and Scott, Russell S.; Christchurch Hospital; Christchurch, New Zealand; *A Phase I Randomized, Placebo Controlled, Open Label Cross-Over Safety and Pharmacodynamic Study of BHT-3021 in Subjects with Recent Onset Type I Diabetes Mellitus*. Sponsor: Bayhill Therapeutics, Inc.

NIH/OBA Receipt Date: 4-21-06. Publicly Reviewed at the June 2006 RAC meeting

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0604-770 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Immunotherapy/In Vivo/Measles Virus/Carcinoembryonic Antigen (CEA) cDNA/Intratumoral Administration

Galanis, Evanthia; Mayo Clinic; Rochester, Minnesota; *Phase I Trial of a Measles Virus Derivative Producing CEA (MV-CEA) in Patients with Recurrent Glioblastoma Multiforme (GBM)*.

NIH/OBA Receipt Date: 4-24-06. Not Selected for RAC Public Review: 5-15-06

0604-771 (Open) Gene Therapy/Phase I/Cancer/EBV-Positive Hodgkin's or Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/LMP-1 and LMP-2-Specific Cytotoxic T Lymphocytes (CTL)/Adenovirus/LMP2A and LMP1 cDNAs/Intravenous Administration

Gottschalk, Stephen; Heslop, Helen; and Rooney, Cliona; Baylor College of Medicine; Houston, Texas; *Administration of LMP-1 and LMP-2-Specific Cytotoxic T-Lymphocytes Following CD45 Antibody to Patients with Relapsed EBV-Positive Hodgkin's or Non-Hodgkin's Lymphoma*.

NIH/OBA Receipt Date: 4-24-06. Not Selected for RAC Public Review: 5-15-06

0604-772 (Open) Gene Therapy/Phase II/Cancer/Pancreas/ Immunotherapy/In Vivo/Adenovirus/Serotype 5/Tumor Necrosis Factor (TNF) cDNA/In Vivo/Intratumoral Injection

Zervos, Emmanuel E.; East Carolina University; Greenville, North Carolina; *A Phase II Study of Direct Tumor Injection of TNFerade™ Followed by KLH-Pulsed Autologous Dendritic Cells in Patients with Unresectable Pancreatic Cancer*.

NIH/OBA Receipt Date: 4-24-06. Not Selected for RAC Public Review: 5-19-06

0604-773 (Open) Gene Therapy/Phase I/Cancer/Prostate/Apoptosis/In Vivo/Adenovirus/Serotype 5/RTVP-1 Gene/Intratumoral Injection

Kadmon, Dov; Baylor College of Medicine; Houston, Texas; *A Phase I Study of In-Situ, Neoadjuvant, Pre-Radical Prostatectomy RTVP-1 Gene Therapy in Patients with Locally Advanced Adenocarcinoma of the Prostate* (Spore # 11-01-30-15).

NIH/OBA Receipt Date: 4-24-06. Not Selected for RAC Public Review: 5-15-06

0604-774 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other Disorders/Overactive Bladder Syndrome and Detrusor Overactivity/In Vivo/Plasmid/Human Maxi-K Channel *hSlo* cDNA/Bladder Administration with a Urethral Catheter

McCullough, Andrew; New York University School of Medicine; and Goldman, Howard Brian; Cleveland Clinic Foundation; Cleveland, Ohio; *A Phase I Multicenter Study Evaluating the Safety and Potential Activity of Three Escalating Doses of hMaxi-K Gene Transfer in Female Participants with Overactive Bladder Syndrome and Detrusor Overactivity: Double Blind, Imbalanced Placebo Controlled Design Within 3 Sequential Active Treatment Groups*. Sponsor: Ion Channel Innovations, LLC

NIH/OBA Receipt Date: 4-25-06. Publicly Reviewed at the June 2006 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0604-775 (Open) Gene Therapy/Phase I/Cancer/Esophageal Carcinoma/Immunotherapy/In Vivo/Adenovirus/Serotype5/CD40 Ligand cDNA/Intratumoral Injection

Korst, Robert J.; Weill Medical College, Cornell University; New York, New York; *Phase I, Initial Safety/Toxicity Study on the Transfer of Adenovirus with the CD40 Ligand Gene ($Ad_{CU}CD40L$) to Patients with Stage III or IV Esophageal Carcinoma*.

NIH/OBA Receipt Date: 4-25-06. Not Selected for RAC Public Review: 5-16-06

0604-776 (Open) Gene Therapy/Phase I/Cancer/Non-Hodgkin's Lymphoma/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Kamble, Rammurti T.; Dotti, Gianpietro; Brenner, Malcolm K.; and Heslop, Helen E.; *Phase I Study of CD19 Chimeric Receptor Expressing T Lymphocytes in B-Cell Non-Hodgkin's Lymphoma and Chronic Lymphocytic Leukemia*.

NIH/OBA Receipt Date: 4-25-06. Not Selected for RAC Public Review: 5-16-06

0605-777 (Open) Gene Therapy/Phase I/Cancer/Renal Cell Cancer/Immunotherapy/In Vitro/Vaccinia Virus/5T4 cDNA/Intramuscular Injection

Amato, Robert J.; The Methodist Hospital; Houston, Texas; *A Phase I Study to Assess the Safety and Immunogenic Efficacy of TroVax® When Added to Sunitnib in Patients with Advanced Metastatic Renal Cancer*. Sponsor Oxford BioMedica, plc

NIH/OBA Receipt Date: 5-5-06. Not Selected for RAC Public Review: 7-5-06

0605-778 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte Macrophage Colony Stimulating Factor/Intradermal Injection

Emens Leisha A.; Johns Hopkins University; Baltimore, Maryland; *A Feasibility Study of Combination Therapy with Trastuzumab, Cyclophosphamide, and an Allogeneic GM-CSF-Secreting Breast Tumor Vaccine for the Treatment of HER-1/neu-Overexpressing Metastatic Breast Cancer*.

NIH/OBA Receipt Date: 5-16-06. Not Selected for RAC Public Review: 6-6-06

0605-779 (Open) Gene Therapy/Phase II/Cancer/Pancreas/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80)/ICAM-1/LFA-3/MUC-1/Subcutaneous Injection

Guha, Chandan; Montefiore Medical Center, Albert Einstein College of Medicine; Bronx, New York; *A Phase II Study Evaluating 5-Flurouracil and Radiation Therapy in Combination with PANVAC™-V and PANVAC™-F in Patients with Locally Advanced Pancreatic Cancer*.

NIH/OBA Receipt Date: 5-16-06. Not Selected for RAC Public Review: 6-7-06

0605-780 (Open) Gene Therapy/Phase II/Cancer/Prostate Cancer/Immunotherapy/In Vivo/Adenovirus Serotype 5/PSA cDNA/ Subcutaneous Injection

Lubaroff, David M.; University of Iowa Health Care; Iowa City, Iowa; *Phase II Study of Adenovirus/PSA Vaccine in Men with Recurrent Prostate Cancer after Local Therapy*.

NIH/OBA Receipt Date: 5-31-06. Not Selected for RAC Public Review: 6-21-06

0605-781 (Open) Gene Therapy/Phase II/Cancer/Prostate Cancer/Immunotherapy/In Vivo/Adenovirus Serotype 5/PSA cDNA/ Subcutaneous Injection

Lubaroff, David M.; University of Iowa Health Care; Iowa City, Iowa; *Phase II Study of Adenovirus/PSA Vaccine in Men with Hormone-Refractory Prostate Cancer*.

NIH/OBA Receipt Date: 5-31-06. Not Selected for RAC Public Review: 6-21-06

0606-782 (Open) Gene Therapy/Phase I/Cancer/Li-Fraumeni /Tumor Suppressor Gene/In Vivo/Adenovirus/Serotype 5/p 53 cDNA/Intratumoral Injection

Nemunaitis, John; Mary Crowley Medical Research Center; Dallas, Texas; *A Pivotal Study of Efficacy and Safety of Intratumoral Advexin® (Ad5CMV-p53) Treatment in Cancer Patients with Li-Fraumeni Syndrome*.

NIH/OBA Receipt Date: 6-23-06. Not Selected for RAC Public Review: 7-25-06

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0606-783 (Closed) Gene Therapy/Phase II/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia Virus/5T4 cDNA/Intramuscular Injection

Salazar, Lupe G.; University of Washington; Seattle, Washington; *A Phase II Study of 5T4-Modified Vaccinia Ankara (MVA) Vaccine (TroVax®) in Patients with Advanced Breast Cancer*. Sponsor: Southwest Oncology Group

NIH/OBA Receipt Date: 6-30-06. Not Selected for RAC Public Review: 7-26-06
Study never initiated: 3-06-08

0607-784 (Closed; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia (CLL)/Small Lymphocytic Lymphoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/CD154 cDNA/Intranodal Injection

Castro, Januario E.; University of California, San Diego; La Jolla, California; *A Phase I, Open Label, Dose-Escalation Pharmacodynamic Study of Intranodal Injection of Adenovirus-CD154 (Ad-ISF35) in Patients with Chronic Lymphocytic Leukemia / Small Lymphocytic Lymphoma*.

NIH/OBA Receipt Date: 7-7-06. Publicly Reviewed at the December 2006 RAC meeting
Closed: 4-05-10

0607-785 (Open) Gene Therapy/Phase I/Cancer/Renal Cell Cancer/Immunotherapy/In Vitro/Vaccinia Virus/5T4 cDNA/Intramuscular Injection

Amato, Robert; The Methodist Hospital; Houston, Texas; Gabrail, Nashat; Canton, Ohio; Arkadiusz, Dudek; University of Minnesota Medical Center; Minneapolis, Minnesota; Khan, Khuda Dad; American Health Network of Indiana; Indianapolis, Indiana; Telwani, Sheela; Henry Ford Health System; Detroit, Michigan; Vuky, Jacqueline; Virginia Mason Medical Center; Seattle, Washington; Minor, David R.; California Kidney Cancer Foundation; San Francisco, California; Perry, David J.; Washington Cancer Institute of Washington Hospital Center; Washington, DC; Ebbinghaus, Scot W., University of Arizona; Tucson, Arizona; Drabkin, Harry; University of Colorado Health Sciences Center; Aurora, Colorado; Forrest, John B.; Urologic Specialists of Oklahoma, Inc.; Tulsa, Oklahoma; Patel, Kamal; Little Rock Hematology Oncology Associates, PA; Little Rock, Arkansas; Henderson, Charles A.; Peachtree Hematology & Oncology Consultants; Atlanta, Georgia; Gelis, George F., Sr.; Charleston Hematology Oncology; Charleston, South Carolina; Ernstoff, Marc; Dartmouth-Hitchcock Medical Center; Lebanon, New Hampshire; Young, Richard W.; Carolina Urologic Research Center; Myrtle Beach, South Carolina; Pierce, Daryl; Cancer Outreach Associates, PC; Abingdon, Virginia; Bodell, Dawn M.; Oregon Urology Institute; Springfield, Oregon; Croot, Christopher C.; Hematology & Oncology Associates at BridgePoint; Tupelo, Mississippi; Kosty, Michael P.; Scripps Clinic; LaJolla, California; Alter, Robert, Hackensack University Medical Center; Hackensack, New Jersey; Kuebler, J. Philip; Riverside Cancer Center; Columbus, Ohio; Millard, Frederick; University of California San Diego; LaJolla, California; Fleming; Mark Tyrone; Virginia Oncology Assoc.; Newport News, Virginia; Keaton, Mark R.; Augusta Oncology Assoc., PC; Augusta, Georgia; Raiker, Anikumar N.; St. Petersburg, Florida; Burke, James M.; Billings Clinic; Billings, Montana; Samuels, Brian L.; Kootenai Cancer Center; Coeur d' Alene, Idaho; Stephenson, Joe; Cancer Centers of the Carolinas; Greenville, South Carolina; Rocha, Rafael L.; Saint Petersburg, Florida; Belldegrun, Arie S.; University of California, Los Angeles; Los Angeles California; Al-Jazayrly, Ghassan; Comprehensive Hematology and Oncology; Los Angeles; Anathakrishnan, Thyagarajan, Gastroenterology and Oncology Associates, P.A.; St. Petersburg, Florida; Amato, Robert J.; The Methodist Hospital; Houston, Texas; and Stephenson, Joe, Jr.; Cancer Centers of the Carolinas; Greenville, South Carolina; *TRIST (TroVax Renal Immunotherapy Survival Trial) an International, Randomized, Double-Blind, Placebo Controlled, Parallel Group Study to Investigate Whether TroVax Added to First-Line Standard of Care Therapy, Prolongs the Survival of Patients with Locally Advanced or Metastatic Clear Cell Renal Adenocarcinoma*. Sponsor: Oxford BioMedica plc

NIH/OBA Receipt Date: 7-10-06. Not Selected for RAC Public Review: 7-28-06

0607-786 (Closed) Gene Therapy/Phase II/Cancer/Primary Cutaneous B-Cell Lymphoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Human Gamma Interferon cDNA/Intratumoral Injection

Duvic, Madeleine; M.D. Anderson Cancer Center; Houston, Texas; Guitart, Joan; Northwestern University; Chicago, Illinois; and Kim, Youn H.; Stanford University Medical Center; Stanford, California; *A Phase II Clinical Trial of Intra-Lesional Administration of TG1042 (Adenovirus-Interferon γ) in Patients with Relapsing Primary Cutaneous B-Cell Lymphoma*. Sponsor: Transgene

NIH/OBA Receipt Date: 7-10-06. Not Selected for RAC Public Review: 8-9-06
Study termination: 12-02-08

0607-787 (Open) Gene Therapy/Phase II/Cancer/Colorectal/Herpes Simplex Virus Type 1/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intratumoral Injection

Reid, Tony; University of California, San Diego; La Jolla, California; *A Phase II Study of the Safety and Efficacy of Oncovex^{GM-CSF} in the Treatment of Hepatic Metastases of Colorectal Cancers*. Sponsor: Biovex Ltd

NIH/OBA Receipt Date: 7-14-06. Not Selected for RAC Public Review: 8-4-06

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0607-788 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Other Diseases Disorders/Parkinson's Disease/In Vivo/Adeno-associated Virus/Neurturin [NTN] cDNA/Intrastriatal Administration

Marks, William; University of California, San Francisco; San Francisco, California; Verhagen, Leo; Rush University Medical Center; Chicago, Illinois; Jankovic, Joseph; Baylor College of Medicine; Houston, Texas; Boulis, Nicholas; Cleveland Clinic; Cleveland, Ohio; Stacy, Mark; Duke University Medical Center; Durham, North Carolina; Watts, Ray L.; University of Alabama at Birmingham; Birmingham, Alabama; Stern, Matthew B.; University of Pennsylvania Health Systems; Philadelphia, Pennsylvania; Tagliati, Michele; Mount Sinai Medical Center; New York, New York; and Nutt, John Gordon; Oregon Health & Science University; Portland, Oregon; *Multicenter, Randomized, Double-Blind, Sham Surgery-Controlled Study of CERE-120 (Adeno-Associated Virus Serotype 2 [AAV2]-Neurturin [NTN]) to Assess the Efficacy and Safety of Bilateral Intraputamenal (IPu) Delivery in Subjects With Idiopathic Parkinson's Disease*. Sponsor: Ceregene, Inc.

NIH/OBA Receipt Date: 7-17-06. Publicly Reviewed at the September 2006 RAC meeting

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0607-789 (Closed) Gene Therapy/Phase I/Cancer/Pancreas/Antisense/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Plasmid Electroporation/Human ß2 Antisense DNA/Intradermal Injection

Nemunaitis, John; Mary Crowley Medical Research Center; Dallas, Texas; *A Single Use Trial of TGF- β2 Antisense Modified Autologous Tumor Cell Vaccine in Pancreatic Cancer*.

NIH/OBA Receipt Date: 7-18-06. Not Selected for RAC Public Review: 8-14-06
Closed, never initiated: 8-29-06

0607-790 (Open) Gene Therapy/Phase I/Cancer/Advanced Solid Malignancies/Immunotherapy/In Vivo/Naked Plasmid/FRAME and PSMA cDNA/Intra-lymphnodal Injection

Weber, Jeffrey; University of Southern California School of Medicine; Los Angeles, California; Vogelzang, Nicholas; Nevada Cancer Institute; Las Vegas, Nevada; Ernstoff, Marc S.; Dartmouth-Hitchcock Medical Center; Lebanon, New Hampshire; Marshall, John; Georgetown University Medical Center; Washington, DC; Cranmer, Lee; University of Arizona; Tucson, Arizona; Goodman, Oscar; Nevada Cancer Institute; Las Vegas, Nevada; *A Phase I Multi-Center Open-Label Clinical Trial of the Immune Response, Safety and Tolerability of DNA Vector pPRA-PSM with Synthetic Peptides E-PRA and E-PSM in Subjects with Advanced Solid Malignancies*.

NIH/OBA Receipt Date: 7-20-06. Not Selected for RAC Public Review: 8-14-06

0607-791 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Lentivirus/Alpha and Beta Chains of T-Cell Receptor (TCR) Specific for MART-1/Intravenous Infusion

Ribas, Antoni and Economou, James S.; University of California, Los Angeles School of Medicine; Los Angeles, California; *Treatment and Biological Imaging of Patients with Locally Advanced or Metastatic Melanoma with Lentiviral Vector MART-1 TCR/HSV1-sr39k (FUW-M1-TCR/sr39k) Engineered Lymphocytes, MART-$1_{26.35}$-Pulsed Dendritic Cells, and Interleukin-2 after a Nonmyeloablative Conditioning Regimen*.

NIH/OBA Receipt Date: 7-21-06. Not Selected for RAC Public Review: 8-11-06

0607-792 (Open) Gene Therapy/Phase II/Other Diseases/Peripheral Artery Disease/In Vivo/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection

Henry, Timothy; Minneapolis Heart Institute Foundation; Minneapolis, Minnesota; *A Phase I, Dose-Escalation, Single Center Study to Assess the Safety and Tolerability of VM202 in Subjects with Critical Limb Ischemia*. Sponsor: ViroMed Co., Ltd.

NIH/OBA Receipt Date: 7-24-06. Not Selected for RAC Public Review: 8-11-06

0607-793 (Open) Gene Therapy/Phase I/Cancer/CD 19+ Leukemia and Lymphoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Lentivirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Porter, David L.; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; and Grupp, Stephen A.; The Children's Hospital of Philadelphia; *Pilot Study of Redirected Autologous T Cells Engineered to Contain Anti-CD19 Attached to TCRζ and 4-1 BB Signaling Domains in Patients with Chemotherapy Resistant or Refractory CD19+ Leukemia and Lymphoma*.

NIH/OBA Receipt Date: 7-24-06. Not Selected for RAC Public Review: 8-14-06

0607-794 (Open) Gene Therapy/Phase I/Cancer/Glioma/Chemoprotection/In Vitro/Peripheral Blood CD34+ Cells/Retrovirus/O^6-Methylguanine DNA Methyltransferase cDNA/Intravenous Infusion

Kiem, Hans Peter; Fred Hutchinson Cancer Research Center and University of Washington; Seattle, Washington; *Dose-Intensive Chemotherapy in Combination with Chemoprotected Autologous Stem Cells for Patients with Malignant Gliomas*.

NIH/OBA Receipt Date: 7-25-06. Not Selected for RAC Public Review: 8-14-06

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0607-795 (Open) Gene Therapy/Phase II/Other Disorders/Retinitis Pigmentosa/In Vitro/Plasmid/Encapsulated Cell-Based Drug Delivery Device/Human Ciliary Neurotrophic Growth Factor (CNTF) cDNA/Intraocular Implantation (via Sclerotomy)

Sieving, Paul A.; National Institutes of Health; Bethesda, Maryland; Weleber, Richard G.; Oregon Health Sciences University; Portland, Oregon; Hopkins, Janet Jill; Retina-Vitreous Associates Medical Center; Beverly Hills, California; Birch, David G.; Retina Foundation of the Southwest; Dallas, Texas; Olsen, Timothy W.; University of Minnesota; Minneapolis, Minnesota; Duncan, Jacque L.; University of California, San Francisco; San Francisco, California; Halperin, Lawrence G.; Retina Group of Florida; Fort Lauderdale; Grover, Sandeep; University of Florida; Jacksonville, Florida; Heckenlively, John R.; Ann Arbor, Michigan; Zhang, Khang; University of Utah; Salt Lake City, Utah; Carr, Ronald Edward; New York, New York; Telander, David; University of California, Davis; Sacramento, California; Iannaccone, Alessandro; University of Tennessee Health Science Eye Institute; Memphis, Tennessee; Heier, Jeffrey S.; Boston, Massachusetts; Haller, Julia A.; The Wilmer Ophthalmological Institute, Johns Hopkins University; Baltimore, Maryland; and Aaberg, Thomas, M., Jr.; Emory University School of Medicine; *A Phase II Study of Encapsulated Human NTC0201 Cell Implants Releasing Ciliary Neurotrophic Factor (CNTF) for Participants with Retinitis Pigmentosa Using Visual Acuity as the Primary Outcome*.

NIH/OBA Receipt Date: 7-25-06. Not Selected for RAC Public Review: 8-14-06

0607-796 (Open) Gene Therapy/Phase II/Other Disorders/Retinitis Pigmentosa/In Vitro/Plasmid/Encapsulated Cell-Based Drug Delivery Device/Human Ciliary Neurotrophic Growth Factor (CNTF) cDNA/Intraocular Implantation (via Sclerotomy)

Sieving, Paul A.; National Institutes of Health; Bethesda, Maryland; Weleber, Richard G.; Oregon Health Sciences University; Portland, Oregon; Hopkins, Janet Jill; Retina-Vitreous Associates Medical Center; Beverly Hills, California; Birch, David G.; Retinal Foundation of the Southwest; Dallas, Texas; Olsen, Timothy W.; University of Minnesota; Minneapolis, Minnesota; Duncan, Jacque L.; University of California, San Francisco; San Francisco, California; Grover, Sandeep; University of Florida Jacksonville Health Science Center; Jacksonville, Florida; Halperin, Lawrence S.; Retina Group of Florida; Fort Lauderdale, Florida; Iannaccone, Alessandro; University of Tennessee Health Science Center; Memphis, Tennessee; Telander, David; University of California, Davis; Sacramento, California; Heckenlively, John R.; University of Michigan; Ann Arbor, Michigan; Zhang, Kang; University of Utah; Salt Lake City, Utah; Heier, Jeffrey S.; Boston, Massachusetts; and Carr, Ronald Edward; New York University School of Medicine; New York, New York; *A Phase II Study of Encapsulated Human NTC0201 Cell Implants Releasing Ciliary Neurotrophic Factor (CNTF) for Participants with Retinitis Pigmentosa Using Visual Field Sensitivity as the Primary Outcome*.

NIH/OBA Receipt Date: 7-25-06. Not Selected for RAC Public Review: 8-14-06

0607-797 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Vaccinia Virus/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) and Humanized *Escherichia coli* β-galactosidase cDNAs/Intratumoral Injection

Reid, Tony; University of California, San Diego; La Jolla, California; *A Phase I Dose-Escalation Trial of JX-594 (Thymidine Kinase-Deleted Vaccinia Virus Plus GM-CSF) Administered by Intravenous Infusion in Patients with Refractory Solid Tumors*. Sponsor: Jennerex Biotherapeutics

NIH/OBA Receipt Date: 7-25-06. Not Selected for RAC Public Review: 8-14-06

0607-798 (Open) Gene Therapy/Phase I-II/Cancer/Acute Myelogenous Leukemia(AML)/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Human Telomerase Reverse Transcriptase (hTERT)/Intradermal Injections

DiPersio, John; Washington University School of Medicine; St. Louis, Missouri; Collins, Robert H., Jr.; University of Texas Southwestern Medical Center at Dallas; Dallas, Texas; Blum, William; The Ohio State University Medical Center; Columbus, Ohio; Khoury, Hanna Jean; Emory University School of Medicine; Atlanta, Georgia; Stiff, Patrick; Loyola University School of Medicine; Maywood, Illinois; and Devetten, Marcel Petrus; University of Nebraska Medical Center; Omaha, Nebraska; *A Phase I/II Study of Active Immunotherapy with GRNVAC1, Autologous Mature Dendritic Cells Transformed with mRNA Encoding Human Telomerase Reverse Transcriptase (hTERT), in Patients with Acute Myelogenous Leukemia (AML) in Complete Clinical Remission*. Sponsor: Geron Corporation

NIH/OBA Receipt Date: 7-25-06. Not Selected for RAC Public Review: 8-14-06

0607-799 (Open) Gene Therapy/Phase II/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Leheru Daniel; Johns Hopkins University School of Medicine; Baltimore, Maryland; *A Safety and Efficacy Trial of Vaccine Boosting with Lethally Irradiated Allogeneic Pancreatic Tumor Cells Transfected with the GM-CSF Gene for the Treatment of Pancreatic Adenocarcinoma*.

NIH/OBA Receipt Date: 7-31-06. Not Selected for RAC Public Review: 8-21-06

0607-800 (Open) Gene Therapy/Phase I/Cancer/B-Chronic Lymphocytic Leukemia (B-CLL)/Immunotherapy/In Vitro/Autologous Leukemia Cells/Adenovirus/Serotype 5/Interleukin-2 cDNA/CD40 Ligand cDNA/Subcutaneous Injection

Brenner, Malcolm; Baylor College of Medicine; Houston, Texas; *Immunization with Autologous CD40 Ligand and IL-2-Expressing Tumor Cells for Treatment of B-Chronic Lymphocytic Leukemia (B-CLL)*.

NIH/OBA Receipt Date: 7-31-06. Not Selected for RAC Public Review: 8-21-06

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0608-801 (Open) Gene Therapy/Phase II/Cancer/Adenocarcinoma of the Lung/Immunotherapy/In Vitro/Allogeneic K562 Cells/Combination with Untransduced Tumor Cells/Plasmid DNA/Electroporation/DMRIE-Cholesterol/Granulocyte-Macrophage Colony Stimulating Factor cDNA/CD40 Ligand cDNA/Intradermal Injection

Chiappori, Alberto; H. Lee Moffitt Cancer Center and Research Institute; Tampa, Florida; *A Phase II Trial Using a GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) in the Formulation of Allogeneic Tumor Cell-Based Vaccines in Combination with ATRA, and Cyclophosphamide for Patients with Stage IV adenocarcinoma of the Lung*.

NIH/OBA Receipt Date: 8-10-06. Not Selected for RAC Public Review: 8-30-06

0608-802 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Peripheral Blood Lymphocytes/Plasmid DNA/Immunoglobulin Heavy (H) Chain Gene/Telomerase Reverse Transcriptase (hTERT) Gene/Intravenous Infusion

Daniels, Gregory A.; University of California, San Diego; La Jolla, California; Faries, Mark; John Wayne Cancer Institute; Santa Monica, California; Margolin, Kim; City of Hope Cancer Center; Duarte, California; Ribas, Antoni; University of California, Los Angeles; Los Angeles, California; and Spitler, Lynn E.; Northern California Melanoma Center; San Francisco, California; *A Phase 2, Open-Label Evaluation of the Safety and Efficacy of CB-10-01, Transgenic Lymphocyte Immunization (TLI) Against Telomerase, as Adjuvant Therapy in Subjects with Stage III Melanoma*. Sponsor: Cosmo Bioscience

NIH/OBA Receipt Date: 8-25-06. Not Selected for RAC Public Review: 9-15-06

0609-803 (Open) Gene Therapy/Phase I/Cancer/Hematologic Malignancies/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Huff, Carol Ann; Johns Hopkins University School of Medicine; Baltimore, Maryland; *Posttransplant Immunotherapy Using Donor Lymphocyte Infusions with Autologous Tumor Vaccines Following HLA-Matched Related Bone Marrow Transplantation for Hematologic Malignancies*.

NIH/OBA Receipt Date: 9-6-06. Not Selected for RAC Public Review: 9-27-06

0609-804 (Open) Gene Therapy/Cancer/Lung/Immunotherapy/In Vitro/Allogeneic Lung Tumor Cell Line/Lethally Irradiated/Plasmid/Interleukine-12 (IL-12) cDNA/Intratumoral Injection of Transduced Cells

Bankert, Richard B.; State University of New York, School of Medicine and Biomedical Sciences at Buffalo; Buffalo, New York; *Clinical Trial of an In Situ Cancer Vaccination Strategy*.

NIH/OBA Receipt Date: 9-29-06. Not Selected for RAC Public Review: 10-20-06

0610-805 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/GM-CSF/Subcutaneous Injection

Gulley, James L.; National Institutes of Health; Bethesda, Maryland; Stein, Mark; The Cancer Institute of New Jersey; New Brunswick, New Jersey; and Posadas, Edwin; The University of Chicago; Chicago, Illinois; *A Randomized Phase 2.5 Study of ^{153}Sm-EDTMP (Quadramet) with or without a PSA/TRICOM Vaccine in Men with Androgen-Insensitive Metastatic Prostate Cancer*.

NIH/OBA Receipt Date: 10-3-06. Not Selected for RAC Public Review: 10-26-06

0610-806 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/Mouse Tyrosinase cDNA/In Vivo Electroporation Using Ichor Medical Systems' Device

Wolchok, Jedd D.; Memorial Sloan-Kettering Cancer Center; New York, New York; *A Phase Ia/Ib Study of the Safety and Immunogenenicity of a Xenogeneic Tyrosinase DNA Vaccine Administered by In Vivo Electroporation in Patients with AJCC Stage IIb-IV Malignant Melanoma*.

NIH/OBA Receipt Date: 10-5-06. Not Selected for RAC Public Review: 10-27-06

0610-807 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Serotype 5/CCL-21 cDNA/Intratumoral Injection

Dubinett, Steven M.; and Lee, Jay M.; University of California, Los Angeles School of Medicine; Los Angeles, California; *A Phase I Trial of Intratumoral Administration of Secondary Lymphoid Chemokine Gene-Modified Autologous Dendritic Cells in Advanced Non-Small Cell Lung Cancer*.

NIH/OBA Receipt Date: 10-6-06. Publicly Reviewed at the December 2006 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0610-808 (Open) Gene Therapy/Phase I-II/Cancer/Prostate Cancer/Pro-Drug/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase Gene/Valacyclovir/Intratumoral Injection

Butler, E. Brian; The Methodist Hospital; Houston, Texas; *Phase I-II Study HSV-TK + Valacyclovir Gene Transfer in Combination with Brachytherapy for Recurrent Prostate Cancer with or without Metastatic Disease*.

NIH/OBA Receipt Date: 10-10-06. Not Selected for RAC Public Review: 12-18-06

0610-809 (Open) Gene Therapy/Other Diseases-Disorders/Heart Failure/In Vivo/Adeno-Associated Virus/Serotype 1/Sarcoplasmic Reticulum Calcium ATPase 2a (SERCA2a) cDNA/Intracoronary Administration

Jessup, Mariell; University of Pennsylvania Health System; Philadelphia, Pennsylvania; Jaski, Brian; Sharpe Memorial Hospital; San Diego, California; Greenberg, Barry H.; University of California, San Diego; San Diego, California; Starling, Randall; The Cleveland Clinic Foundation; Cleveland, Ohio; Pauly, Daniel; University of Florida; Gainesville, Florida; Mancini, Donna; Columbia University Medical Center; New York, New York; Kfoury, A. G.; Intermountain Medical Center; Murray, Utah; Hauptman, Paul J.; Saint Louis University; Saint Louis, Missouri; Archer, Stephen L.; The University of Chicago Medical Center; Chicago, Illinois; Kalman, Jill; Mount Sinai Medical Center; New York, New York; Gupta, Dinesh K.; Tennessee Center for Clinical Trials, Tullahoma, Tennessee; Andrew Kao; Mid America Heart Institute; Kansas City, Missouri; London, Barry; University of Pittsburgh Medical Center; Pittsburgh, Pennsylvania; Thohan, Vinay; Wake Forest School of Medicine; Winston-Salem, North Carolina; Eichhorn, Eric; Medical City Dallas Hospital; Dallas, Texas; Dunlap, Stephanie; University of Cincinnati Medical Center; Cincinnati, Ohio; Johnson, Maryl R.; University of Wisconsin School of Medicine and Public Health; Madison, Wisconsin; Klapholz, Marc; UMDNJ - New Jersey Medical School; Newark, New Jersey; and Dunlap, Mark; MetroHealth MC; Cleveland, Ohio; *A Phase I, Randomized, Double-Blinded, Placebo-Controlled Dose Escalation Trial of Intracoronary Administration of MYDICAR™ (AAV1/SERCA2a) in Subjects with Heart Failure*. Sponsor: Celladon Corporation

NIH/OBA Receipt Date: 10-10-06. Not Selected for RAC Public Review: 10-31-06

0610-810 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Infectious Disease/Hepatitis B/In Vivo/Plasmid/RNAi (shRNA) Targeted at RNA of Hepatitis B Virus (HBV)/Intravenous Injection

Gish, Robert G.; University of California, San Francisco and California Pacific Medical Center; San Francisco, California; Mohanty, Smruti; University of Chicago; Chicago, Illinois; Tomic, Dragan and Dragan, Delic; Clinical Centre of Serbia; Belgrade, Serbia *A Phase I Open-Label, Rising-Dose Study of the Safety and Tolerability of Single Doses of NUC B1000, an RNAi-Based Therapy for Chronic Hepatitis B*. Sponsor: Nucleonics, Inc.

NIH/OBA Receipt Date: 10-10-06. Publicly Reviewed at the December 2006 RAC meeting

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0610-811 (Duplicate Submission of 0502-697) Gene Therapy/Phase I-II/Cancer/Pancreatic Adenocarcinoma/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection

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0610-812 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Vector-Directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection

Curry, William T.; Massachusetts General Hospital; Boston, Massachusetts; *A Phase I Study of Combining Treatment with Temozolomide with Intratumoral Injection of a Multimutated Oncolytic Herpes Simplex Virus-1 (G207) in Patients Undergoing Craniotomy for Recurrent Malignant Glioma*.

NIH/OBA Receipt Date: 10-10-06. Not Selected for RAC Public Review: 11-17-06

0610-813 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/B-lineage Acute Lymphoblastic Leukemia/Immunotherapy/In Vitro/Retrovirus/Anti-CD19-41BB-CDzeta cDNA/Intravenous

Campana, Dario; St. Jude Children's Research Hospital; Memphis, Tennessee; *Pilot Study of Genetically Modified Haploidentical Natural Killer Cell Infusions for B-lineage Acute Lymphoblastic Leukemia*.

NIH/OBA Receipt Date: 10-10-06. Publicly Reviewed at the December 2006 RAC meeting

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0610-814 (Open) Gene Therapy/Phase II/Other-Cardiovascular Diseases/Peripheral Artery Disease/In Vivo/Plasmid DNA/VOP32E VEGF-A Transcription Factor cDNA/Intramuscular Injection

Annex, Brian H.; Duke University School of Medicine and Durham VA Medical Center; Durham, North Carolina; and Mitchell, Robert; Duke University Medical School; Durham, North Carolina; *Phase II, Multicenter, Randomized, Double Blind, Placebo Controlled, Study of the Safety and Efficacy of EW-A-401 in Critical Limb Ischemia*. Sponsor: Edwards LifeSciences LLC

NIH/OBA Receipt Date: 10-10-06. Not Selected for RAC Public Review: 10-31-06

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0610-815 (Open) Gene Therapy/Phase I/Monogenic Diseases/Limb Girdle Muscular Dystrophy/In Vivo/Adeno-Associated Virus/Serotype 1/alpha-Sarcoglycan cDNA/Intramuscular Injection

Mendell, Jerry R.; Columbus Children's Research Institute; Columbus, Ohio; *Phase I Gene Transfer of rAAV1.tMCK.hαSG for Limb Girdle Muscular Dystrophy Type 2D (LGMD2D)*.

NIH/OBA Receipt Date: 10-10-06. Not Selected for RAC Public Review: 10-31-06

0610-816 (Open) Gene Therapy/Phase II/Cancer/Ovarian Carcinoma/Immunotherapy/In Vivo and In Vitro/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD80).U/caM-1/LFA-3/MUC-1/Subcutaneous and/or Intravenous Injection

Avigen, David; Beth Israel Deaconess Medical Center; Boston, Massachusetts; *A Phase II Randomized Trial Comparing Vaccination with PANVAC-V and PANVAC-F with Ex Vivo Generated Dendritic Cells Transduced with PANVAC-F in Patients with Ovarian Carcinoma in Early Relapse*.

NIH/OBA Receipt Date: 10-20-06. Not Selected for RAC Public Review: 11-9-06

0610-817 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Allogeneic K562 Cells/Cationic Liposome Complexes/GM-CSF cDNA/Intradermal and Subcutaneous Injection

Wu, Catherine J.; Brown, Jennifer; and Alyea, Edwin; Dana-Farber Cancer Institute; Boston, Massachusetts; *Reduced Intensity Stem Cell Transplantation for Advanced Chronic Lymphocytic Leukemia Followed by Vaccination with Lethally Irradiated Autologous Tumor Cells Admixed with Granulocyte Macrophage-Colony Stimulating Factor Secreting K562 Cells*.

NIH/OBA Receipt Date: 10-25-06. Not Selected for RAC Public Review: 11-14-06

0611-818 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Retrovirus/Tyrosinase cDNA/Intradermal Injection

Young, James W.; Memorial Sloan-Kettering Cancer Center; New York, New York; *Immune Responses to Autologous Dendritic Cells Bearing Tumor Antigen Expressed by a Retroviral Transgene in Patients with Malignancy: A Phase I Trial in Melanoma*.

NIH/OBA Receipt Date: 11-8-06. Not Selected for RAC Public Review: 11-29-06

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0611-819 (Open) Gene Therapy/Phase III/Cancer/Non-Small Cell Lung Cancer/Antisense/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid DNA-Electroporation/TGF-β cDNA/Subcutaneous Injection

Dillman, Robert O.; Hoag Cancer Center; Newport Beach, California; Giaccone, Giuseppe; National Institutes of Health; Bethesda, Maryland; Molina, Julian; Mayo Clinic; Rochester, Minnesota; Stephenson, Joe; Jr.; Cancer Center of the Carolinas; Greenville, South Carolina; Bazhenova, Lyudmila; University of California; San Diego, California; Dudek, Arkadiusz; University of Minnesota; Minneapolis, Minnesota; Chu, Siu-Chung Quincy; Cross Cancer Institute; Alberta, Canada; Eaton, Keith D.; Seattle Cancer Care Alliance; Seattle, Washington; Leighl, Natasha B.; Princess Margaret Hospital/University Health Network; Ontario, Canada; Nemunaitis, John J.; Mary Crowley Cancer Research Centers; Dallas, Texas; Burke, James M.; Billings Clinic; Billings, Montana; Shaffer, David R.; Garbo, Lawrence; New York Oncology Hematology, P.C.; Albany, New York; Barve, Minal; Texas Oncology, P.A.; Dallas, Texas; Zenk, David W.; Iowa Blood and Cancer Care, PLC; Cedar Rapids, Iowa; Kovcin, Vladimir; Clinical Hospital Centre Bezanijska Kosa; Belgrade, Serbia; Rancid, Milan; Clinic for Pulmonary Diseases and TB “Knez Selo”; Nis, Serbia; Richard, Donald A.; Texas Oncology; Tyler, Texas; Secen, Nevena; Institute for Pulmonary Diseases of Vojvodina; Sremska Kamenica, Serbia; Dunlop, David; Beatson West of Scotland Cancer Centre; Glasgow, Scotland; Marshall, Ernie; Clatterbridge Centre for Oncology; Wirral, United Kingdom; Juhasz, Ersebet; Orszagos Koranyi TBC es Pulmonologial Intezet; Budapest, Hungary; Balint, Beatrix; Csongrad Megyei Onkormanyzat; Deszk, Hungary; Tolnay, Edina; Pest megyei Tudogyogyintezet; Torokbalint, Hungary; Viscontin, John L; Warren Billhartz Cancer Center; Maryville, Illinois; Onitilo, Adedayo; Marshfield Clinic; Marshfield, Wisconsin; Melnyk, Anton M. S; Jr.; Texas Cancer Center; Abilene, Texas; Bonomi, Philip D.; Rush University Medical Center; Chicago, Illinois; Losonczy, Gyorgy; Semmelweis University; Budapest, Hungary; Papai-Szekely, Zsolt; Fejer Megyei Szent Gyorgy Korhaz; Szejesfegervar, Hungary; Rankin, Elaine; Ninewell Hospital and Medical School; Scotland, United Kingdom; Vinkler, Ilona; Szabolcs-Szatmar-Bereg Megyei Onkormanyzat Josa Andras Oktato Korhaz; Nyiregyhaza, Hungary; Britell, Jonathan; Valley Medical Center; Renton, Washington; Ross, Helen; Mayo Clinic Arizona; Scottsdale, Arizona; Nemunaitis, John; Texas Oncology, P.A.; Dallas, Texas; Lal, Rohit; Guy’s Hospital; London, United Kingdom; Wold, Howard G.; Hematology Oncology Life Center, LLC; Alexandria, Louisiana; Ellison, Donald M.; Charleston Hematology Oncology Associates, PA; Charleston, South Carolina; Steis, Ronald G.; Atlanta Cancer Care; Roswell, Georgia; Gummaraju, Srinivas Chakravarthy; Apollo Hospitals; Juvilee Hills, Hyberbad; Powderly, John D., II; Carolina BioOncology Institute PLLC; Huntersville, North Carolina; Panchal, Harsha P.; The Gujarat Cancer & Research Institute; Asarwa, Ahmedabad; Prabhash, Kumar; Tata Memorial Hospital; Parel, Mumbai; Gurtler, Jayne; Metairie Oncologists; Metairie, Louisiana; Chay, Christopher H.; Cancer Care of WNC, PA; Asheville, North Carolina; Holt, Lawrence B., Jr.; Myrtle Beach, South Carolina; Rao, Raju V. Pasco Hernando Oncology Associates, P.A.; Brooksville, Florida; Reynolds, Craig H.; Ocala Oncology Center; Ocala, Florida; DiCarlo, Brian A.; Central Coast Medical Oncology Corporation; Santa Maria, California; George, Jeffrey R.; Mobile, Alabama; Ali, Haythem, Henry Ford Health Systems; Detroit, Michigan; Melancon, Diane, St. Mary’s Hospital Regional Cancer Center; Grand Junction, Colorado; Parikh, Rupesh; Henderson, Nevada; Chan, David; Redondo Beach, California; Kass, Frederic C.; Santa Barbara Hematology Oncology Medical Group, Inc.; Santa Barbara, California; Koralewski, Piotr; Niezalezny Zespol Opieki Zdrowotnej Verslius Sp z oo; Krakow, Poland; Milanowski, Janusz; Samodzielny Publiczny Szpital Kliniczny nr 4; Lublin; Taguchi Julie A.; SANSUM Clinic; Santa Barbara; California; Krzakowski, Marciej J.; Centrum Onkologii – Instytut im. Marii Sklodowskiej-Curie; Warsaw, Poland; Patel, Ravi; Comprehensive Blood and Cancer Center; Bakersfield, California; Fleming, Donald R.; Cancer Care Center; Elkins, West Virginia; Jassem, Jacek; Akademickie Centrum Kliniczne Szpital Akademii Medycznej w Gdansku; Gdansk; Forlenza, Thomas J.; Richmond University Medical Center; Staten Island, New York; Freimann, Jack H., Jr; Oncology Care Medical Associates; Whittier, California; Huh, Sang Yoon; Coleman Cancer Center; Terre Haute, Indiana; Osarogiagbon, Raymond; University of Tennessee Cancer Institute; Memphis, Tennessee; Volterra, Fabio; Eastchester Center for Cancer Care; Bronx, New York; Oton, Ana; University of Colorado Cancer Center; Aurora, Colorado; Monte, Marc; Clopton Clinic Hematology/Oncology; Jonesboro, Arkansas; Garon, Edward B.; University of California at Los Angeles; Los Angeles, California; Slaughter, Michael; Indiana Oncology-Hematology Consultants; Indianapolis, Indiana; Dingemans, A.C.; University Hospital Maastricht; Maastricht, The Netherlands; Tezcan, Haluk; Kootenai Cancer Center; Coeur d’Alene, Idaho; Brandon, Donald M.; Innovative Research Center of California; San Diego, California; Feinstein, Trevor; Medical Specialists of the Palm Beaches; Lake Worth, Florida; Mahendra, Minish Jain; Noble Hospital; Pune, Maharashtra; van den Heuvel, M. M.; NKI-AVL, Amsterdam, The Netherlands; Kunst, Peter William Alexander; University of Amsterdam; Amsterdam, The Netherlands; Maru, Anish; SEAROC Cancer Center; Rajasthan, India; Ramiau, Rodryg; Wielkoapiskie Centrum Pulmunologii il Torakochirugii; Poznan, Poland; Raina, Vinod; All India Institute of Medical Sciences; New Delhi, India; Ebrahimi, Behnam; Wilshire Oncology Medical Group, Inc.; LaVerne, California; Devan, Sivanandan C.; Regional Cancer Centre; India; Levine, Richard M.; Space Coast Medical Associates, LLP; Titusville, Florida; Kloecker, Goetz H.; University of Louisville, Louisville, Kentucky; Damste, H.E.J. Sinningheghe; Ziekenhuis Groep Twente; Almelo, The Netherlands; Stewart, Mary; Alaska Oncology and Hematology Group; Anchorage, Alaska; Kirmani, Saeeda; Veterans Affairs Healthcare System, San Diego; San Diego, California; Lin, Paul; Medical Specialists of the Palm Beaches; Lake Worth, Florida; Braiteh, Fadi S.; Comprehensive Cancer Centers of Nevada; Las Vegas, Nevada; Dichmann, Robert A.; Central Coast Medical Oncology Corporation; Santa Maria, California; and Wong, Peter P.; Valley Medical Oncology Consultants; Pleasanton, California; *Phase III Registration Study of Lucanix™, a TGF-β2 Antisense Gene-Modified, Allogeneic Tumor Cell Cocktail vs. Pemetrexed in Patients with Stages III/IV Non-Small Cell Lung Cancer*. Sponsor: NovaRx Corporation

NIH/OBA Receipt Date: 11-13-06. Not Selected for RAC Public Review: 12-7-06

0611-820 (Open) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3)galactosyltransferase Gene/Intradermal Injection

Govindan, Ramaswamy; Washington University School of Medicine; St. Louis, Missouri; and Patel, Jyoti D.; Northwestern University; Chicago, Illinois; *A Phase II Study of HyperAcute ®-Lung Cancer Vaccine in Subjects with Advanced Non-Small Cell Lung Cancer Who Responded to First Line Platinum-Doublet Treatment*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 11-13-06. Not Selected for RAC Public Review: 12-1-06

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0612-821 (Open; RAC reviewed with recommendations) Gene Therapy/Phase III/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Fibroblast Growth Factor (FGF) cDNA/Intracoronary Administration

Watkins, Matthew W.; University of Vermont College of Medicine; Burlington, Vermont; Molk, Barry L.; Aurora Denver Cardiology Associates; Aurora, Colorado; Thomas, Gregory; Mission Hospital/St. Joseph Health System; Mission Viejo, California; Kereiakes, Dean; The Christ Hospital; Cincinnati, Ohio; Hodes, Zachary; St. Vincent Hospital; Indianapolis, Indiana; Lieberman, Scott Michael; Cardiovascular Associates of East Texas; Tyler, Texas; Panchal, Vipul R.; Cardiovascular Associates, PSC; Louisville, Kentucky; Chronos, Nicholas A.; St. Joseph's Research Institute; Atlanta, Georgia; Grantham, J. Aaron; St. Luke's Hospital; Kansas City, Missouri; Bethala, Vasanth K.; Medical Research Institute; Slidell, Louisiana; Karlsberg, Ronald P.; Beverly Hills, California; Skelding, Kimberly A.; Geisinger Medical Center; Danville, Pennsylvania; Watkins, Matthew; Fletcher Allen Health Care; Burlington, Vermont; Gill, Santosh K.; Fox Valley Clinical Research Center, LLC; Aurora, Illinois; Guigauri, Pavel; Cardiovascular Associates of Northern Wisconsin; Wausau, Wisconsin; Starling, Mark R.; Banner Health Research Institute; Mesa, Arizona; Henry, Timothy; Minneapolis Heart Foundation; Minneapolis, Minnesota; Caulfield, Todd; Providence Heart Institute; Portland, Oregon; Epstein, Stephen; Washington Hospital Center; Washington, DC; Nadar, Venkatesh K.; Heritage Cardiology Associates; Camp Hill, Pennsylvania; Saenz, Carlos; Florida Hospital Cardiovascular Research; Orlando, Florida; Krueger, Steven; BryanLGH Heart Institute; Lincoln, Nebraska; Ginete, Wilson; Duluth Clinic; Duluth, New Mexico; Shah, Prediman K.; Cedars-Sinai Medical Center; Los Angeles, California; Brott, Brigitta C.; University of Alabama at Birmingham; Birmingham, Alabama; Orlow, Steven W.; Medical Group of Fort Wayne, P.C.; Fort Wayne, Indiana; Lee, Colin D.; St. Luke's Idaho Cardiology Associates; Boise, Idaho; McGrew, Frank A.; III; The Stern Cardiovascular Center, PA; Germantown, Tennessee; Morton, David J.; St. Anthony's Medical Center; St. Louis, Missouri; Rabinowitz, Abram C.; South Texas Cardiovascular Consultants; San Antonio, Texas; Yasuada, Tsunehiro; Massachusetts General Hospital and Harvard Medical School; Boston, Massachusetts; Peart, Brenda; Southwest Heart; Tucson, Arizona; McKeever, Louis; Midwest Heart Foundation; Lombard, Illinois; East, Cara A.; Baylor Heart & Vascular Hospital; Dallas, Texas; Mahmud, Ehtisham; University of California, San Diego; San Diego, California; Dauber, Ira M.; South Denver Cardiology Associates; Littleton, Colorado; Gordon, Paul C.; The Miriam Hospital; Providence, Rhode Island; O'Shaughnessy, Charles D.; North Ohio Research, Ltd.; Elria, Ohio; Tahirkheli, Naeem; Oklahoma Cardiovascular Research Group, Inc.; Oklahoma City, Oklahoma; Pepine, Carl J.; University of Florida; Gainesville, Florida; Reisman, Mark; Swedish Health Services; Seattle, Washington; and Agarwal, Arvind; The Valley Hospital; Ridgewood, New Jersey; *A Randomized, Double-Blind, Placebo-Controlled, Parallel Group, Multicenter Study to Evaluate the Efficacy and Safety of Ad5FGF-4 in Female Patients with Stable Angina Pectoris Who Are Not Candidates for Revascularization*. Sponsor: Cardium Therapeutics, Inc.

NIH/OBA Receipt Date: 12-19-06. Publicly Reviewed at the March 2007 RAC meeting

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0612-822 (Open) Gene Therapy/Phase II/Other/Diabetic Peripheral Neuropathy/In Vivo/Plasmid DNA/ZPF-TF cDNA (Zinc Finger DNA Binding Protein)/Intramuscular Injection

Rendell, Marc S.; Creighton University School of Medicine; Omaha, Nebraska; and Langsdorf, Jennifer; Weill Medical College of Cornell University; New York, New York; *A Phase 2 Repeat Dosing Clinical Trial of SB-509 in Subjects with Diabetic Neuropathy*. Sponsor: Sangamo BioSciences, Inc.

NIH/OBA Receipt Date: 12-22-06. Not Selected for RAC Public Review: 1-17-07

0701-823 (Open) Gene Therapy/Phase I/Cancer/Hodgkin's Lymphoma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Kasamon, Yvette Leslie; Johns Hopkins University School of Medicine; Baltimore, Maryland; *KGEL Vaccine after Initial Therapy of Hodgkin's Lymphoma*.

NIH/OBA Receipt Date: 1-05-07. Not Selected for RAC Public Review: 1-29-07

0701-824 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia (MVA)/HER2 cDNA/Subcutaneous Injection

Guardino, Alice; Stanford University; Stanford, California; *A Phase I Trial of a Fixed Dose of MVA-BN®-HER2 Following 1st – or 2nd – Line Chemotherapy for HER-2-Positive Metastatic Breast Cancer*. Sponsor: BN ImmunoTherapeutics, Inc.

NIH/OBA Receipt Date: 1-12-07. Not Selected for RAC Public Review: 2-05-07

0701-825 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vitro/P4E6/Retrovirus/Human Papilloma Virus E6 cDNA/Subcutaneous Injection

Gulley, James L.; National Institutes of Health; Bethesda, Maryland; *A Double-Blind Randomized Phase 2.5 Trial of ONY-P1 Vaccine Versus Placebo in Men with D0 Prostate Cancer Following Limited Androgen Ablation*.

NIH/OBA Receipt Date: 1-12-07. Not Selected for RAC Public Review: 2-05-07
Closed to accrual: 5-08-09

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0701-826 (Open) Gene Therapy/Phase I/Cancers Expressing HER-2 and-or CEA/Immunotherapy/In Vivo/Plasmid DNA/Adenovirus Type 6/HER-2 and CEA Genes/Intramuscular Injections with Electroporation

Chiappori, Alberto A.; Moffitt Cancer Center & Research Institute; Tampa, Florida; and Montero, Alberto J.; Medical University of South Carolina; Charleston, South Carolina; *A Phase I Study to Evaluate the Safety/Tolerability and Immunogenicity of V930/V932 in Patients with Cancers Expressing HER-2 and/or CEA*. Sponsor: Merck & Co., Inc.

NIH/OBA Receipt Date: 1-12-07. Not Selected for RAC Public Review: 2-05-07

0701-827 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I-II/Monogenic Disease/Recessive Dystrophic Epidermolysis Bullosa (RDEB)/In Vitro/Autologous Keratinocytes/Retrovirus/Human Col7A1 cDNA/Skin Graft

Lane, Albert T.; Stanford University; Stanford, California; *Gene Transfer for Recessive Dystrophic Epidermolysis Bullosa (RDEB)*.

NIH/OBA Receipt Date: 1-16-07. Publicly Reviewed at the March 2007 RAC meeting

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0701-828 (Open) Gene Therapy/Cancer/Ovarian/Immunotherapy/In Vivo/DNA Complex with PEG-PEI-Cholesterol/Interleukin 12 cDNA/Intraperitoneal Injection

Alvarez, Ronald D.; University of Alabama at Birmingham; Birmingham, Alabama; Kelly, Frank Joseph; Huntsville Hospital; Huntsville, Alabama; and Chu, Christina S.; University of Pennsylvania; Philadelphia, Pennsylvania; *A Phase I, Open-Label, Dose Escalation Study of the Safety and Preliminary Efficacy of EGEN 001 in Combination with Carboplatin and Docetaxel in Women with Recurrent, Platinum-Sensitive, Epithelial Ovarian Cancer*. Sponsor: Expression Genetics, Inc.

NIH/OBA Receipt Date: 1-16-07. Not Selected for RAC Public Review: 2-06-07

0701-829 (Open) Gene Therapy/Phase II/Cancer/Pancreatic Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3)galactosyltransferase Gene/Intradermal Injection

Statkute, Laisvyde; Northwestern University, McGaw Medical Center; Chicago, Illinois; Obel, Jennifer; Evanston Northwestern Healthcare; Evanston, Illinois; Hardacre, Jeffrey; University Hospitals Case Medical Center; Cleveland, Ohio; Lenz, Heinz-Josef; USC/Norris Comprehensive Cancer Center; Los Angeles, California; Rocha-Lima, Caio M.; University of Miami; Miami, Florida; Callister, Matthew D.; Mayo Clinic; Scottsdale, Arizona; Chiorean, Elena Gabriela; Indiana University Cancer Center; Indianapolis, Indiana; Safran, Howard; The Miriam Hospital; Providence, Rhode Island; Nugent, Francis W.; Lahey Clinic; Burlington, Massachusetts; Heywood, Glenroy; Albuquerque, New Mexico; Imagawa, David K.; University of California, Irvine; Orange, California; Wang, Yubao; University of Texas Health Science Center at San Antonio; San Antonio, Texas; Espat, N. Joseph; Roger Williams Hospital; Providence, Rhode Island; Greeno, Edward; University of Minnesota; Minneapolis, Minnesota; Baron, Ari; California Pacific Medical Center; San Francisco, California; Weeks, Colin; University of Colorado Health Sciences Center; Aurora, Colorado; Kennedy, Eugene P.; Thomas Jefferson University; Philadelphia, Pennsylvania; Fisher, William, E.; Baylor College of Medicine; Houston, Texas; and Mahalingam, Devalingam; The University of Texas Health Sciences Center at San Antonio; San Antonio, Texas; *A Phase II Study of HyperAcute®–Pancreatic Cancer Vaccine in Subjects with Surgically Resected Pancreatic Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 1-17-07. Not Selected for RAC Public Review: 2-06-07

0701-830 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta cDNAs/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Melanoma Using Lymphodepleting Conditioning Followed by Infusion of Anti-gp100:154-162 TCR-Gene Engineered Lymphocytes*.

NIH/OBA Receipt Date: 1-16-07. Not Selected for RAC Public Review: 2-06-07

0702-831 (Closed) Gene Therapy/Cancer/Malignant Glioma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid/Cationic Liposome Complexes/GM-CSF cDNA/Intradermal and Subcutaneous Injection

Curry, William T., Jr.; Massachusetts General Hospital; Boston Massachusetts; *A Phase I Study of Vaccination with Lethally Irradiated Glioma Cells Mixed with GM-K562 Cells in Patients Undergoing Craniotomy for Recurrent Tumor*.

NIH/OBA Receipt Date: 2-05-07. Not Selected for RAC Public Review: 2-27-07
Closed to accrual: 9-8-09

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0702-832 (Open) Gene Therapy/Cancer/Follicular Lymphoma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid/Cationic Liposome Complexes/GM-CSF cDNA/Intradermal and Subcutaneous Injection

Jacobson, Eric D.; *A Phase I Trial of Vaccination with Lethally Irradiated Lymphoma Cells Admixed with Granulocyte-Macrophage Colony Stimulating Factor Secreting K562 Cells for the Treatment of Previously Untreated or Relapsed Follicular Lymphoma*.

NIH/OBA Receipt Date: 2-08-07. Not Selected for RAC Public Review: 3-01-07

0702-833 (Open) Gene Therapy/Phase I-II/Cancer/Pancreatic Adenocarcinoma/Pro-Drug/Valacyclovir/In Vitro/Adenovirus/ Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection

Fischer, Craig P.; The Methodist Hospital; Houston, Texas; *A Phase 1/2 Study of AdV-TK Gene Transfer in Combination with Chemoradiation for Patients with Pancreatic Adenocarcinoma*.

NIH/OBA Receipt Date: 2-22-07. Not Selected for RAC Public Review: 3-08-07

0703-834 (Open) Gene Therapy/Phase I/Cancer/EBV-Positive Nasopharyngeal Carcinoma/Immunotherapy/In Vitro/LMP1 and LMP2-Specific Cytotoxic T Lymphocytes (CTL)/Adenovirus/LMP2A and LMP-1 cDNAs/Intravenous Administration

Gottschalk, Stephen; Heslop, Helen; Chrystal, Louis; and Rooney, Cliona; Baylor College of Medicine; Houston, Texas; *Administration of LMP1- and LMP2-Specific Cytotoxic T-Lymphocytes to Patients with EBV-Positive Nasopharyngeal Carcinoma*.

NIH/OBA Receipt Date: 3-09-07. Not Selected for RAC Public Review: 3-30-07

0703-835 (Open) Gene Therapy/Phase I/Cancer/EBV-Positive Nasopharyngeal Carcinoma/Immunotherapy/In Vitro/LMP1 and LMP2-Specific Cytotoxic T Lymphocytes (CTL)/Adenovirus/LMP2A and LMP-1 cDNAs/Intravenous Administration

Gottschalk, Stephen; Heslop, Helen; Chrystal, Louis; and Rooney, Cliona; Baylor College of Medicine; Houston, Texas; and Riker, Adam I.; Oschner Cancer Institute; New Orleans, Louisiana; *Administration of LMP1- and LMP2-Specific Cytotoxic T-Lymphocytes Following CD45 Antibody Administration to Patients with EBV-Positive Nasopharyngeal Carcinoma*.

NIH/OBA Receipt Date: 3-09-07. Not Selected for RAC Public Review: 3-30-07

0703-836 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus (MVA)/5T4 cDNA/Intramuscular Injection

Amato, Robert; The Methodist Hospital; Houston, Texas; *A Phase II Trial to Assess the Activity of MVA 5T4 (Trovax®) Plus Docetaxel Versus Docetaxel Alone in Patients with Progressive Hormone Refractory Prostate Cancer (HRPC)*.

NIH/OBA Receipt Date: 3-22-07. Not Selected for RAC Public Review: 4-16-07

0703-837 (Open) Gene Therapy/Phase II/Other/Diabetic Peripheral Neuropathy/In Vivo/Plasmid DNA/ZPF-TF cDNA (Zinc Finger DNA Binding Protein)/Intramuscular Injection

Rendell, Marc S.; Creighton University School of Medicine; Omaha, Nebraska; and Langsdorf, Jennifer; Weill Medical College of Cornell University; New York, New York; *A Phase 2 Repeat Dosing Clinical Trial of SB-509 in Subjects with Moderate to Severe Diabetic Neuropathy and Unmeasurable Nerve Conduction Velocity*. Sponsor: Sangamo BioSciences, Inc.

NIH/OBA Receipt Date: 3-26-07. Not Selected for RAC Public Review: 4-16-07

0703-838 (Closed) Gene Therapy/Phase III/Peripheral Artery Disease/In Vivo/Plasmid/Fibroblast Growth Factor-1 (FGF-1) cDNA/Intramuscular Injections

Comerota, Anthony James; Jobst Vascular Center; Toledo, Ohio; Mendelsohn, Farrell O.; Cardiology, P.C.; Birmingham, Alabama; Martinez, Jeffrey; Peripheral Vascular Associates; San Antonio, Texas; Han, David C.; Penn State Heart & Vascular Institute; Hershey, Pennsylvania; Jamnadas, Pradipkumar; Florida Hospital; Orlando, Florida; Moneta, Gregory L.; Oregon Health & Sciences University; Portland, Oregon; Wittgen, Catherine; St. Louis University; St. Louis, Missouri; Daniel, George; Palm Beach Heart Research Institute; Atlantis, Florida; Russell, Sean Steven; MedCenter One, Inc.; Bismarck, North Dakota; Rockson, Stanley G.; Stanford University; Stanford, California; Sarfati, Mark; University of Utah; Salt Lake City, Utah; Yonehiro, Layne; Baptist Research; Pensacola, Florida; Choksi, Nishit; St. Joseph Mercy Research; Pontiac, Michigan; Clagett, George Patrick; Southwestern Medical Center at Dallas; Dallas, Texas; McGarvey, Joseph F.X., Jr.; Central Bucks Specialists Heart & Vascular; Doylestown, Pennsylvania; Baiju, Pradip; Florida Hospital; Winter Park, Florida; Ballard, Jeffrey L.; Vascular and Interventional Specialists of Orange County, Inc.; Orange, California; Chervu, Arun; Vascular Surgical Associates, P.C.; Austell, Georgia; Jackson, Bruce; A.R.I. Clinical Trials, Inc.; Redondo Beach, California; LaPerna, Lucy; Riverside Methodist Hospital; Columbus, Ohio; Marshall, J. Jeffrey; Northeast Georgia Heart Center, PC; Gainesville, Georgia; Russo, Gilberto C.; Desert Medical Group, dba Desert Oasis Healthcare Medical Group; Rancho Mirage, California; Serena, Thomas E.; Warren General Hospital; Warren, Pennsylvania; Turco, Mark; Washington Adventist Hospital; Takoma Park, Maryland; Heuser, Richard Ross; Affiliated Cardiologists of Arizona; Phoenix, Arizona; Hye, Robert J.; Southern California Permanente Medical Group; San Diego, California; Saucedo, Jorge; The University of Oklahoma Health Sciences Center; Oklahoma City, Oklahoma; Snell, R. Jeffrey; Rush University Medical Center; Chicago, Illinois; Tamim, Wael Z.; Berma Research Group; Fort Lauderdale, Florida; Passman, Marc; University of Alabama at Birmingham; Birmingham, Alabama; Payne, Wyatt G.; Bay Pines VA Healthcare System; Bay Pines, Florida; Islam, M. Ashequl; Baystate Medical Center; Springfield, Massachusetts; Bianchi, Christian; VA Loma Linda Healthcare System; Loma Linda, California; Gosset, James B.; Medical College of Wisconsin; Milwaukee, Wisconsin; Lantis, John C., II; St. Luke's Roosevelt Hospital Center; New York, New York; Arora, Rohit; North Chicago VA Medical Center; North Chicago, Illinois; Zelen, Charles M.; Professional Education and Research Institute, Inc.; Roanoke, Virginia; Buitrago, Martha I.; Idaho Falls Infectious Disease, PLLC; Idaho Falls, Idaho; and Rapp, Joseph H.; San Francisco Veterans Affairs Medical Center; San Francisco, California; *A Randomized Double-Blind Placebo-Controlled Parallel Group Study of the Efficacy and Safety of XRP0038/NV1FGF on Amputation or Any Death in Critical Limb Ischemia Patients With Skin Lesions*. Sponsor: Sanofi-Aventis

NIH/OBA Receipt Date: 3-26-07. Not Selected for RAC Public Review: 4-16-07
Closed to enrollment: 01/31/2012

0703-839 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma/Cytomegalovirus (CMV) Infection/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/CMV pp65-LAMP mRNA/Intradermal Injections

Sampson, John H.; Duke University Medical Center; Durham, North Carolina; *REGULATe: REGULATory T-Cell Inhibition with Daclizumab (Zenapax ®) During Recovery from Therapeutic Temozolomide-Induced Lymphopenia During Antitumor Immunotherapy Targeted Against Cytomegalovirus in Patients with Newly-Diagnosed Glioblastoma Multiforme*.

NIH/OBA Receipt Date: 3-26-07. Not Selected for RAC Public Review: 4-16-07

0703-840 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta cDNAs (Anti-MART-1 F5 TCR)/Intravenous Infusion

Rosenberg, Seven A.; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Melanoma Using Lymphodepleting Conditioning Followed by Infusion of Anti-MART-1 F5 TCR-Gene Engineered Lymphocytes*.

NIH/OBA Receipt Date: 3-28-07. Not Selected for RAC Public Review: 4-18-07

0704-841 (Open) Gene Therapy/Phase II/Other/Diabetic Ulcer/In Vivo/Adenovirus/Serotype 5/Platelet Derived Growth Factor 9PDGF)-β cDNA/Intra-Ulcer Administration

Sheehan, Peter; Mount Sinai School of Medicine; New York, New York; Tallis, Arthur J.; Associated Foot & Ankle Specialists, PLC; Phoenix, Arizona; Brem, Harold; Columbia University Medical Center; New York, New York; Kirsner, Robert; Miami, Florida; Nixon, Brent; Smyrna, Georgia; Aung, Barbara J.; Tucson, Arizona; Starling, Mark; Banner Baywood Medical Center; Mesa, Arizona; D'Costa, Walter; Northern California Foot and Ankle Center Santa Rosa; Santa Rosa, California; Moosa, Hans H.; Southern Illinois Vascular Surgery, PC; Bellesville, Illinois; Gasparis, Antonio; University Hospital Medical Center at Stony Brook University; Stony Brook, New York; Marston, William; University of North Carolina; Chapel Hill, North Carolina; Blume, Peter; North American Center for Limb Preservation; New Haven, Connecticut; Lazarus, Gerald S.; Johns Hopkins Wound Center; Baltimore, Maryland; Gordon, Ian; Long Beach VA Health Care System; Long Beach, California; Kroeker, Roy; Podiatric Medicine & Surgery; Fresno, California; Garner, Warren L.; LAC-USC Medical Center; Los Angeles, California; Sheehan, Peter; Mount Sinai School of Medicine; New York, New York; Payne Wyatt; Bay Pines VAHCS; Bay Pines, Florida; Guidry, Maria T.; University of Texas Medical Branch; Galveston, Texas; Shah, Bhavesh; South Texas Foot Institute; San Antonio; Texas; Serena, Thomas E.; New Bridge Medical Research; Warren, Pennsylvania; Driver, Vicki R.; Boston Medical Center; Boston, Massachusetts; Dove, Cyaandi R.; Advanced Foot & Ankle Center; Las Vegas, Nevada; Mukker, Jay S.; Foot Surgery Sports Medicine; Fresno, California; Longobardi, James; Absolute Foot Care; Chula Vista, California; Stone, Jeffrey A.; Wound Care Consultants; Dallas, Texas; Abdoo, David C.; The Foo Doctors of Santa Cruz County; Watsonville, California; Karr, Jeffrey C.; Karr Foot Kare, PA; Lakeland, Florida; Wali, Soma; Olive View-UCLA Education and Research Institute; Sylmar, California; Lipkin, Scott; Podiatry Associates of LVPG; Allentown, Pennsylvania; Treadwell, Terry A.; Baptist S. Medical Center; Montgomery, Alabama; Halperin, Gabriel J.; Innovative Medical Technologies, LLC; Los Angeles, California; Mendicino, Robert W.; The Western Pennsylvania Hospital; Pittsburgh, Pennsylvania; Etter, E. Leon; St. Joseph Medical Center; Houston, Texas; Ibrado, Ana; Washington, DC; and Brem, Harold; New York University; New York, New York; *A Randomized, Double-Blind, Placebo-Controlled, Single- and Double-Dose, Comparator Arm (Standard of Care), Multicenter Phase 2b Study of Topical GAM501 (AdSPDGF- β/Bovine Type I Collagen Gel) in the Treatment of Non-Healing Diabetic Ulcers of the Lower Extremities*. Sponsor: Tissue Repair Company

NIH/OBA Receipt Date: 4-09-07. Not Selected for RAC Public Review: 4-30-07

0704-842 (Open; RAC reviewed with recommendations) Gene Therapy/Phase III/Cancer/Prostate/Vector-Directed Cell Lysis/Replication-Competent Virus/Pro-Drug/In Vivo/Adenovirus/Yeast Cytosine Deaminase cDNA/5-Flurocytosine/Herpes Simplex Thymidine Kinase cDNA/Valganciclovir /Adenovirus Death Protein/Intratumoral Injection

Movsas, Benjamin; and Freytag, Svend O.; Henry Ford Health System; Detroit, Michigan; and Giles, Francis Joseph; University of Texas Health Science Center at San Antonio; San Antonio, Texas; *A Randomized, Controlled Phase III Trial of Replication-Competent Adenovirus-Mediated Suicide Gene Therapy in Combination with IMRT Versus IMRT Alone for the Treatment of Newly-Diagnosed Intermediate-Risk Prostate Cancer*.

NIH/OBA Receipt Date: 4-05-07. Publicly Reviewed at the June 2007 RAC meeting

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0704-843 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous Lymphocytes/Adenovirus/Serotype 5/cDNA of Engineered Zinc Finger Nucleases (ZFNs) Targeting the Human CCR5 Locus (SB-728)/Intravenous

Tebas, Pablo; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; and Stein, David; Albert Einstein College of Medicine; Bronx, New York; *A Phase I Study of Autologous T-Cells Genetically Modified at the CCR5 Gene by Zinc Finger Nuclease SB-728 in HIV-Infected Patients*.

NIH/OBA Receipt Date: 4-13-07. Publicly Reviewed at the June 2007 RAC meeting

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0704-844 (Open) Gene Therapy/Phase II/Cancer/Pancreatic Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3)galactosyltransferase Gene/Intradermal Injection

Scoggins, Charles Raben; University of Louisville; Louisville, Kentucky; *A Phase II Study of Low Dose HyperAcute®-Panceatic Cancer Vaccine in Subjects with Surgically Resected Pancreatic Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 4-16-07. Not Selected for RAC Public Review: 5-07-07

0704-845 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Malignancies/Immunotherapy/In Vivo/Venezuelan Equine Encephalitis (VEE) Virus Replicon/Carcinoembryonic Antigen (CEA)/Intramuscular (IM)

Morse, Michael; Duke University Medical Center; Durham, North Carolina; *A Phase I/II Study of Active Immunotherapy with CEA(6D) VRP Vaccine (AVX701) in Patients with Advanced or Metastatic Malignancies Expressing CEA*. Sponsor: AlphaVax, Inc.

NIH/OBA Receipt Date: 4-17-07. Not Selected for RAC Public Review: 5-08-07

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0704-846 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/In Vivo/Adenovirus/Serotype 5/Fas-TNF Receptor Chimera Transgene/Intravenous Injection

Triozzi, Pierre L.; The Cleveland Clinic Foundation; Cleveland, Ohio; and Brenner, Andrew; The University of Texas Health Sciences Center; San Antonio, Texas; *A Phase I, Dose Ranging Study to Assess Safety and Distribution of GT-111 in Patients with Advanced Metastatic Cancer*. Sponsor: Vascular Biogenics

NIH/OBA Receipt Date: 4-18-07. Publicly Reviewed at the June 2007 RAC meeting

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0704-847 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vitro/P4E6/Retrovirus/Human Papilloma Virus E6 cDNA/Subcutaneous Injection

Markovic, Svetomir N.; and Vuk-Pavlovic, Stanimir; Mayo Clinic; Rochester, Minnesota; *A Randomized Phase II Clinical Trial to Determine the Safety, Tolerability, and Efficacy of an Allogeneic Whole Cell Vaccine Administered With or Without Autologous Myeloid Dendritic Cells to Patients with Non-Metastatic Androgen Independent Prostate Carcinoma*.

NIH/OBA Receipt Date: 4-19-07. Not Selected for RAC Public Review: 5-11-07

0704-848 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Malignant Glioma/Immunotherapy/In Vitro/Alloclone-002, A Spinal Cord Blood-Derived Cytolytic T-Cell Clone2-Specific/Plasmid DNA/Electroporation/IL13R scFvFc-Zeta T Cell Receptor/Intratumoral Administration

Badie, Behnam; City of Hope National Medical Center; Duarte, California; *A Phase I Study of Intratumoral Administration of Cellular Immunotherapy for Recurrent/Refractory Malignant Glioma Using Alloclone-002 Modified for Glucocorticoid Resistance and Interleukin-2*.

NIH/OBA Receipt Date: 4-20-07. Publicly Reviewed at the June 2007 RAC meeting

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0704-849 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Hematological Malignancies/Elimination of Graft Versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/Inducible Caspase 9 Suicide Gene/AP 1903/Intravenous

Brenner, Malcolm K.; and Heslop, Helen; Baylor College of Medicine; Houston, Texas; and DiStasi, Antonio and Martinez, Caridad; Texas Children's Hospital; Houston, Texas; *A Phase I Study Evaluating the Use of Allodepleted T Cells Transduced with Inducible Caspase 9 Suicide Gene After Haploidentical Stem Cell Transplantation*.

NIH/OBA Receipt Date: 4-23-07. Publicly Reviewed at the June 2007 RAC meeting

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0704-850 (Open) Phase I/Non-therapeutic/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag, Pol cDNAs/Interleukin-15 and Interleukin-12 cDNAs/Intramuscular Injections

Parker, Scott D.; University of Alabama at Birmingham; Birmingham, Alabama; and Parker, Scott; University of Alabama at Birmingham; Birmingham, Alabama; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of PENNVAX-B (gag, pol, env) Given Alone, With IL-12 DNA, Or With a Dose Escalation of IL-15 DNA, In Healthy, HIV-1 Uninfected Adult Participants*.

NIH/OBA Receipt Date: 4-23-07. Not Selected for RAC Public Review: 5-14-07

0704-851 (Open) Gene Therapy/Phase I/Other Diseases-Disorders/Autoimmune Disease/Multiple Sclerosis (MS)/Immunotherapy/In Vitro/Retrovirus/Myelin Basic Protein (hMBP) cDNA/Subcutaneous Implant of a Peptide-Permeable Immunoisolation Device

Weiner, Leslie; USC Keck School of Medicine; Los Angeles, California; *Cell-Based Gene Therapy Using MRC-MBP for Treatment of Multiple Sclerosis-Phase I/II*.

NIH/OBA Receipt Date: 4-24-07. Not Selected for RAC Public Review: 5-15-07

0704-852 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/ β-Thalassemia/In Vitro/Autologous CD34+ Cells/Lentivirus/Human β-Globin Gene/Intravenous

Boulad, Farid; Memorial Sloan-Kettering Cancer Center; New York, New York; *A Phase I Open-Label Clinical Trial for the Treatment of β -Thalassemia Major with Autologous CD34+ Hematopoietic Progenitor Cells Transduced with Thalagen™, a Lentiviral Vector Encoding the Normal Human β -Globin Gene*. Sponsor: Errant Gene Therapeutics, LLC

NIH/OBA Receipt Date: 4-24-07. Publicly Reviewed at the June 2007 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0704-853 (Closed; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Carcinoma of the Ovary or Pancreas, NSCL, or Mesothelioma/Immunotherapy/In Vivo/*Listeria monocytogenes*/Mesothelin cDNA/Intravenous Injection

Jaffee, Elizabeth; and Le, Dung Sidney Kimmel Cancer Center at Johns Hopkins University; Baltimore, Maryland; Nemunaitis, John J.; Mary Crowley Medical Research Center; Dallas, Texas; Sterman, Daniel H.; University of Pennsylvania Medical Center; Philadelphia, Pennsylvania; Hassan, Raffit; National Institutes of Health; Bethesda, Maryland; and Nir-Paz, Ran; Hadassah Hebrew University Medical Center; Jerusalem, Israel; *A Phase I, Open-Label, Dose-Escalation, Multiple Dose Study of the Safety, Tolerability, and Immune Response of CRS-207 in Adult Subjects Who Have Failed or Who Are Not Candidates for Standard Treatment*. Sponsor: Cerus Corporation

NIH/OBA Receipt Date: 4-24-07. Publicly Reviewed at the June 2007 RAC meeting

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0704-854 (Closed) Gene Therapy/Phase III/Other Diseases-Disorders/End Stage Renal Disease/Stenosis Prevention/In Vivo/Adenovirus/Vascular Endothelial Growth Factor D/Perivascular Collagen Collar Device

Lawson, Jeffrey Harold; Duke University Medical Center; Durham, North Carolina; Santana-Aponte, Dixon; Vascular & Endovascular Surgery; Lubbock, Texas; Gallichio, Michael H.; Albany Medical College; Albany, New York; Pescovitz, Mark; Indiana University-Purdue University; Indianapolis, Indiana; McLafferty, Southern Illinois University; Springfield, Illinois; Greenstein, Stuart M.; Albert Einstein College of Medicine; Bronx, New York; Khetan, Umakant, St. Vincent Medical Center; Los Angeles, California; Lee, Timmy Chang; University of Cincinnati; Cincinnati, Ohio; Sam, Albert; Baton Rouge General Medical Center; Baton Rouge, Louisiana; Vouyouka, Ageliki, Mount Sinai School of Medicine; New York, New York; Abreo, Kenneth; Louisiana State University Health Sciences Center; Shreveport, Louisiana; Agarwal, Anil K.; The Ohio State University; Columbus, Ohio; Charytan, Chaim; New York Hospital Queens; Flushing, New York; Lawson, Jeffrey Harold; Duke University Medical Center; Durham, North Carolina; Gasparis, Antonios P.; Stony Brook University Medical Center; Stony Brook, New York; Fulton, Joseph John; University of North Carolina at Chapel Hill; Chapel Hill, North Carolina; Casey, Gregory D.; Hurley Medical Center; Flint, Michigan; Erzurum, Victor Z.; Akron General Medical Center; Akron, Ohio; Gordon, Ian L.; Long Beach VAMC research Health Care Group; Long Beach, California; Josephs, Leon G.; St. Vincent Hospital; Worcester, Massachusetts; and Munir, Jawad; Four Rivers Clinical Research, Inc.; Paducah, Kentucky; *A Phase III, Randomized, Controlled, Open Label, Multicentre Study of the Efficacy and Safety of Trinam® (EG004); an Assessment of Vascular Access Graft Survival in Hemodialysis Patients*. Sponsor: Ark Therapeutics, Ltd.

NIH/OBA Receipt Date: 4-24-07. Not Selected for RAC Public Review: 5-15-07
Closed: 11-2-10

0704-855 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3)galactosyltransferase Gene/Intradermal Injection

Riker, Adam I.; University of South Alabama; Mobile, Alabama; *A Phase II Study of an Anti-Tumor Immunotherapy Regimen Comprised of Pegylated Interferon-Alpha 2b (PEG-Intron®) and HyperAcute® Melanoma Vaccine for Subjects with Advanced Melanoma*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 4-24-07. Not Selected for RAC Public Review: 5-15-07

0704-856 (Open) Gene Therapy/Phase I/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag, Pol cDNAs/Interleukin-15 and Interleukin-12 cDNAs/Intramuscular Injections

Tebas, Pablo; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; *A Phase Ib Partially Randomized Pilot Study Intended to Evaluate the Safety and Immunological Effects of HIV-1 DNA Immunization (PENNVX-B) With or Without Co-Administration of Constructs Containing DNA Encoding for the Expression of Either IL-12 or IL-15 in HIV Infected Individuals*.

NIH/OBA Receipt Date: 4-24-07. Not Selected for RAC Public Review: 5-21-07

0705-857 (Open) Gene Therapy/Phase II/Cancer/Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/p53 cDNA/Intradermal Injection

Antonia, Scott J.; H. Lee Moffitt Cancer Center, University of South Florida; Tampa, Florida; *A Randomized Phase II Trial Using Dendritic Cells Transduced with an Adenoviral Vector Containing the p53 Gene to Immunize Patients with Extensive Stage Small Cell Lung Cancer in Combination with Chemotherapy With or Without All Trans Retinoic Acid*.

NIH/OBA Receipt Date: 5-7-07. Not Selected for RAC Public Review: 5-29-07

0706-858 (Open) Gene Therapy/Phase II/Cancer/Small Cell Lung Cancer/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/p53 cDNA/Intradermal Injection

Kharfan-Dabaja, Mohamed; H. Lee Moffitt Cancer Center & Research Institute, University of South Florida; Tampa, Florida; *Phase II Trial of Autologous Peripheral Blood Hematopoietic Cell Transplantation (PBHCT) Followed by Dendritic Cell p53 Vaccination and Adoptive T Cell Transfer in Patients with Limited Stage Small Cell Lung Cancer*.

NIH/OBA Receipt Date: 6-4-07. Not Selected for RAC Public Review: 6-27-07

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0706-859 (Open) Gene Therapy/Cancer/Leiomyosarcoma/Immunotherapy/In Vitro/Autologous Tumor Cells/Plasmid/TGF β2 Antisense/GMCSF

Hensley, Martee Leigh; Memorial Sloan Kettering Cancer Center; New York, New York; *Compassionate Trial of TGF-β2 Antisense-GMCSF Gene Modified Autologous Xenograft Tumor Cell (TAG) Vaccine in a Single Patient with Leiomyosarcoma*.

NIH/OBA Receipt Date: 6-11-07. Not Selected for RAC Public Review: 6-15-07

0706-860 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Serotype 5/Hepes Simplex Thymidine Kinase cDNA/Intratumoral Injection

Kieran, Mark W.; Dana-Farber Cancer Institute; Boston, Massachusetts; *A Phase I Study of AdV-tk + Prodrug Therapy in Combination with Radiation Therapy for Pediatric Brain Tumors*. Sponsor: Advantagene, Inc.

NIH/OBA Receipt Date: 6-20-07. Not Selected for RAC Public Review: 7-17-07

0706-861 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vivo and In Vitro/Vaccinia Virus/Fowlpox Virus/Carcinoembryonic Antigen (CEA)/B7.1 (CD 80)/ICAM-1/LFA-3/MUC-1/Intratumoral and Subcutaneous and/or Intravenous Injection

Poplin, Elizabeth; UMDNJ-Robert Wood Johnson Medical School, The Cancer Institute of New Jersey; New Brunswick, New Jersey; *Immunotherapy for Unresectable Pancreas Cancer: A Phase 1 Study of Intratumoral Recombinant Fowlpox PANVAC (PANVAC-F) Plus Subcutaneous Recombinant Vaccinia PANVAC (PANVAC-V), PANVAC-F and Recombinant Granulocyte-Macrophage Colony Stimulating Factor (rHGM-CSF)*.

NIH/OBA Receipt Date: 6-22-07. Not Selected for RAC Public Review: 7-19-07

0707-862 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Serotype 5/CCL-21 cDNA/Intradermal Injection

Weber, Jeffrey S.; H. Lee Moffitt Cancer Center & Research Institute, University of South Florida; Tampa, Florida; *A Dose Ranging Trial of Adenovirus CCL-21 Transduced MART-1/gp 100/Tyrosinase/NY-ESO-1 Peptide-Pulsed Dendritic Cells Matured Using Cytokines for Patients With Chemotherapy-Naïve Metastatic Melanoma*.

NIH/OBA Receipt Date: 7-18-07. Not Selected for RAC Public Review: 8-8-07

0707-863 (Open) Gene Therapy/Phase I/Cancer/Mantle Cell and Indolent B Cell Lymphoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Plasmid DNA/Electroporation/CD20-Specific scFvFc-Zeta T Cell Receptor/Intravenous Infusion

Press, Oliver W.; Fred Hutchinson Cancer Research Center; Seattle, Washington; *A Pilot Study to Evaluate the Safety and Feasibility of Cellular Immunotherapy Using Genetically Modified Autologous CD20-Specific T Cells for Patients with Relapsed or Refractory Mantle Cell and Indolent B Cell Lymphomas*.

NIH/OBA Receipt Date: 7-19-07. Not Selected for RAC Public Review: 8-9-07

0707-864 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/Hemophilia B/In Vivo/Self Complementary Adeno-Associated Virus (2/8)/Factor IX Gene/Intravenous Administration

Nienhuis, Arthur W.; St. Jude Children's Research Hospital; Memphis, Tennessee; Glader, Bertil; Stanford University; Stanford, California; and Nathwani, Amit C.; Royal Free Hospital; London; *An Open Label Dose-Escalation Study of a Self Complementary Adeno-Associated Viral Vector (scAAV2/8-LPI-hFIXco) for Gene Therapy of Hemophilia B*.

NIH/OBA Receipt Date: 7-20-07. Publicly Reviewed at the December 2007 RAC meeting

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0707-865 (Open) Gene Therapy/Phase II/Cancer/Non-Small Cell Lung Cancer (NSCLC)/Immunotherapy/In Vivo/*Saccharomyces cerevisiae*/Mutated Ras Oncoprotein cDNAs/Subcutaneous Injections

Azzoli, Christopher G.; Memorial Sloan-Kettering Cancer Center; New York, New York; *A Pilot Trial of the Immunogenicity and Safety of GI-4000; an Inactivated Recombinant Saccharomyces cerevisiae Expressing Mutant Ras Protein, as Consolidation Therapy Following Curative Treatment for Stage I-III Non-Small Cell Lung Cancer (NSCLC) with Tumor Sequence Confirmation of K-ras Mutation*. Sponsor: GlobeImmune, Inc.

NIH/OBA Receipt Date: 7-20-07. Not Selected for RAC Public Review: 8-10-07

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0707-866 (Open) Gene Therapy/Phase II/Cancer/Hepatocellular Carcinoma/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) and Humanized *Escherichia coli* β-galactosidase cDNAs/Intratumoral Injection

Reid, Tony; University of California, San Diego; La Jolla, California; Burke, James M.; The Billings Clinic; Billings, Montana; Ruo, Leyo; McMasters University; Ontario, Canada; Gamblin, Thomas Clark; University of Pittsburgh; Pittsburgh, Pennsylvania; Heo, Jeong; Pursan National University Hospital; Busan, Korea; Lim, Ho Yenong; Samsung Medical Center; Seoul, Korea; Hyun Cheol Chung; Yonsei Cancer Center; Seoul, Korea; Bloomston, Mark; Ohio State University; Columbus, Ohio; and Galanis, Evanthia; Mayo Clinic; Rochester, Minnesota; *A Phase II-a, Open-Label, Randomized Study of JX-594 (Thymidine Kinase-Deleted Vaccinia Virus Plus GM-CSF) Administered by Intratumoral Injection in Patients with Unresectable Primary Hepatocellular Carcinoma*. Sponsor: Jennerex Biotherapeutics.

NIH/OBA Receipt Date: 7-20-07. Not Selected for RAC Public Review: 8-10-07

0707-867 (Open) Phase I/Non-Therapeutic/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag/Pol and Nef/Tat/Vif/Env cDNAs/Interleukine-12 cDNA/Intramuscular Injections

Fischl, Margaret A.; University of Miami School of Medicine; Miami, Florida; *A Phase 1, Multicenter, Randomized, Modified Double-Blind, Placebo-Controlled Trial to Evaluate the Safety and Immunogenicity of an HIV-1 Gag/Pol and the Combination of HIV-1Gag/Pol and Nef/Tat/Vif/Env DNA Vaccine Administered with IL-12 DNA Molecular Adjuvant in HIV Negative Healthy Adults*. Sponsor: Wyeth Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 7-23-07. Not Selected for RAC Public Review: 8-16-07

0707-868 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other Diseases/Peanut Allergy/In Vivo/Heat-Phenol Killed *E. coli*Ara h1, Ara h2, and Ara h3 Genes/Rectal Administration

Wood, Robert A.; Johns Hopkins University School of Medicine; Baltimore, Maryland; and Sicherer, Scott; Mount Sinai Medical Center; New York, New York; *A Phase 1 Safety Study of Heat/Phenol-Killed, E. coli-Encapsulated, Recombinant Modified Peanut Proteins Ara h1, Ara h2, and Ara h3 (EMP-123) in Normal Volunteers Followed by Subjects Allergic to Peanuts*. Sponsor: Allertein Therapeutics, LLC.

NIH/OBA Receipt Date: 7-23-07. Publicly Reviewed at the September 2007 RAC meeting

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0707-869 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection

Sung, Max W.; Mount Sinai School of Medicine; New York, New York; *Phase I Trial of Adenoviral Vector Delivery of the Human Interleukin-12 cDNA by Intratumoral Injection in Patients with Metastatic Breast Cancer*.

NIH/OBA Receipt Date: 7-25-07. Not Selected for RAC Public Review: 8-15-07

0707-870 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma/Cytomegalovirus (CMV) Infection/Immunotherapy/In Vitro/CMV-Specific Autologous Dendritic Cells/RNA Transfer/CMV pp65-LAMP mRNA/Intradermal Injections

Mitchell, Duane Anthony; Duke University Medical Center; Durham, North Carolina; *ERaDICATe: Evaluation of Recovery from Drug-Induced Lymphopenia using Cytomegalovirus-Specific T-Cell Adoptive Transfer*.

NIH/OBA Receipt Date: 7-31-07. Not Selected for RAC Public Review: 8-22-07

0708-871 (Open) Gene Therapy/Phase I-II/Infectious Diseases/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Lentivirus/HIV-1/Antisense env/Intravenous

Gandhi, Rajesh; and Kuritzkes, Daniel, R.; Massachusetts General Hospital; Boston, Massachusetts; *A Phase I/II, Open-Label Study to Evaluate the Safety and Antiviral Activity of Autologous T-Cells Transduced with VRX496 in Treatment of Naïve Subjects with Earl Stage HIV-1 Infection*. Sponsor: VIRxSYS Corporation

NIH/OBA Receipt Date: 8-06-07. Not Selected for RAC Public Review: 8-27-07

0708-872 (Open) Gene Therapy/Phase I/Cancer/Colorectal Carcinoma/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/Intradermal Injection

Schulick, Richard; Johns Hopkins University School of Medicine; Baltimore, Maryland; *A Safety and Feasibility Study of an Allogeneic Colon Cancer Cell Vaccine Administered with a GM-CSF Producing Bystander Cell Line in Patients with Metastatic Colorectal Cancer*.

NIH/OBA Receipt Date: 8-20-07. Not Selected for RAC Public Review: 9-10-07

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0708-873 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Canarypox Virus/gp100, MART-1, MAGE-1, MAGE-3, NY-ESO-1, TRICOM cDNAs/Subcutaneous Injections

Urba, Walter; Robert W. Frantz Cancer Center, Earles A. Chiles Research Institute, Portland Providence Medical Center; Portland, Oregon; Agarawala, Sanjiv; St. Lukes Hospital & Health Network; Bethlehem, Pennsylvania; Albertini, Mark; University of Wisconsin; Madison, Wisconsin; Linett, Gerald; Washington University in St. Louis; St. Louis, Missouri; Ernstoff, Marc S.; Dartmouth-Hitchcock Medical Center; Lebanon, New Hampshire; Lawson, David; Emory University School of Medicine; Atlanta, Georgia; Nemunaitis, John J.; Mary Crowley Cancer Research Centers; Dallas, Texas; Ribas, Antoni; UCLA Medical Center; Los Angeles, California; Gonzalez, Rene Catarelo; University of Colorado Health Sciences Center; Aurora, Colorado; Kirkwood, John; University of Pittsburgh; Pittsburgh, Pennsylvania; Kessinger, Margaret Anne; University of Nebraska Medical Center; Omaha, Nebraska; Ernst, Donald Scott; London Health Sciences Centre; London, Ontario, Canada; Christensen, Scott; UC Davis Cancer Center; Sacramento, California; Stephenson, Joe, Jr.; Cancer Centers of the Carolinas; Greenville, South Carolina; and Kuzel, Timothy; Northwestern University; Chicago, Illinois; *Phase I/II Study of a Multi-Antigen Therapeutic Vaccine in Patients with Metastatic Melanoma*. Sponsor: Sanofi Pasteur

NIH/OBA Receipt Date: 8-20-07. Not Selected for RAC Public Review: 9-12-07

0709-874 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/MART-1, Tyrosinase, gp100, MAGE-3 cDNAs/Intradermal Injections

Pruitt, Scott K.; Duke University Medical Center; Durham, North Carolina; *Active Immunotherapy of Melanoma with Autologous Dendritic Cells Transfected with Tumor Antigen RNA and Small Inhibitory RNAs to Alter Proteasomal Antigen Processing*.

NIH/OBA Receipt Date: 9-10-07. Not Selected for RAC Public Review: 10-3-07

0709-875 (Open) Gene Therapy/Phase II/Infectious Diseases/Human Immunodeficiency Virus/Replication Inhibition/Antisense/In Vitro/CD4+ Autologous Peripheral Blood Cells/Lentivirus/HIV-1/Antisense env/Intravenous

Stein, David; Jacobi Medical Center; South Bronx, New York; and Blick, Gary; Norwalk, Connecticut; *A Rollover Study to Evaluate Safety and Therapeutic Effect of Re-infusing Subjects Who Completed Participation in the VRX-496-USA-05-002 Trial with Autologous T Cells with VRX496*. Sponsor: VIRxSYS Corporation

NIH/OBA Receipt Date: 9-10-07. Not Selected for RAC Public Review: 10-16-07

0710-876 (Open) Gene Therapy/Phase II/Cancer/p53 Overexpression/Immunotherapy/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/Anti-p53 TCR Gene/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Cancer that Overexpresses p53 Using Lymphodepleting Conditioning Followed by Infusion of Anti-p53 TCR-Gene Engineered Lymphocytes and Dendritic Cell Vaccination*.

NIH/OBA Receipt Date: 10-3-07. Not Selected for RAC Public Review: 10-30-07

0710-877 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Other/Parkinson's Disease/In Vivo/Adeno-associated Virus/Glutamic Acid Decarboxylase 65-67 cDNA/Intracerebral Administration

LeWitt, Peter L.; Henry Ford Health System, Franklin Pointe Clinic; Southfield, Michigan; Siddiqui, Mustafa; Wake Forest University Health Sciences; Winston-Salem, North Carolina; Eskandar, Emad N.; Massachusetts General Hospital; Boston, Massachusetts; Kurlan, Roger; University of Rochester; Rochester, New York; Leehey, Maureen A.; University of Colorado School of Medicine; Aurora, Colorado; Thomas, Karen M.; The Ohio State University; Columbus, Ohio; Flaherty, Alice Weaver; Massachusetts General Hospital; Boston, Massachusetts; Kostyk, Sandra K.; The Ohio State University Medical Center; Columbus, Ohio; Richard, Irene Hegeman; Westfall Neurology; Rochester, New York; and Poston, Kathleen L.; Stanford University Medical Center; Stanford, California; *Phase 2 Safety and Efficacy Study Evaluating Glutamic Acid Decarboxylase Gene Transfer to the Subthalamic Nuclei in Subjects with Advanced Parkinson's Disease*. Sponsor: Neurologix, Inc.

NIH/OBA Receipt Date: 10-5-07. Publicly Reviewed at the December 2007 RAC meeting

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0710-878 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Glioma/Pro-Drug/In Vitro/Neural Stem Cells (NSC), Line HB1.F3/Retrovirus/v-myc cDNA/*E. coli* Cytosine Deaminase (CD) cDNA/Intracerebral Administration

Portnow, Jana; City of Hope National Medical Center; Duarte, California; *A Pilot Feasibility Study of Oral 5-Flurocytosine and Genetically-Modified Neural Stem Cells Expressing E. coli Cytosine Deaminase for Treatment of Recurrent High-Grade Gliomas*.

NIH/OBA Receipt Date: 10-9-07. Publicly Reviewed at the December 2007 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0710-879 (Open) Gene Therapy/Cancer/Pancreas/Immunotherapy/In Vivo/*Saccharomyces cerevisiae*/Mutated Ras Oncoprotein cDNAs/Subcutaneous Injections

O'Dwyer, Peter J.; University of Pennsylvania; Philadelphia, Pennsylvania; *Pilot Study of Safety and Feasibility of GI-4000, an Inactivated Recombinant Saccharomyces cerevisiae Expressing Mutant Ras Protein Combined with Adoptive T Cell Transfer and Chemoradiotherapy in Locally Advanced Pancreatic Cancer*.

NIH/OBA Receipt Date: 10-9-07. Not Selected for RAC Public Review: 10-30-07

0710-880 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I-IIA/Other Disorders/Ischemic Stroke/In Vitro/Allogeneic Adult Bone-Marrow Derived Neuroprogenitor Cells/Plasmid/Notch-1 Intracellular Domain (NICD) Gene/Intracranial Administration

Kondziolka, Douglas; University of Pittsburgh School of Medicine; Pittsburgh, Pennsylvania; *A Phase 1/2A Study of the Safety and Efficacy of Modified Stromal Cells (SB623) in Patients with Stable Ischemic Stroke*. Sponsor: SanBio, Inc.

NIH/OBA Receipt Date: 10-9-07. Publicly Reviewed at the December 2007 RAC meeting

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0710-881 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Interleukin-12 cDNA/RheoSwitch® Therapeutic System/Intratumoral Injection

Tarhini, Ahmad; University of Pittsburgh School of Medicine; Pittsburgh, Pennsylvania; Gordon, Michael S.; Pinnacle Oncology Hematology; Scottsdale, Arizona; Richards, Jon M.; Oncology Specialists, S.C.; Park Ridge, Illinois; Guarino, Michael J.; Christiana Care Health Services; Newark, Delaware; Burke, James M.; Billings Clinic; Billings, Montana; Nemunaitis, John J.; Mary Crowley Medical Research Center; Dallas, Texas; Guarino, Michael J.; Christiana Care Health Services; Newark, Delaware; O'Day, Steven; The Angeles Clinic and Research Center; Los Angeles, California; Schwartzentruber, Douglas; Goshen, Indiana; Zaren, Howard A.; St. Joseph's/Candler Hospital; Savannah, Georgia; Geller, Robert B.; Billings Clinic; Billings, Montana; and Drabick, Joseph J.; Penn State College of Medicine, Hershey; Hershey, Pennsylvania; *Phase 1b, Open Label Trial to Define the Safety, Tolerance, Transgene Function, and Immunological Effects of Intratumoral Injection(s) of Adenoviral Transduced Autologous Dendritic Cells Engineered to Express hIL1-12 Under Control of the RheoSwitch® Therapeutic System in Subjects With Stage III and IV Melanoma*. Sponsor: Intrexon Corporation

NIH/OBA Receipt Date: 10-9-07. Publicly Reviewed at the December 2007 RAC meeting

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0710-882 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta Chain cDNAs/Canarypox Virus/B7.1, ICAM-1, and LFA-3 (TRICOM) cDNAs/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Melanoma Using Lymphodepleting Conditioning Followed by Infusion of Anti-MART-1 F5 TCR-Gene Engineered Lymphocytes and ALVAC Virus Immunization*.

NIH/OBA Receipt Date: 10-24-07. Not Selected for RAC Public Review: 1-02-08

0710-883 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta Chain cDNAs/Canarypox Virus/B7.1, ICAM-1, and LFA-3 (TRICOM) cDNAs/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Melanoma Using Lymphodepleting Conditioning Followed by Infusion of Anti-gp100:154-162 TCR-Gene Engineered Lymphocytes and ALVAC Virus Immunization*.

NIH/OBA Receipt Date: 10-24-07. Not Selected for RAC Public Review: 1-02-08

0711-884 (Open) Gene Therapy/Phase I-II/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Allogeneic Neuroblastoma Cell Lines/Retrovirus/Cytokine/Interleukin-2 cDNA/Plasmid/Electroporation/Chemokine/Lymphotactin/Subcutaneous Injection

Louis, Chrystal; and Brenner, Malcolm; Baylor College of Medicine; Houston, Texas; *A Phase I/II Study of Immunization with Lymphotactin and Interleukin 2 Gene Modified Neuroblastoma Tumor Cells after High-Dose Chemotherapy and Autologous Stem Cell Rescue in Patients with High Risk Neuroblastoma (CheSAT)*.

NIH/OBA Receipt Date: 11-15-07. Not Selected for RAC Public Review: 12-07-07

0712-885 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta Chain cDNAs/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Transfer of Autologous T Cells Transduced with the Anti-Mart-1 F5 T Cell Receptor in High Risk Melanoma*.

NIH/OBA Receipt Date: 12-02-07. Not Selected for RAC Public Review: 1-07-08

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0712-886 (Open) Gene Therapy/Phase II/NY-ESO Expressing Cancer/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta Chain cDNAs/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Cancer that Expresses NY-ESO-1 Using Lymphodepleting Conditioning Followed by Infusion of Anti-NY-ESO-1 TCR-Gene Engineered Lymphocytes*.

NIH/OBA Receipt Date: 12-20-07. Not Selected for RAC Public Review: 1-15-08

0712-887 (Open) Gene Therapy/ Phase I-II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Plasmids/CD40L cDNA/Intravenous Infusion

Ernstoff, Marc; Dartmouth Hitchcock Medical Center; Lebanon, New Hampshire; and Wong, Michael K. K.; Roswell Park Cancer Institute; Buffalo, New York; *A Phase I/II Study Testing the Biologic Activity and Safety of AGS-003 as an Immunotherapeutic in Subjects with Newly Diagnosed Advanced Stage Renal Cell Carcinoma (RCC)*. Sponsor: Argos Therapeutics, Inc.

NIH/OBA Receipt Date: 12-21-07. Not Selected for RAC Public Review: 2-04-08

0712-888 (Open) Gene Therapy/ Phase II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Plasmids/CD40L cDNA/Intravenous Infusion

Ernstoff, Marc; Dartmouth Hitchcock Medical Center; Lebanon, New Hampshire; *A Phase II Study Testing the Safety and Activity of AGS-003 as an Immunotherapeutic in Subjects with Newly Diagnosed Advanced Stage Renal Cell Carcinoma in Combination with a Tyrosine Kinase Inhibitor*. Sponsor: Argos Therapeutics, Inc.

NIH/OBA Receipt Date: 12-21-07. Not Selected for RAC Public Review: 3-10-08

0712-889 (Open) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Muscle Cells/ Adenovirus/Serotype 2/Hypoxia Inducible Factor (HIF)-1/VP16 cDNA/Intramuscular Injection

Kramer, Christopher M.; University of Virginia Health System; Charlottesville, Virginia; *MRI Assessment of Angiogenic Response in Patients with Peripheral Artery Disease: A Feasibility Study*. Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 12-21-07. Not Selected for RAC Public Review: 1-17-08

0801-890 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Cachexia/In Vivo/Plasmid/Human Growth Hormone Releasing Hormone (GHRH) cDNA/Intramuscular Injection

Kamble, Rammurti; Baylor College of Medicine; Houston, Texas; *Phase I, Single Center, Open-Label, Dose Escalation Study to Evaluate the Safety and Tolerability of GHRH DNA Plasmid (VGX-3200) + Electroporation in Adults with Cancer Cachexia*. Sponsor: VGX Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 1-14-08. Publicly Reviewed at the March 2008 RAC meeting

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0801-891 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Plasmid/Mammaglobin-A cDNA/Biojector/Intramuscular Injection

Gilllanders, William E.; Washington University School of Medicine; Saint Louis, Missouri; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of a Mammaglobin-A Single Chain Trimer DNA Vaccine in Breast Cancer Patients with Metastatic Disease*.

NIH/OBA Receipt Date: 1-14-08. Not Selected for RAC Public Review: 2-05-08

0801-892 (Closed) Gene Therapy/Phase I/Cancer/CD19-CMV+ Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Lentivirus/CD 19 Antigen Specific Chimeric Antigen Receptor (CAR)/Intravenous Injections

Popplewell, Leslie; City of Hope; Duarte, California; *Phase I Study to Evaluate Cellular Immunotherapy Using Genetically-Modified Autologous CMV x CD19 Bispecific T Cells Following T-Cell Depleted Autologous Peripheral Blood Stem Cell Transplantation for Patients with Recurrent/Refractory Intermediate Grade B-Lineage Non-Hodgkin Lymphoma*.

NIH/OBA Receipt Date: 1-14-08. Not Selected for RAC Public Review: 2-05-08
Never initiated: 7-13-10

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0801-893 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Immunotherapy/In Vivo/Plasmid/Adenovirus/Serotype 6/Human Telomerase Reverse Transcriptase (hTERT) cDNA/Intramuscular Injection/Electroporation

Camacho, Luis H.; Oncology Consultants, P.A.; Houston, Texas; *A Phase I Investigation of the Safety, Tolerability and Immunogenicity of V934/935 hTERT Vaccination in Cancer Patients with Selected Solid Tumors*. Sponsor: Merck & Co., Inc.

NIH/OBA Receipt Date: 1-15-08. Not Selected for RAC Public Review: 2-05-08

0801-894 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Immunotherapy/In Vivo/Naked Plasmid/Melan-A/Tyrosinase/Intra-lymphnodal Injection

Weber, Jeffrey S.; University of South Florida; Tampa, Florida; Ernstoff, Marc S.; Dartmouth-Hitchcock Medical Center; Lebanon, New Hampshire; Ribas, Antoni; University of California at Los Angeles; Los Angeles, California; Samlowski, Wolfram E.; Nevada Cancer Institute; Las Vegas, Nevada; Abesada-Terk, Guillermo; Martin Memorial Cancer Center; Stuart, Florida; Boasberg, Peter D.; The Angeles Clinic and Research Center; Los Angeles, California; and Goodman, Oscar; Nevada Cancer Institute; Las Vegas, Nevada; *A Phase 1/2, Open Label, Non-Randomized Dose Escalation Study to Evaluate the Safety, Tolerability, Immune Response and Clinical Response of Multiple Doses of MKC1106-MT in Subjects with Advanced Melanoma*.

NIH/OBA Receipt Date: 1-15-08. Not Selected for RAC Public Review: 2-05-08

0801-895 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/In Vitro/CD34+ Autologous Peripheral Blood Cells/SIN Lentivirus/Fanconi Anemia Complementation Group A cDNA/Intravenous

Becker, Pamela S.; University of Washington School of Medicine; Seattle, Washington; *A Phase I Study of Gene Transfer for Patients with Fanconi Anemia Complementation Group A (FANCA)*.

NIH/OBA Receipt Date: 1-15-08. Publicly Reviewed at the March 2008 RAC meeting

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0801-896 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Heart Failure/In Vivo/Adenovirus/Serotype 5/Stromal Cell-Derived Factor (SDF-1) cDNA/Intracoronary Administration Using Bioheart's MyoCath™ Needle-Injection Catheter System

Pepine, Carl J.; University of Florida College of Medicine; Gainesville, Florida; *MYOHEART-SDF™ (Myogenesis Heart Efficiency and Regeneration Trial), A Phase I, Open-Label, Non-Randomized, Dose Escalation, Multi Center Study to Assess the Safety and Cardiovascular Effects of the Implantation of Autologous Skeletal Myoblasts Modified to Express the SDF-1 Protein (MyoCell™ SDF-1) via Multi-Electrode Percutaneous Transendocardial Catheter (MyoStar™) with Cardiac Navigation Guidance (NOGA™) in Congestive Heart Failure Patients Post Myocardial Infarction(s) With Prior Placement of an Implantable Cardioverter Defibrillator (ICD)*. Sponsor: Bioheart, Inc.

NIH/OBA Receipt Date: 1-15-08. Publicly Reviewed at the March 2008 RAC meeting

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0801-897 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I-II/Cardiovascular Diseases/Peripheral Artery Disease (PAD)/In Vivo/Sendai Virus/Fibroblast Growth Factor (FGF) cDNA/Intramuscular Injection

Annex, Brian H.; Duke University Medical Center; Durham, North Carolina; *A Phase 1/2 Multi-Center, Open Label, Dose Escalation Study to Evaluate the Safety and Tolerability of DVC1-0101 Administered Intramuscularly in Subjects with Stable Peripheral Artery Disease*. Sponsor: DNAVEC Corp.

NIH/OBA Receipt Date: 1-15-08. Publicly Reviewed at the March 2008 RAC meeting

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0801-898 (Open) Gene Therapy/Phase I/Cancer/Glioma/Chemoprotection/In Vitro/Peripheral Blood CD34+ Cells/Lentivirus/O^6-Methylguanine DNA Methyltransferase cDNA/Intravenous Infusion

Gerson, Santon L.; Case Western Reserve University; Cleveland, Ohio; *O^6-Benzylguanine (BG) and Temozolomide (TMZ) Therapy of Glioblastoma Multiforme in Patients with MGMT Positive Tumors with Infusion of Autologous P140KMGMT+ Hematopoietic Progenitors to Protect Hematopoiesis*.

NIH/OBA Receipt Date: 1-15-08. Not Selected for RAC Public Review: 2-05-08

0801-899 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Vector-Directed Cell Lysis/In Vivo/Herpes Simplex Virus Type 1/Tumor Lysis/Intracerebral Injection

Markert, James M.; University of Alabama at Birmingham; Birmingham, Alabama; *A Phase I Study of M032, a Genetically Engineered HSV-1 Expressing IL-12, in Patients with Recurrent/Progressive Glioblastoma Multiforme, Anaplastic Astrocytoma, or Gliosarcoma*.

NIH/OBA Receipt Date: 1-16-08. Not Selected for RAC Public Review: 2-05-08

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0801-900 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia (MVA)/Prostate Specific Antigen (PSA) and Prostatic Acid Phosphatase (PAP) cDNA/Subcutaneous Injection

Bilhartz, David L.; Urology Associates; Nashville, Tennessee; Ruiz, Henry E.; Urology Associates of South Texas, PA; McAllen, Texas; Cochran, James S.; Urology Clinics of North Texas, PA; Dallas, Texas; Karlin, Gary S.; Lawrenceville Urology, P.A.; Lawrenceville, New Jersey; McLeod, David G.; Walter Reed Army Medical Center; Washington, DC; Reiling, Richard; Presbyterian Hospital; Charlotte, North Carolina; Adams, George W., Jr.; Urology Centers of Alabama, PC; Homewood, Alabama; and Gittelman, Marc C.; South Florida Medical Research; Aventura, Florida; *An Open-Label, Phase I Dose Escalation Trial of MVA-BN ®-PRO in Men with Androgen-Insensitive Prostate Cancer*. Sponsor: BN ImmunoTherapeutics, Inc.

NIH/OBA Receipt Date: 1-16-08. Not Selected for RAC Public Review: 2-05-08

0802-901 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta Chain cDNAs/Intravenous Infusion

Ribas, Antoni; UCLA Medical Center; Los Angeles, California; *Adoptive Transfer of MART-1 F5 TCR Engineered Peripheral Blood Mononuclear Cells (PBMC) after a Nonmyeloablative Conditioning Regimen, with Administration of MART-$1_{26.35}$-Pulsed Dendritic Cells and Interleukin-2, in Patients with Advanced Melanoma*.

NIH/OBA Receipt Date: 2-05-08. Not Selected for RAC Public Review: 2-27-08

0802-902 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Laheru, Daniel; Johns Hopkins School of Medicine; Baltimore, Maryland; *A Randomized Three-arm Neoadjuvant and Adjuvant Feasibility and Toxicity Study of a GM-CSF Secreting Allogeneic Pancreatic Cancer Vaccine Administered Either Alone or in Combination with Either a Single Intravenous Dose or Daily Metronomic Oral Doses of Cyclophosphamide for the Treatment of Patients with Surgically Resected Adenocarcinoma of the Pancreas*.

NIH/OBA Receipt Date: 2-06-08. Not Selected for RAC Public Review: 2-28-08

0802-903 (Closed) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal Injection

Vuky, Jacqueline; Virginia Mason Medical Center; Seattle, Washington; *Phase II Trial of Neoadjuvant Docetaxel and CG1940/CG8711 Followed by Radical Prostatectomy in Patients with High-Risk, Clinically Localized Prostate Cancer*.

NIH/OBA Receipt Date: 2-12-08. Not Selected for RAC Public Review: 2-25-08
Study terminated: 10-14-08

0802-904 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Vaccinia Virus/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) and Humanized *Escherichia coli* β-galactosidase cDNAs/Intravenous Injection

Burke, James; Billings Clinic; Billings, Montana; Stephenson, Joe, Jr.; Cancer Centers of the Carolina; Greenville, South Carolina; Hass, Andrew R.; University of Pennsylvania; Philadelphia, Pennsylvania; and Chow, Laura; Ottawa Hospital Regional Cancer Center; Ottawa, Ontario; *A Phase I Dose Escalation Study of JX-594 (Thymidine Kinase-deleted Vaccinia Virus Plus GM-CSF) Administered by Intravenous Infusion in Patients with Refractory Solid Tumors*. Sponsor: Jennerex Inc.

NIH/OBA Receipt Date: 2-19-08. Not Selected for RAC Public Review: 3-10-08

0802-905 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Metastatic Non-Hematologic Neoplasms/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interleukin-12 cDNA/Intratumoral Injection

Sung, Max W; Mount Sinai School of Medicine; New York, New York; *Phase I Trial of Intravenous Recombinant Human 4-1BB Ligand Fusion Protein (hIg-h4-1BB-Ls) in Combination with Intratumoral Adenovirus Vector Expressing Human Interleukin-12 cDNA (Adv.hIL12) and Oral Sunitinib Malate in Patients with Metastatic Non-Hematologic Neoplasms*.

NIH/OBA Receipt Date: 2-21-08. Publicly Reviewed at the June 2008 RAC meeting

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0802-906 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid DNA/Prostatic Acid Phosphatase (PAP) cDNA/Intradermal Injection

McNeel, Douglas; University of Wisconsin; Madison, Wisconsin; and Fong, Lawrence; University of California San Francisco; San Francisco, California; *Randomized Phase II Trial of a DNA Vaccine Encoding Prostatic Acid Phosphatase (pTVG-HP) Versus GM-CSF Adjuvant in Patients with Non-Metastatic Prostate Cancer*.

NIH/OBA Receipt Date: 2-29-08. Not Selected for RAC Public Review: 3-21-08

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0803-907 (Open) Gene Therapy/Phase I/Cancer/Myelodysplastic Syndrome (MDS)/Immunotherapy/In Vitro/Allogeneic K562 Cells/Plasmid DNA/GM-CSF cDNA/CD40L cDNA/Intradermal Injection

Pinilla, Javier; H. Lee Moffitt Cancer & Research Institute; Tampa, Florida; *A Phase I Pilot Study of Immunotherapy Using Lenalidomide Plus "Bystander" Vaccine in Patients with High-Risk Myelodysplastic Syndrome (MDS)*.

NIH/OBA Receipt Date: 3-05-08. Not Selected for RAC Public Review: 3-27-08

0803-908 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Immunotherapy/In Vitro/Autologous Tumor Cells/Plasmid/GM-CSF cDNA/TGF β2 Antisense/Intradermal Injection

Olivares, Jairo R.; Mary Crowley Medical Research Center; Dallas, Texas; *Phase I Trial of TGF β2 Antisense GM-CSF Gene Modified Autologous Tumor Cell (TAG) Vaccine for Advanced Cancer*.

NIH/OBA Receipt Date: 3-10-08. Not Selected for RAC Public Review: 3-31-08

0803-909 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid DNA/Prostatic Acid Phosphatase (PAP) cDNA/Intradermal Injection

McNeel, Douglas; University of Wisconsin, School of Medicine and Public Health; Madison, Wisconsin; *A Pilot Randomized Two-Arm Study of a DNA Vaccine Encoding Prostatic Acid Phosphatase (PAP) in Patients with Non-Metastatic Castrate-Resistant Prostate Cancer*.

NIH/OBA Receipt Date: 3-20-08. Not Selected for RAC Public Review: 4-10-08

0803-910 (Open) Gene Therapy/Phase I/Infectious Disease/Cytomegalovirus (CMV), Adenovirus, EBV Infections/In Vitro/EBV/Allogeneic Virus-Specific Cytotoxic T Lymphocytes/Adenovirus/CMV pp65 Gene/Intravenous

Heslop, Helen E.; Baylor College of Medicine; Houston, Texas; Antin, Joseph H.; Dana Farber Cancer Institute; Boston, Massachusetts; Szabolcs, Paul; Duke University Medical Center; Durham, North Carolina; Kohn, Donald; Children's Hospital of Los Angeles; Los Angeles, California; Uday, Popat and Shpall, Elizabeth; University of Texas MD Anderson Cancer Center; Houston, Texas; Avigan, David E.; Beth Israel Deaconess Medical Center; Boston, Massachusetts; Dey, Bimalangshu; Massachusetts General Hospital; Boston, Massachusetts; Pai, Sung-Yun; Children's Hospital Boston; Boston, Massachusetts; Kleiner, Gary Ira; University of Miami; Miami, Florida; Rowley, Scott D.; Hackensack University Medical Center; Hackensack, New Jersey; and De Oliveira, Satiro N.; University of California, Los Angeles; Los Angeles, California; *Closely HLA-Matched Allogeneic Virus Specific Cytotoxic T-Lymphocytes (CTL) to Treat Persistent Reactivation or Infection with Adenovirus, CMV and EBV after Hemopoietic Stem Cell Transplantation*.

NIH/OBA Receipt Date: 3-26-08. Not Selected for RAC Public Review: 4-16-08

0804-911 (Open) Gene Therapy/Phase I-II/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/GM-CSF-Carbonic Anhydrase IX (GMCAIX) Fusion Protein cDNA/Intradermal Injections

Belldegrun, Arie S.; UCLA School of Medicine; Los Angeles, California; *Phase I/II Study of Carbonic Anhydrase IX – Molecular Targeted Kidney Cancer Vaccine Therapy*.

NIH/OBA Receipt Date: 4-16-08. Not Selected for RAC Public Review: 5-13-08

0804-912 (Open) Gene Therapy/Phase II/Cancer/Glioblastoma Multiforme/Immunotherapy/In Vivo/Plasmid in Poly (DL-lactide-coglycolide) (PLG) Microparticles/Cytochrome P450 Isoenzyme 1B1 (CYP1B1) cDNA/Subcutaneous Injection

Heimberger, Amy B.; University of Texas MD Anderson Cancer Center; Houston, Texas; *Phase II Clinical Trial of ZYC300 in Recurrent Glioblastoma Multiforme (GBM) Patients*.

NIH/OBA Receipt Date: 4-14-08. Not Selected for RAC Public Review: 5-08-08

0804-913 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Breast/Apoptosis/In Vivo/Cationic Liposome Complexes/DOTAP-Cholesterol/BikDD (*Bik*, also known as *nbk*) Gene/Intravenous Infusion

Hortobagyi, Gabriel N.; University of Texas MD Anderson Cancer Center; Houston, Texas; *A Phase I Study of BikDD Therapy in Advanced Breast Cancer*.

NIH/OBA Receipt Date: 4-18-08. Publicly Reviewed at the June 2008 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0804-914 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Pancreas/Apoptosis/In Vivo/Cationic Liposome Complexes/DOTAP-Cholesterol/BikDD (*Bik*, also known as *nbk*) Gene/Intravenous Infusion

Javle, Milind; University of Texas MD Anderson Cancer Center; Houston, Texas; *A Phase I Open-Label Dose Escalation Study to Assess the Safety and Tolerability of the BikDD nanoparticle in Patients with Advanced Pancreatic Cancer*.

NIH/OBA Receipt Date: 4-18-08. Publicly Reviewed at the June 2008 RAC meeting

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0804-915 (Open) Gene Therapy/Phase I/Cancer/Non-Hodgkin's Lymphoma/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Ramos, Carlos A.; Dotti, Gianpietro; Brenner, Malcolm K.; and Heslop, Helen E.; Baylor College of Medicine; Houston, Texas; *Phase I Study of the Administration of Peripheral Blood T Cells or EBV Specific CTLs Expressing CD19 Chimeric Chronic Lymphocytic Leukemia Receptors for Advanced B-Cell Non-Hodgkin's Lymphoma and Chronic Lymphocytic Leukemia*.

NIH/OBA Receipt Date: 4-21-08. Not Selected for RAC Public Review: 5-12-08

0804-916 (Open) Gene Therapy/Phase I/Cancer/Cervical/In Vivo/Plasmid DNA/HPV16 E6-E7 Fusion Protein cDNA/HPV18 E6-E7 Fusion Protein cDNA/Intramuscular Injection in Combination with Electroporation

Chu, Christina S.; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; Parker, Robert L.; Lyndhurst Gynecologic Associates; Winston-Salem, North Carolina; Sunyecz, John; Laurel Highlands OB/GYN, P.C.; Hopwood, Pennsylvania; and Morales-Ramirez, Javier O.; Clinical Research Puerto Rico; San Juan, Puerto Rico; *Phase I, Open-Label, Dose Escalation Study to Evaluate the Safety, Tolerability, and Immunogenicity of Human Papillomavirus (HPV) DNA Plasmid (VGX-3100) + Electroporation (EP) in Adult Females with Histological Diagnosis of Grade 2 or 3 Cervical Intraepithelial Neoplasia (CIN)*. Sponsor: VGX Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 4-21-08. Not Selected for RAC Public Review: 5-12-08

0804-917 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Other Diseases-Disorders/Autoimmune Disease/Ulcerative Colitis/Lactococcus lactis/Human Interleukin 10 (hIL-10) cDNA/Combination of Oral Administration (Enteric-Coated Capsule) and Topical Rectal Application (Enema)

Isaacs, Kim L.; University of North Carolina at Chapel Hill; Chapel Hill, North Carolina; *A Phase 2a Randomized, Placebo-Controlled, Double-Blind, Multi-Center Dose Escalation Study, to Evaluate the Safety, Tolerability, Pharmacodynamics and Efficacy of AG011, in Subjects with Moderately Active Ulcerative Colitis*. Sponsor: ActoGeniX.

NIH/OBA Receipt Date: 4-21-08. Publicly Reviewed at the June 2008 RAC meeting

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0804-918 (Open) Gene Therapy/Phase I/Monogenic Diseases/Leber Congenital Amaurosis Type 2/Retinal Disease Due to RPE65 Mutations/In Vivo/Adeno-Associated Virus/Serotype 2/RPE65 cDNA/Subretinal Injection

Stout, J. Timothy; Oregon Health & Science University; Portland, Oregon; *A Multiple-Site, Phase 1/2, Safety and Efficacy Trial of a Recombinant Adeno-Associated Virus Vector Expressing RPE65 (rAAV2-CB-hRPE65) in Patients with Leber Congenital Amaurosis Type 2*. Sponsor: Applied Genetic Technologies Corporation.

NIH/OBA Receipt Date: 4-22-08. Not Selected for RAC Public Review: 5-13-08

0804-919 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Venezuelan Equine Encephalitis (VEE) Virus Replicon/Prostate-Specific Membrane Antigen (PSMA) cDNA/Subcutaneous Injection

Slovin, Susan; Memorial Sloan-Kettering Cancer Center; New York, New York; *A Phase I Dose Escalation Trial of Vaccine Replicon Particles (VRP) Expressing Prostate-Specific Membrane Antigen (PSMA) in Subjects with Prostate Cancer*. Sponsor: PSMA Development Company [subsidiary of Progenics Pharmaceuticals, Inc.]

NIH/OBA Receipt Date: 4-22-08. Not Selected for RAC Public Review: 5-13-08

0804-920 (Open) Gene Therapy/Phase I-II/Cancer/Her-2 Expressing Cancer/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor Her-2 scFv CD28 zeta cDNA/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Phase I/II Study of Metastatic Cancer that Expresses Her-2 Using Lymphodepleting Conditioning Followed by Infusion of Anti-Her-2 Gene Engineered Lymphocytes*.

NIH/OBA Receipt Date: 4-22-08. Not Selected for RAC Public Review: 5-13-08

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0804-921 (Open) Gene Therapy/Phase I/Human Immunodeficiency Virus/Immunotherapy/In Vitro/Autologous T Lymphocytes/Lentivirus/Alpha and Beta Chains of T-Cell Receptor (TCR) Specific for HIV-Gag/Intravenous Infusion

Tebas, Pablo; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; *A Phase I, Open Label, Dual Cohort, Single Center Study to Evaluate the Safety and Tolerability of Escalating Doses of Autologous T Cells Modified with Lentiviral Vectors Expressing High Affinity Gag-Specific TCRs in HLA-A*02 Patients with HIV*.

NIH/OBA Receipt Date: 4-22-08. Not Selected for RAC Public Review: 5-13-08

0804-922 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/CD 19+ B-Lymphoid Malignancies/Immunotherapy/In Vitro/Autologous T Lymphocytes/Sleeping Beauty (SB) Transposon/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Kebriaei, Partow; and Cooper, Laurence J. N; University of Texas MD Anderson Cancer Center; Houston, Texas; *Adoptive Immunotherapy for CD19+ B-Lymphoid Malignancies Using Sleeping Beauty Transposition to Express a CD19-Specific Chimeric Antigen Receptor in Autologous Ex Vivo Expanded T Cells*.

NIH/OBA Receipt Date: 4-21-08. Publicly Reviewed at the June 2008 RAC meeting

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0806-923 (Open; RAC reviewed with recommendations) Gene Therapy/Single Subject/Monogenic Disease/Hereditary Inclusion Body Myopathy-2 (HIBM2)/In Vivo/Cationic Liposome Complexes/DOTAP-Cholesterol/GNE cDNA/Intramuscular Injection

Nemunaitis, John J.; Mary Crowley Cancer Research Center; Dallas, Texas; *Compassionate Trial of Nanocomplex Mediated GNE Gene Replacement in Hereditary Inclusion Body Myopathy – 2*.

NIH/OBA Receipt Date: 6-27-08. Publicly Reviewed at the September 2008 RAC meeting

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0807-924 (Open) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Intravenous Infusion

Mitchell, Duane A.; Duke University Medical Center; Durham, North Carolina; *RE-START: REcurrent GBM Stem Cell Tumor Amplified RNA Immunotherapy Trial*.

NIH/OBA Receipt Date: 7-7-08. Not Selected for RAC Public Review: 7-28-08

0807-925 (Open) Gene Therapy/Phase I-II/Cancer/CEA Expressing Cancer/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta cDNAs Chains/Intravenous Infusion

Rosenberg, Steven A.; National Institutes of Health; Bethesda, Maryland; *Phase I/II Study of Metastatic Cancer that Expresses Carcinoembryonic Antigen (CEA) Using Lymphodepleting Conditioning Followed by Infusion of Anti-CEA TCR-Gene Engineered Lymphocytes*.

NIH/OBA Receipt Date: 7-11-08. Not Selected for RAC Public Review: 8-1-08

0807-926 (Open) Gene Therapy/Single Subject/Cancer/Lung Cancer/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/HER2-neu-Specific scFvFc-Zeta T Cell Receptor/Intravenous Injections

Brenner, Malcolm K. and Gottschalk, Stephen; Baylor College of Medicine; Houston, Texas; *Patient Specific Protocol for Administration of EBV Specific CTLs Expressing HER2/neu Chimeric Receptors for Lung Cancer*.

NIH/OBA Receipt Date: 7-11-08. Not Selected for RAC Public Review: 7-22-08

0807-927 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Primary Hepatocellular Carcinoma or Metastatic Colorectal Carcinoma in the Liver/Vector-Directed Cell Lysis/In Vivo/Vesicular Stomatitis Virus (VSV)/Tumor Lysis/Hepatic Arterial Delivery

Sung, Max W.; Mount Sinai School of Medicine; New York, New York; *Phase I Translational Trial of Oncolytic Virotherapy with Recombinant Vesicular Stomatitis Virus (rVSV(MΔ51)-M3) by Hepatic Arterial Delivery in Patients with Primary Hepatocellular Carcinoma or Metastatic Colorectal Carcinoma in the Liver*.

NIH/OBA Receipt Date: 7-14-08. Publicly Reviewed at the September 2008 RAC meeting

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0807-928 (Open) Gene Therapy/Phase I/Cancer/Cervical/Immunotherapy/In Vivo/Plasmid DNA/Vaccinia/HPV16+18 E6+E7 cDNA/Intramuscular Injection

Trimble, Cornelia L.; Sidney Kimmel Comprehensive Cancer Center, Johns Hopkins; Baltimore; Maryland; *A Phase I Efficacy and Safety Study of HPV16-Specific Therapeutic DNA-rVaccinia Vaccination in Combination with Topical Imiquimod in Patients with HPV16+ High Grade Cervical Dysplasia (CIN3)*.

NIH/OBA Receipt Date: 7-14-08. Not Selected for RAC Public Review: 8-5-08

0807-929 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Pro-Drug/In Vivo/Tropism-Modified Adenovirus/Serotype 5/Herpes Simplex Virus Thymidine Kinase cDNA/Somatostatin Receptor cDNA/Ganciclovir/Intraperitoneal Injection

Alvarez, Ronald D.; University of Alabama at Birmingham; Birmingham, Alabama; *A Phase I Study of Ad5.SSTWTK.RGD; a Tropism Modified Adenovirus Vector for Intraperitoneal Delivery of Therapeutic Genes and Additional Capability of Noninvasive Imaging of Gene Transfer in Patients with Recurrent Ovarian and Other Selected Gynecologic Cancers*.

NIH/OBA Receipt Date: 7-15-08. Not Selected for RAC Public Review: 8-5-08

0807-930 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Other Diseases-Disorders/Alzheimer's Disease/In Vivo/Adeno-Associated Virus/Serotype 2/Nerve Growth Factor cDNA/Intracranial Injection

Asien, Paul S.; University of California, San Diego; La Jolla, California; Watts, Ray L.; University of Alabama at Birmingham; Birmingham, Alabama; Turner, Raymond Scott; Georgetown University Medical Center; Washington, DC; Neugroschi, Judith; Mount Sinai School of Medicine; New York, New York; Grill, Joshua D. University of California, Los Angeles; Los Angeles, California; Burke, James R.; Duke University Medical Center; Durham, North Carolina; Sabbagh; Sun Health Research Institute, Banner Boswell Medical Center; Sun City, Arizona; Lerner, Alan J. Case Western Reserve University; Beachwood, Ohio; Rafii, Michael; University of California, San Diego; Mintzer, Jacobo E.; Medical University of South Carolina; North Charleston, South Carolina; Zamrini, Edward Y.; The University of Utah; Salt Lake City, Utah; and Lah, James J.; Emory University School of Medicine; Atlanta, Georgia; *A double-Blind, Placebo-Controlled (Sham Surgery), Randomized, Multicenter Study Evaluating CERE-110 Gene Delivery in Subjects with Mild to Moderate Alzheimer's Disease*. Sponsor: Ceregene.

NIH/OBA Receipt Date: 7-15-08. Publicly Reviewed at the September 2008 RAC meeting

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0807-931 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I-II/Monogenic Diseases/Pompe Disease/In Vivo/Adeno-Associated Virus Serotype 1/Acid-alpha Glycosidase Gene/Intramuscular Injections to the Diaphragm

Byrne, Barry J.; University of Florida College of Medicine; Gainesville, Florida; *Phase I/II Trial of Diaphragm Delivery of Recombinant Adeno-Associated Virus Acid Alpha-Glycosidase (rAAV1-CMV-GAA Gene Vector) in Patients with Pompe Disease*.

NIH/OBA Receipt Date: 7-15-08. Publicly Reviewed at the September 2008 RAC meeting

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0807-932 (Closed; RAC reviewed with recommendations) Gene Therapy/Phase I/Infectious Diseases/Hepatitis C/In Vivo/*Listeria monocytogenes*/HCV NS5BNS3 Consensus Sequence cDNA/Intravenous Injection

Lawitz, Eric J.; Alamo Medical Research; San Antonio, Texas; and Marbury, Thomas C; Orlando Clinical Research Center; Orlando, Florida; *A Randomized, Placebo-Controlled, Double-Blind, Dose-Escalation Study to Evaluate the Safety, Tolerability, and Pharmacodynamics of Multiple Intravenous Doses of ANZ-521 in Treatment-Naive Hepatitis C Patients*. Sponsor: Anza Therapeutics, Inc.

NIH/OBA Receipt Date: 7-16-08. Publicly Reviewed at the September 2008 RAC meeting

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0807-933 (Submission not complete) Gene Therapy/Phase III/Cancer/Malignant Glioma/Antisense/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Plasmid DNA/Electroporation/TGF-β2/Subcutaneous Injection

Pivotal Phase III Study of an Allogeneic Malignant Glioma Tumor Cell Vaccine for Subjects with World Health Organization (WHO) Stage IV Astrocytoma. Sponsor: NovaRx

NIH/OBA Receipt Date: 7-28-08.

0808-934 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Infectious Diseases/Hepatitis C/In Vivo/Cationic Liposome Complex/DOTIM-Cholesterol/No Transgene/Intravenous Injection

McHutchinson, John G.; Duke University Medical Center; Durham, North Carolina; *A Phase I, Open-label Study of the Safety, Tolerability, and Therapeutic Activity of JVRS-100 Cationic Lipid-DNA Complex in Patients with Chronic Hepatitis C Infection Who Relapsed After Receiving Interferon-Ribavirin Treatment*. Sponsor: Juvaris BioTherapeutics, Inc.

NIH/OBA Receipt Date: 8-04-08. Publicly Reviewed at the December 2008 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0808-935 (Open) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Autologous HIV RNA/Plasmids/CD40L cDNA/Intravenous Infusion

Jacobson, Jeffrey M.; Drexel University; Philadelphia, Pennsylvania; *A Phase II Study Testing the Activity and Safety of AGS-004 as an Immunotherapeutic in Successfully ART-Treated Subjects Infected with HIV-1 in Combination with ART Followed by ART Interruption*. Sponsor: Argos Therapeutics, Inc.

NIH/OBA Receipt Date: 8-04-08. Not Selected for RAC Public Review: 8-25-08

0808-936 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Leukemia/Immunotherapy/In Vivo/Cationic Liposome Complex/DOTIM-Cholesterol/No Transgene/Intravenous Injection

Claxton, David F.; Penn State Milton S. Hershey Medical Center; Hershey, Pennsylvania; *A Phase I Trial of the Immunostimulant JVRS-100 for the Treatment of Patients with Relapsed or Refractory Leukemia*. Sponsor: Juvaris BioTherapeutics, Inc.

NIH/OBA Receipt Date: 8-06-08. Publicly Reviewed at the December 2008 RAC meeting

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0808-937 (Open) Gene Therapy/Phase I/Cancer/MDS/AML/Immunotherapy/In Vitro/Allogeneic K562 Cells/Cationic Liposome Complexes/GM-CSF cDNA/Intradermal and Subcutaneous Injection

Ho, Vincent T.; Dana-Farber Cancer Institute, Harvard Medical School; Boston, Massachusetts; *Vaccination with Lethally Irradiated Autologous Myeloblast Admixed with Granulocyte Macrophage-Colony Stimulating Factor Secreting K562 Cells (GM-K562) in Patients with Advanced MDS or AML after Allogeneic Hematopoietic Stem Cell Transplantation*.

NIH/OBA Receipt Date: 8-13-08. Not Selected for RAC Public Review: 9-03-08

0808-938 (Open) Gene Therapy/Phase I/Cancer/NY-ESO or LAGE-1 Expressing Tumors/Immunotherapy/In Vivo/Canarypox Virus/NY-ESO-1, TRICOM cDNAs/Subcutaneous Injections

Bhardwaj, Nina; NYU Cancer Institute; New York, New York; and Odunsi, Kunle; Roswell Park Cancer Institute; Buffalo, New York; *Phase 1 Study of ALVAC(2)-NY-ESO- I (M)/TRICOM (vCP2292) in Patients with Epithelial Ovarian, Fallopian Tube or Primary Peritoneal Carcinoma Whose Tumors Express NY-ESO- 1 or LAGE-1 Antigen*.

NIH/OBA Receipt Date: 8-06-08. Not Selected for RAC Public Review: 9-22-08

0809-939 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta cDNAs Chains/Intravenous Infusion

Rosenberg, Steven A; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Melanoma Using a Chemoradiation Lymphodepleting Conditioning Regimen Followed by Infusion of Anti-Mart-1 and Anti-gp100 TCR-Gene Engineered Lymphocytes and Peptide Vaccines*.

NIH/OBA Receipt Date: 9-05-08. Not Selected for RAC Public Review: 9-26-08

0809-940 (Open) Gene Therapy/Phase I-II/Cancer/ B Cell Malignancies/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Rosenberg, Steven A; National Institutes of Health; Bethesda, Maryland; *Treatment of B Cell Malignancies with T Cells Expressing an Anti-CD19 Chimeric Receptor: Assessment of the Impact of Lymphocyte Depletion Prior to T Cell Transfer*.

NIH/OBA Receipt Date: 9-05-08. Not Selected for RAC Public Review: 9-26-08

0809-941 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/B-Cell Lymphoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/Chimeric Antigen Receptor-Kappa-CD28 Endodomain/Intravenous Injections

Brenner, Malcolm; Baylor College of Medicine; Houston, Texas; *Phase I Study of Adoptive Transfer of Autologous T Lymphocytes Engrafted with a Chimeric Antigen Receptor Targeting the Kappa Light Chain of Immunoglobulin Expressed in Patients with Chronic Lymphocytic Leukemia or B-Cell Lymphoma (CHARKALL)*.

NIH/OBA Receipt Date: 9-24-08. Not Selected for RAC Public Review: 10-15-08

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0810-942 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Oral Mucositis/Lactococcus lactis/Human Trefoil Factor 1 (hTFF1) cDNA/Oral Topical Application

Murphy, Barbara; Vanderbilt University; Nashville, Tennessee; Limaye, Sewanti; Dana Farber Cancer Institute; Boston, Massachusetts; Brennan, Michael; Carolinas Medical Center; Charlotte, North Carolina; Hu, Kenneth; Beth Israel Medical Center; New York, New York; Colevas, Alexander Dimitrios; Stanford University; Stanford, California; and Epstein, Joel B.; University of Illinois at Chicago; Chicago, Illinois; *A Phase Ib, Multi-Center, Single Blinded, Placebo-Controlled, Sequential Dose Escalation Study to Assess the Safety of Topically Applied AGO13 in Subjects Receiving Induction Chemotherapy for the Treatment of Cancers of the Head and Neck*. Sponsor: ActoGeniX

NIH/OBA Receipt Date: 10-06-08. Publicly Reviewed at the December 2008 RAC meeting

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0810-943 (Open) Gene Therapy/Phase I/Cancer/Mesothelioma or Pleural Malignancies/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interferon-alpha 2b/Intrapleural Administration

Sterman, Daniel H.; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; *A Pilot and Feasibility Trial of Repeated Dose Intrapleural Adenoviral-Mediated Interferon-alpha (Ad.hIFN-α2b) Gene Transfer for Malignant Mesothelioma*.

NIH/OBA Receipt Date: 10-06-08. Not Selected for RAC Public Review: 10-27-08

0810-944 (Open) Gene Therapy/Phase I/Infectious Disease/Cytomegalovirus (CMV) and Adenovirus Infections/In Vitro/EBV and CMV-Specific Cytotoxic T Lymphocytes/Adenovirus/CMV pp65 Gene/Intravenous

Bollard, Catherine M. and Martinez, Caridad; Barylor College of Medicine; Houston, Texas; *Adoptive Transfer of Cord Blood T cells to Prevent and Treat CMV and Adenovirus Infections after Transplantation*.

NIH/OBA Receipt Date: 10-07-08. Not Selected for RAC Public Review: 10-28-08

0810-945 (Open) Gene Therapy/Phase I/Cancer/Acute Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Krance, Robert; Dotti, Gianpietro; and Bollard, Catherine M.; Baylor College of Medicine; Houston, Texas; *Phase I/II Study of the Administration of Multi-virus-specific Cytotoxic T Lymphocytes (CTLs) Expressing CD19 Chimeric Receptors for Prophylaxis or Therapy of Relapse of Acute Lymphoblastic Leukemia Post Hemopoietic Stem Cell Transplantation (MultiPRAT)*.

NIH/OBA Receipt Date: 10-07-08. Not Selected for RAC Public Review: 10-28-08

0810-946 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Hepatocellular Carcinoma/Vector-Directed Cell Lysis/In Vivo/Vesicular Stomatitis Virus (VSV)/Tumor Lysis/Intratumoral Injection

Borad, Mitesh J.; Rakela, Jorge; and Vile, Richard G.; Mayo Clinic; Rochester, Minnesota; *Phase I Trial of Intratumoral Injection of Vesicular Stomatitis Virus Expressing Interferon Beta in Patients with Hepatocellular Carcinoma*.

NIH/OBA Receipt Date: 10-07-08. Publicly Reviewed at the December 2008 RAC meeting

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0810-947 (Open) Gene Therapy/Phase III/Cancer/Melanoma/Herpes Simplex Virus Type-1/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intratumoral Injection

Kaufman, Howard; Rush University Medical Center; Chicago, Illinois; Brett, Peter B.; Redwood Regional Medical Group; Sebastopol, California; Pennock, Gregory Keith; M. D. Anderson Cancer Center Orlando; Orlando, Florida; Guthrie, Troy H., Jr.; Baptist Cancer Institute; Jacksonville, Florida; Daniels, Gregory; UCSD Moores Cancer Center; LaJolla, California; Logan, Theodore F.; Indiana University School of Medicine; Indianapolis, Indiana; Reintgen, Douglas S.; Lakeland Regional Cancer Center; Lakeland, Florida; Amatruda, Thomas T., III; Hubert H. Humphrey Cancer Center; Fridley, Minnesota; Melnyk, Anton M.S., Jr.; Texas Cancer Center-Abilene; Abilene, Texas; Oleksowicz, Leslie; University of Cincinnati; Cincinnati, Ohio; Richards, Paul D.; Blue Ridge Cancer Care; Salem, Virginia; Senzer, Neil Nathan; Mary Crowley Cancer Research Center; Dallas, Texas; Watkins, David L.; Texas Oncology – Allison Cancer Center; Midland, Texas; Argarwala, Sanjiv; St. Luke's Hospital & Health Network; Bethlehem, Pennsylvania; Andtbacka, Robert; Huntsman Cancer Institute; Salt Lake City, Utah; Chesney, Jason, University of Louisville Medical Center; Louisville, Kentucky; Collichio, Frances A.; University of North Carolina at Chapel Hill; Chapel Hill, North Carolina; Curti, Brendan; Providence Portland Medical Center; Portland, Oregon; Rothschild, Neal; Palm Beach Cancer Institute; West Palm Beach, Florida; Whitman, Eric; Atlantic Melanoma Center; Morristown, New Jersey; Linette, Gerald; Washington University School of Medicine; St. Louis, Missouri; Kaufman, Howard L.; Mount Sinai Medical Center; New York, New York; Minor, David R.; San Francisco Oncology Associates and California Pacific Medical Center and Pacific Hematology Oncology Associates; San Francisco, California; Spitler, Lynn; St. Mary's Medical Center; San Francisco, California; Delman, Keith; Winship Cancer Institute; Atlanta, Georgia; Stewart, John H., IV; Wake Forest University School of Medicine; Winston-Salem, North Carolina; Abernethy, Amy P.; Duke Cancer Care Research Program; Durham, North Carolina; Cranmer, Lee D.; Arizona Cancer Center; Tucson, Arizona; Lewis, Karl; University of Colorado Denver; Aurora, Colorado; Wong, Michael K. Roswell Park Cancer Center; Buffalo, New York; Puzanov, Igor; Vanderbilt-Ingram Cancer Center; Nashville, Tennessee; Gabrail, Nashat Y.; Gabrail Cancer Center Research; Canton, Ohio; Schultz, Stephen M.; Investigative Clinical Research of Indiana, LLC; Indianapolis, Indiana; Lutzky, Jose; Boca Raton Comprehensive Cancer Center; Boca Raton, Florida; Borden, Ernest C. The Cleveland Clinic Foundation; Cleveland, Ohio; Richards, Jon; Oncology Specialists, S.C.; Park Ridge, Illinois; Stephenson, Joe J., Jr.; Institute for Translational Oncology Research; Greenville, South Carolina; Wargo, Jennifer; Massachusetts General Hospital; Boston, Massachusetts; Taback, Bret; Columbia University Medical Center; New York, New York; Shirai, Keisuke; Medical University of South Carolina; Charleston, South Carolina; Treisman, Jonathan; Aurora St. Luke's Medical Center; Milwaukee, Wisconsin; Amatruda, Thomas; Hubert H. Humphrey Cancer Center; Robbinsdale, Minnesota; Richards, Paul D.; Blue Ridge Cancer Care; Salem, Virginia; Cohen, Mark Steven; University of Kansas Medical Center; Kansas City, Kansas; Glaspy, John A.; University of California, Los Angeles; Los Angeles, California; Lutzky, Jose; Mount Sinai Comprehensive Cancer Center; Miami Beach, Florida; Noyes, R. Dirk; Intermountain Medical Center; Murray, Utah; Vezeridis, Michael P.; Rhode Island Hospital; Providence, Rhode Island; Senzer, Neil Nathan; Mary Crowley Cancer Research Center; Dallas, Texas; Feeney, Kendra J.; Thomas Jefferson University; Philadelphia, Pennsylvania; Hauke, Ralph; Nenraska Methodist Hospital; Omaha, Nebraska; McWilliams, Robert R.; Mayo Clinic; Rochester, Minnesota; Pendergrass, Kelly B.; Kansas City Cancer Center; Kansas City, Missouri; Ross, Merrick I.; The University of Texas MD Anderson Cancer Center; Houston, Texas; Milhem, Mohammed; The University of Iowa; Iowa City, Iowa; Khushalani, Nikhil I.; Roswell Park Cancer Center; Buffalo, New York; Saenger Yvonne; Mount Sinai School of Medicine; New York, New York; Venigalla, Madhavai L.; Lakeland Regional Cancer Center; Lakeland, Florida; Friedlander, Philip; Mount Sinai School of Medicine; New York, New York; Hutchins, Laura; University of Arkansas for Medical Sciences; Little Rock, Arkansas; Schultz, Stephen M.; Investigative Clinical Research of Indiana, LLC; Indianapolis, Indiana; Lee, Wes; Redwood Regional Medical Group; Santa Rosa, California; Hsueh, Eddy C; St. Louis University; St. Louis, Missouri; and Zager, Jonathon Scott; H. Lee Moffitt Cancer Center & Research Institute; Tampa, Florida; *A Randomized Phase 3 Clinical Trial to Evaluate the Efficacy and Safety of a Treatment with OncoVEX^{GM-CSF} Compared to Subcutaneously Administered GM-CSF in Previously Treated Melanoma Patients with Unresectable Stage IIIb, IIIc and IV Disease*. Sponsor: Biovex, Inc.

NIH/OBA Receipt Date: 10-07-08. Not Selected for RAC Public Review: 10-28-08

0810-948 (Open) Gene Therapy/Phase I/Other Disorders/Macular Degeneration/In Vivo/Adeno-Associated Virus 2/sFLT01 cDNA/Intravitreal Injection

Flotte, Terence R.; University of Massachusetts Medical School; Worcester, Massachusetts; Heier, Jeffrey S.; Boston, Massachusetts; Campochiario, Peter A.; Johns Hopkins University; Baltimore, Maryland; and Dugel Pravin U.; Retinal Consultants of Arizona; Phoenix, Arizona; *A Phase 1, Open-Label, Multi-Center, Dose Escalating, Safety and Tolerability Study of a Single Intravitreal Injection of AAV2-sFLT01 in Patients With Neovascular Age-Related Macular Degeneration*. Sponsor: Genzyme Corporation

NIH/OBA Receipt Date: 10-07-08. Not Selected for RAC Public Review: 10-28-08

0810-949 (Open) Gene Therapy/Phase I/Cancer/Renal Cancer/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta cDNAs Chains/Intravenous Infusion

Yang, James C.; National Institutes of Health; Bethesda, Maryland; *Treatment of Patients with Metastatic Renal Cancer with T-cells Transduced with a T Cell Receptor which Recognizes TRAIL Bound to the DR4 Receptor*.

NIH/OBA Receipt Date: 10-07-08. Not Selected for RAC Public Review: 10-28-08

0810-950 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/In Vitro/Autologous CD34+ Cells from Bone Marrow/Retroviral Vector/γc cDNA/Intravenous Infusion

Notarangelo, Luigi; Harvard Medical School; Boston Massachusetts; Kohn, Donald B.; University of California at Los Angeles; Los Angeles, California; and Filipovich, Alexandra H.; Cincinnati Children's Hospital Medical Center; Cincinnati, Ohio; *Gene Therapy for SCID-X1 Using a Self-Inactivating (SIN) Gammaretroviral Vector*.

NIH/OBA Receipt Date: 10-07-08. Publicly Reviewed at the December 2008 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0810-951 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Carcinoma/Immunotherapy/In Vivo/Saccharomyces cerevisiae/CEA cDNAs/Subcutaneous Injections

Gulley, James L.; National Institutes of Health; Bethesda, Maryland; *An Open Label Phase I Study to Evaluate the Safety and Tolerability of a Vaccine Consisting of Whole, Heat-Killed Recombinant Saccharomyces cerevisiae (Yeast) Genetically Modified to Express CEA Protein in Adults with Metastatic CEA-Expressing Carcinoma*.

NIH/OBA Receipt Date: 10-07-08. Not Selected for RAC Public Review: 10-28-08

0810-952 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Fludarabine-Refractory and/or del(17p) Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous Leukemic Cells/Adenovirus/Serotype 5/CD154 cDNA/Intravenous Infusion

Castro, Januario E.; University of California, San Diego; San Diego, California; Beck, J. Thaddeus; Highland Oncology; Fayetteville, Arizona; Schwartzberg, Lee S.; and The West Clinic; Memphis, Tennessee; Pinilla-Ibarz; H. Lee Moffitt Cancer Center; Tampa, Florida; *Phase Ib Study of Autologous Ad-ISF35-Transduced CLL B Cells and Fludarabine, Cyclophosphamide, and Rituximab (FCR) in Subjects with Fludarabine-Refractory and/or del(17p) Chronic Lymphocytic Leukemia (CLL)*. Sponsor: Memgen.

NIH/OBA Receipt Date 10-07-08. Publicly Reviewed at the June 2010 RAC meeting

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0810-953 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Emens, Leisha A.; Johns Hopkins University School of Medicine; Baltimore, Maryland; *A Safety and Bioactivity Study of Combination Therapy with Trastuzumab, Cyclophosphamide, and an Allogeneic GM-CSF-secreting Breast Tumor Vaccine for the Treatment of Patients with High Risk/Metastatic HER-2/neu-Overexpressing Breast Cancer with No Evidence of Disease*.

NIH/OBA Receipt Date: 10-14-08. Not Selected for RAC Public Review: 11-04-08

0810-954 (Open) Gene Therapy/Phase II/Other/Amyotrophic Lateral Sclerosis/In Vivo/Plasmid DNA/ZPF-TF cDNA (Zinc Finger DNA Binding Protein)/Intramuscular Injection

Maragakis, Nicholas; Johns Hopkins University School of Medicine; Baltimore, Maryland; Katz, Jonathan; California Pacific Medical Center; San Francisco, California; and Barohn, Richard; Landon Center on Aging; Kansas City, Kansas; *A Phase 2 Repeat Dosing Clinical Trial of SB-509 in Subjects with Amyotrophic Lateral Sclerosis*. Sponsor: Sangamo BioSciences, Inc.

NIH/OBA Receipt Date: 10-27-08. Not Selected for RAC Public Review: 11-17-08

0811-955 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other/Pain in Diabetic Neuropathy/In Vivo/Herpes Simplex Virus Type 1/Human Glutamic Acid Decarboxylase 67 (GAD67) Gene/Subcutaneous Inoculation

Fink, David J.; University of Michigan; Ann Arbor, Michigan; *HSV Gene Transfer of GAD for Painful Diabetic Neuropathy*. Sponsor: Diamyd, Inc.

NIH/OBA Receipt Date: 11-19-08. Publicly Reviewed at the March 2009 RAC meeting

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0811-956 (Open) Gene Therapy/Phase I/Immunotherapy/Cancer/Breast/In Vitro/Autologous Tumor Cells/Lethally Irradiated/Adenovirus/Serotype 5/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) cDNA/Subcutaneous and Intradermal Injection

Overmoyer, Beth; Dana-Farber Cancer Institute; Boston, Massachusetts; A Phase lb Study Of Autologous Vaccination With Lethally Irradiated, Autologous Breast Cancer Cells Engineered By Adenoviral Mediated Gene Transfer To Secrete GM-GSF Following Preoperative Chemotherapy In Women With Operable Breast Cancer.

NIH/OBA Receipt Date: 10-27-08. Not Selected for RAC Public Review: 11-17-08

0812-957 (Open) Non-therapeutic (Healthy Volunteers)/Immunotherapy/In Vivo/Vaccinia Virus/Human Papilloma Virus E6 and E7/Interleukin-2/Intramuscular Injection

Rudin, Dan; Charles River Clinical Services Northwest; Tacoma, Washington; and Morrison, Royce; Comprehensive Clinical Development NW, Inc.; Tacoma, Washington; *A Phase I Trial of R05217790, a Human Papilloma Virus 16 Targeted Immunotherapy, in Healthy Adult Subjects*. Sponsor: Hoffmann-La Roche, Inc.

NIH/OBA Receipt Date: 12-19-08. Not Selected for RAC Public Review: 01-22-09

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0812-958 (Open) Gene Therapy/Phase II/Cancer/Cervical Intraepithelial Neoplasia (CIN)/Immunotherapy/In Vivo/Vaccinia Virus/Human Papilloma Virus E6 and E7/Interleukin-2/Intramuscular Injection

Garcia, Francisco A. R.; University of Arizona College of Medicine; Tucson, Arizona; Feldman, Robert A.; Miami Research Associates; Miami, Florida; Ackerman, Ronald T.; Comprehensive Clinical Trials, LLC and Advanced Women's Healthcare; West Palm Beach, Florida; Parker, Robert Lamar, Jr.; Lyndhurst Clinical Research; Winston-Salem, North Carolina; Harris, Micah S.; Women's Health Research; Phoenix, Arizona; Nicholoson, C. Scott; York Clinical Consulting and Westbank Women's Health; Marrero, Louisiana; Wentworth, Jeffrey; The Group for Women and Tidewater Physicians for Women; Norfolk, Virginia; Abdelsayed, Nader Y.; Centennial Hills OB-GYN; North Las Vegas, Nevada; Ault, Kevin A.; Emory University School of Medicine; Atlanta, Georgia; Fine, Paul M.; Planned Parenthood of Houston and Southeast Texas; Houston, Texas; :Huey, James; HWC Women's Research Center; Englewood, Ohio; Mabey, R. Garn, Jr; Las Vegas, Nevada; Romaguera, Josefina; University District Hospital Medical Center; San Juan, Puerto Rico; Strafford, James C.; Holzer Clinic, Inc.; Gallipolis, Ohio; Swenson, Karen; Women's Partners in Health; Austin, Texas; Fehnel, Stephen H.; West Reading, Pennsylvania; Harper, Diane M.; Truman Medical Center – Lakewood; Kansas City, Missouri; Shields, Ruth L.; Coastal Clinical Research, Inc.; Mobile, Alabama; Ferris, Daron; Medical College of Georgia; Augusta, Georgia; Michelson, Jeffrey A.; Eastern Carolina Women's Center; New Bern, North Carolina; Goldberg, Cynthia C.; Visions Clinical Research-Tucson; Tucson, Arizona; Lucci, Joseph A, III; University of Miami; Miami, Florida; Lederman, Samuel; Altus Research; Lake Worth, Florida; Swor, Michael; Physician Care Clinical Research, LLC; Sarasota, Florida; Wilson, Wayne B.; Research Associates Rio Grande Valley, Inc.; McAllen, Texas; Vasilev, Steven A.; Kaiser Permanente Medical Group; Los Angeles, California; Jacobs, Corey D.; Wilmax Clinical Research, Inc. Mobile, Alabama; Malloy, Tyrone C.; Soapstone Center for Clinical Research; Decatur, Georgia; Wininger, Steven Joel; Precision Trials; Phoenix, Arizona; Cohen, Jay S.; Discovery Clinical Research, Inc.; Plantation, Florida; Twede, Michael L.; Salt Lake Women's Center, PC; Sandy, Utah; Morales-Ramirez; San Juan, Puerto Rico; Swanson, Stephen G., Women's Clinic of Lincoln, P.C.; Lincoln, Nebraska; Sussman, Steven A.; Lawrence OB-GYN Associates, P.C.; Lawrenceville, New Jersey; Kirkpatrick, Helena P.; Magnolia OB/GYN Research Center; Myrtle Beach, South Carolina; Bruck, Lance, R.; Stamford Hospital; Stamford, Connecticut; Gomez-Lobo, Veronica; Washington Hospital Center; Washington, DC; Goldberg, David; MetroWest Medical Center; Framingham, Massachusetts; Schwartz, Benjamin, M.; Island Gynecologic Oncology; Brightwaters, New York; and Landrum, Lisa; University of Oklahoma Health Sciences Center; Oklahoma City, Oklahoma; *A Randomized, Double Blind, Placebo Controlled, Parallel Group, Multicenter Study of the Safety and Response Rate of 3 Subcutaneously Administered Doses of 5 x 10^7 pfu R05217790 in Patients with High Grade Cervical Intraepithelial Neoplasia Grade 2 or 3 Associated with High Risk HPV Infection*. Sponsor: Hoffmann-La Roche, Inc.

NIH/OBA Receipt Date: 12-19-08. Not Selected for RAC Public Review: 01-22-09

0812-959 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Le, Dung; Johns Hopkins University; Baltimore, Maryland; *A Phase Ib Trial Evaluating the Safety and Feasibility of Ipilimumab (BMS-734016) Alone or in Combination with Allogeneic Pancreatic Tumor Cells Transfected with a GM-CSF Gene for the Treatment of Locally Advanced, Unresectable or Metastatic Pancreatic Adenocarcinoma*.

NIH/OBA Receipt Date: 12-22-08. Not Selected for RAC Public Review: 01-16-09

0901-960 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Vector-Directed Cell Lysis/In Vivo/Adenovirus/Type 5-3/Replication-Competent Virus/Intraperitoneal Injection

Alvarez, Ronald D.; University of Alabama at Birmingham; Birmingham, Alabama; *A Phase I Study of a Tropism Modified Conditionally Replicative Adenovirus Vector (Ad5/3-Delta24) for Intraperitoneal Delivery in Patients With Recurrent Ovarian and Other Selected Gynecologic Cancers*.

NIH/OBA Receipt Date: 01-06-09. Not Selected for RAC Public Review: 07-30-09

0901-961 (Closed) Gene Therapy/Phase I/Cancer/Advanced Solid Tumors/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Interleukin-12 cDNA/RheoSwitch® Therapeutic System/Intratumoral Injection

Antonia, Scott J.; H. Lee Moffitt Cancer Center; Tampa, Florida; *A Phase 1 Open Label Trial to Evaluate the Safety, Tolerance, Transgene Function, and Immunological Effects of an Intratumoral Injection of Adenoviral Transduced Autologous Dendritic Cells Engineered to Express hIL-12 in Subjects With Advanced Solid Tumors*. Sponsor: Intrexon Transcriptional Therapeutics

NIH/OBA Receipt Date: 01-06-09. Not Selected for RAC Public Review: 01-28-09
Closed: 03-21-11, never activated, no enrollment

0901-962 (Open) Gene Transfer/Phase I/Other Diseases-Disorders/Parkinson's Disease/In Vivo/Adeno-Associated Virus/Glial Cell Line-Derived Neurotrophic Factor (GDNF) cDNA/Intracranial Administration

Losner, Russell R.; National Institutes of Health; Bethesda, Maryland; *A Phase 1 Open-Label Dose Escalation Safety Study of Convection-Enhanced Delivery (CED) of Adeno-Associated Virus Encoding Glial Cell Line-Derived Neurotrophic Factor (AAV2-GDNF) in Subjects with Advanced Parkinson's Disease*.

NIH/OBA Receipt Date: 01-06-09. Not Selected for RAC Public Review: 01-28-09

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0901-963 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/In Vitro/Autologous CD34+ Cells From Bone Marrow/Lentiviral Vector/γc cDNA/Intravenous Infusion

Sorrentino, Brian P.; St. Jude Children's Research Hospital; Memphis, Tennessee; *A Pilot Feasibility Study of Gene Transfer for X-Linked Severe Combined Deficiency in Newly Diagnosed Infants Using A Self-Inactivating Lentiviral Vector to Transduce Autologous CD34+ Hematopoietic Cells*.

NIH/OBA Receipt Date: 01-06-09. Publicly Reviewed at the March 2009 RAC meeting

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0901-964 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/X-Linked Severe Combined Immune Deficiency/In Vitro/Autologous CD34+ Cells/Lentiviral Vector/γc cDNA/Intravenous Infusion

DeRavin, Suk See; Kang, Elizabeth M.; and Malech, Harry; National Institutes of Health; Bethesda, Maryland; *Lentiviral Gene Transfer for Treatment of Children Older Than One Year of Age with X-Linked Severe Combined Immunodeficiency*.

NIH/OBA Receipt Date: 01-06-09. Publicly Reviewed at the March 2009 RAC meeting

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0901-965 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/anti-PSMA-Zeta T Cell Receptor/Intravenous Infusion

Slovin, Susan F.; Memorial Sloan-Kettering Cancer Center; New York, New York; *Adoptive Transfer Of Autologous T Cells Targeted To Prostate Specific Membrane Antigen (PSMA) For The Treatment Of Castrate Metastatic Prostate Cancer (CPMC)*.

NIH/OBA Receipt Date: 01-07-09. Not Selected for RAC Public Review: 01-28-09

0901-966 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other Diseases Disorders/Diabetic Ulcer/In Vitro/Linear Plasmid/Cathelicidin (hCAP-18/LL-37)/Skin Graft Tissue

Schurr, Michael J.; University of Wisconsin School of Medicine and Public Health; Madison, Wisconsin; *A Prospective, Randomized, Controlled, Multicenter, Unblinded, Safety and Early Efficacy Trial of ExpressGraftenhance Skin Tissue Versus Wet to Dry Dressings in the Treatment of Recently Occurring, Non-Infected, Foot Ulcers in Diabetic Patients*. Sponsor: Stratatech Corporation

NIH/OBA Receipt Date: 01-06-09. Publicly Reviewed at the March 2009 RAC meeting

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0901-967 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Ovarian/Immunotoxin/In Vivo/Plasmid/Diphtheria Toxin A Chain (DT-A)/H19 Promoter/Intraperitoneal Administration

Coukos, George; University of Pennsylvania Medical Center; Philadelphia, Pennsylvania; *Phase 1/2a, Dose-Escalation, Safety, Pharmacokinetic, and Preliminary Efficacy Study of Intraperitoneal Administration of DTA-H19 in Subjects with Advanced Stage Ovarian Cancer*. Sponsor: BioCancell Therapeutics

NIH/OBA Receipt Date: 01-07-09. Publicly Reviewed at the March 2009 RAC meeting

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0902-968 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/RNA Transfer/Prostate, Prostate Cancer Specific Antigens/Intradermal Injection

Vieweg, Johannes; University of Florida; Gainesville, Florida; *Phase I/IIa Study of RNActive®-Derived Therapeutic Vaccine in Metastatic Hormone Refractory Prostate Cancer*.

NIH/OBA Receipt Date: 02-02-09. Not Selected for RAC Public Review: 02-26-09

0903-969 (Open) Gene Therapy/Phase I/Cancer/Osteosarcoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/Chimeric Antigen Receptor-HER 2-CD28 Endodomain/Intravenous Injection

Ahmed, Nabil; Baylor College of Medicine, Texas Children's Hospital; Houston, Texas; *Administration of HER2 Chimeric Antigen Receptor Expressing T Cells for Subjects with Advanced Osteosarcoma*.

NIH/OBA Receipt Date: 03-03-09. Not Selected for RAC Public Review: 03-25-09

0903-970 (Open) Gene Therapy/Cancer/Glioblastoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/Chimeric Antigen Receptor-HER 2-CD28 Endodomain/Intravenous Injection

Ahmed, Nabil; Baylor College of Medicine, Texas Children's Hospital; Houston, Texas; *Administration of HER2 Chimeric Antigen Receptor Expressing CMV-Specific Cytotoxic T Cells in Patients with Glioblastoma Multiforme*.

NIH/OBA Receipt Date: 03-03-09. Not Selected for RAC Public Review: 03-25-09

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0903-971 (Open) Gene Therapy/Phase I/Cancer/Lung/Immunotherapy/In Vitro/Autologous TGFβ-Resistant T Lymphocytes/Retrovirus/ Chimeric Antigen Receptor-HER 2-CD28 Endodomain/Dominant Negative TGFβ Receptor II (DNRII)/Intravenous Injection

Gottschalk, Stephen; Baylor College of Medicine, Texas Children's Hospital; Houston, Texas; *Administration of HER2 Chimeric Receptor and TGFβ Dominant Negative Receptor (DNR) Expressing EBV Specific Lymphocytes for Subjects with Advanced HER2 Positive Lung Malignancy (HERCREEM)*.

NIH/OBA Receipt Date: 03-03-09. Not Selected for RAC Public Review: 03-25-09

0903-972 (Open) Gene Therapy/Phase I/Cancer/Head and Neck (SCHN)/Antisense/Naked Plasmid DNA/EGFR Antisense DNA/Intratumoral Injection

Argiris, Athanossios; University of Pittsburgh Medical Center; Pittsburgh, Pennsylvania; *Safety and Efficacy Evaluation of Radiation and Cetuximab plus Intratumoral EGFR Antisense DNA in Elderly or Cisplatin-ineligible Patients with Locally Advanced Head and Neck Squamous Cell Carcinoma*.

NIH/OBA Receipt Date: 03-05-09. Not Selected for RAC Public Review: 03-26-09

0903-973 (Closed) Gene Therapy/Phase I/Cancer/Glioblastoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/ Chimeric Antigen Receptor-HER 2-CD28 Endodomain/Intratumoral Injection

Ahmed, Nabil; Baylor College of Medicine, Texas Children's Hospital; Houston, Texas; *Phase I/II Study in Subjects with Recurrent and Resistant Glioblastoma Multiforme (GBM) of Intracavitary CMV–specific Cytotoxic T Lymphocytes (CTL) Expressing Chimeric Antigen Receptors Targeting HER2 (GLITCHER)*.

NIH/OBA Receipt Date: 03-18-09. Not Selected for RAC Public Review: 04-09-09
Closed: 04/24/2012

0903-974 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/CEA-Expressing Malignancies/In Vivo/Adenovirus/Carcinoembryonic Antigen cDNA/Subcutaneous Injection

Morse, Michael A.; Duke University Medical Center; Durham, North Carolina; *A Phase I/II Study of Active Immunotherapy with Ad5 [E1-, E2b-] CEA(6D) Vaccine (ETBX-011) in Patients with Advanced or Metastatic Malignancies Expressing CEA*.

NIH/OBA Receipt Date: 03-27-09. Not Selected for RAC Public Review: 04-17-09

0904-975 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vivo/Autologous Dendritic and Langerhans Cells/Lentivirus/HIV-1HXB2 gag, truncated pol, vpr, rev, tat, nef, and Envelope Proteins/Subcutaneous Injection

Fischl, Margaret; University of Miami School of Medicine; Miami, Florida; *A Phase I Dose-escalation Clinical Trial to Evaluate the Safety and Immunogenicity of a Replication-Defective HIV1 Vaccine (HIVAX^{TM}) in HIV-1 Infected Subjects Receiving Highly Active Antiretroviral Therapy*. Sponsor: GeneCure Biotechnologies

NIH/OBA Receipt Date: 04-13-09. Publicly Reviewed at the June 2009 RAC meeting

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0904-976 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Pro-drug/Replication Competent Retrovirus/In Vivo/Yeast 1 Cytosine Deaminase cDNA/5-Flurocytosine/Intratumoral (Stereotactic) Injection

Aghi, Manish; University of California, San Francisco; San Francisco, California; Vogelbaum, Michael A.; Cleveland Clinic Foundation; Cleveland, Ohio; Kesari, Santosh; University of San Diego; La Jolla, California; and Mikkelsen, Tom; Henry Ford Health System; Detroit, Michigan; *A Phase I Ascending Dose Trial of the Safety and Tolerability of Toca 511 in Patients with Recurrent Glioblastoma Multiforme*. Sponsor: Tocagen Inc.

NIH/OBA Receipt Date: 04-16-09. Publicly Reviewed at the June 2009 RAC meeting

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0904-977 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/Late Infantile Neuronal Ceroid Lipofuscinoses/In Vivo/Brain/Adeno-Associated Virus 10/CLN2 cDNA/Intraparenchymal Injection

Crystal, Ronald G.; Weill Cornell Medical College; New York, New York; *Direct CNS Administration of a Replication Deficient Adeno-Associated Virus Gene Transfer Vector Serotype rh.10 Expressing the Human CLN2 cDNA to Children with Late Infantile Neuronal Ceroid Lipofuscinosis*.

NIH/OBA Receipt Date: 04-17-09. Publicly Reviewed at the June 2009 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0904-978 (Open) Gene Therapy/Phase I/Cancer/Pancreatic/Immunotoxin/In Vivo/Plasmid/Diphtheria Toxin A Chain (DT-A)/H19 Promoter/Intratumoral Administration

Roberts, John D.; Virginia Commonwealth University; Richmond, Virginia; Hanna, Nader; University of Maryland Medical Center; Baltimore, Maryland; and Czerniak, Abraham; The Chaim Sheba Medical Center; Tel Hashomer, Israel; *Phase 1/2a, Dose-Escalation, Safety, Pharmacokinetic, and Preliminary Efficacy Study of Intratumoral Administration of DTA-H19 in Patients with Unresectable Pancreatic Cancer*. Sponsor: BioCancell Therapeutics

NIH/OBA Receipt Date: 04-20-09. Not Selected for RAC Public Review: 05-13-09

0904-979 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag cDNA/Interleukine-12 cDNA/Intramuscular Injection via Electroporation

Kalams, Spyros; Vanderbilt University; Nashville, Tennessee; *A Phase I Clinical Trial to Evaluate the Safety and Immunogenicity of PENNVAX-B (gag, pol, env) Vaccine, with or without IL-12 DNA Plasmid, Delivered via Electroporation in Healthy, HIV-1 Uninfected Adult Participants*. Sponsor: HVTN

NIH/OBA Receipt Date: 04-21-09. Not Selected for RAC Public Review: 05-12-09

0904-980 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vitro/Lentivirus/a-mesothelin-scFv with Signaling Domains Comprised of TCRζ, CD28, and 4-1BB cDNA/Intravenous or Intratumoral Injection

Chu, Christina; University of Pennsylvania; Philadelphia, Pennsylvania; *Phase I Clinical Trial of Autologous Mesothelin Redirected T Cells Administered by Intravenous or Intratumoral Injection in Ovarian Cancer Patients*.

NIH/OBA Receipt Date: 04-22-09. Not Selected for RAC Public Review: 05-13-09

0904-981 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I-II/Other Diseases-Disorders/Parkinson's Disease/In Vivo/ Adeno-Associated Virus/Neurturin [NTN] cDNA/Intraputaminal and Intrastriatal Administration

Marks, William J.; University of California, San Francisco; San Francisco, California; Stacy, Mark; Duke University; Durham, North Carolina; Cho, Catherine; Mount Sinai School of Medicine; New York, New York; Boulis, Nicholas; Emory University; Atlanta, Georgia; Watts, Ray L.; University of Alabama at Birmingham; Birmingham, Alabama; Jankovic, Joseph; Baylor College of Medicine; Houston, Texas; Goetz, Christopher G.; Rush University Medical Center; Chicago, Illinois; Ford, Blair; Columbia University; New York, New York; Henderson, Jamie MacDonald; Stanford University School of Medicine; Stanford, California; Severt, William Lawrence; Beth Israel Medical Center; New York, New York; and Stern, Matthew B.; University of Pennsylvania Health System; Philadelphia, Pennsylvania; *A Phase 1/2 Trial Assessing the Safety and Efficacy of Bilateral Intraputaminal and Intranigral Administration of CERE-120 (Adeno-Associated Virus Serotype 2 [AAV2]-Neurturin [NTN]) in Subjects with Idiopathic Parkinson's Disease*. Sponsor: Ceregene, Inc.

NIH/OBA Receipt Date: 04-22-09. Publicly Reviewed at the June 2009 RAC meeting

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0905-982 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Emens, Leisha A.; Johns Hopkins School of Medicine; Baltimore, Maryland; *A Randomized, Open-Label Comparative Study of Combination Therapy with Cyclophosphamide and an Allogeneic GM-CSF-secreting Breast Tumor Vaccine With or Without Trastuzumab for the Treatment of Metastatic Breast Cancer that Does Not Over-express HER-2/neu*.

NIH/OBA Receipt Date: 05-01-09. Not Selected for RAC Public Review: 05-21-09

0906-983 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/Autologous Dendritic Cells/RNA Transfer/MART-1, Tyrosinase, gp100, MAGE-3 cDNAs/GITR-L, CTLA-4 cDNAs/Intranodal Injections

Pruitt, Scott K.; Duke University; Durham, North Carolina; *Local Modulation of Immune Receptor Function to Enhance Immune Responses to Dendritic Cell Vaccination in Subjects with Metastatic Melanoma*.

NIH/OBA Receipt Date: 06-25-09. Not Selected for RAC Public Review: 07-15-09

0906-984 (Open) Gene Therapy/Phase I-II/Cancer/Cervical/Immunotherapy/In Vivo/Plasmid DNA/HPV 16 E7 cDNA/Intramuscular and Intralesional Injection

Trimble, Cornelia; Johns Hopkins Medical Institutions; Baltimore, Maryland; and Alvarez, Ronald D.; University of Alabama at Birmingham; Birmingham, Alabama; *A Pilot Study of pNGVL4a-CRT/E7(detox) for the Treatment of Patients with HPV16+ Cervical Intraepithelial Neoplasia 2/3 (CIN2/3)*.

NIH/OBA Receipt Date: 06-23-09. Not Selected for RAC Public Review: 07-17-09

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0907-985 (Open) Gene Therapy/Phase I/Cancer/Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Brentjens, Renier; Memorial Sloan-Kettering Cancer Center, New York, New York; *A Phase I Trial of Precursor B Cell Acute Lymphoblastic Leukemia (B-ALL) Treated with Autologous T Cells Genetically Targeted to the B Cell Specific Antigen CD19*.

NIH/OBA Receipt Date: 07-13-09. Not Selected for RAC Public Review: 08-03-09

0907-986 (Open) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Vaccinia (MVA)/HER2 cDNA/Subcutaneous Injection

Guardino, Alice; Stanford University; Stanford, California; Cassidy, Michael; Alta Bates Summit Comprehensive Cancer Center; Berkeley, California; and Fisher, George A.; Stanford Cancer Center; Stanford, California; *A Phase I Safety and Immunogenicity Trial of MVA-BN®HER2 Vaccine in HER-2-Positive Breast Cancer Patients Following Adjuvant Therapy*. Sponsor: BN ImmunoTherapeutics, Inc.

NIH/OBA Receipt Date: 07-13-09. Not Selected for RAC Public Review: 08-03-09

0907-987 (Open) Gene Therapy/Phase I/Cancer/Head and Neck or Solid Tumors/Pro-Drug/Fludarabine Monophosphate/In Vivo/Adenovirus/Serotype 5/E. coli Purine Nucleoside Monophosphate (PNP) cDNA/Intratumoral Injection

Rosenthal, Eben; University of Alabama at Birmingham; Birmingham, Alabama; and Yarbrough, Wendell G.; Vanderbilt Ingram Cancer Center; Nashville, Tennessee; *A Phase I Study of Ad/PNP-F-araAMP a First-Generation Replication Defective Adenoviral Vector (E1a and E3 Deleted) Expressing E. coli Purine Nucleoside Phosphorylase (PNP) Administered in Combination with Fludarabine Monophosphate (F-araAMP) to Patients with Locally-Invasive Head and Neck Cancer and Other Solid Tumors*. Sponsor: PNP Therapeutics, Inc.

NIH/OBA Receipt Date: 07-13-09. Not Selected for RAC Public Review: 08-03-09

0907-988 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Non-Therapeutic (Healthy Volunteers)/Respiratory Illness/Human Rhinovirus (RG-HRV16)/Intranasal Administration

Gern, James; University of Wisconsin School of Medicine and Public Health; Madison, Wisconsin; *A First-in-Human Safety and Dose-Finding Study of a New Type-16 Human Rhinovirus (RG-HRV16) Inoculum in Healthy Volunteers*.

NIH/OBA Receipt Date: 07-14-09. Publicly Reviewed at the September 2009 RAC meeting

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0907-989 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Familial Adenomatous Polyposis (FAP)/E. coli/β-Catenin shRNA/Oral Administration

Steinbach, Gideon; Seattle Cancer Care Alliance; Seattle, Washington; and Chung, Daniel C.; Massachusetts General Hospital; Boston, Massachusetts; *A Phase I Open-Label, Escalating-Dose Study, of the Safety and Tolerability of Single Daily Doses of CEQ50B, an RNAi-Based Therapy for Familial Adenomatous Polyposis*. Sponsor: Cequent Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 07-14-09. Publicly Reviewed at the September 2009 RAC meeting

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0907-990 (Open) Gene Therapy/Phase I/Cancer/Malignant Glioma/Pro-Drug/Valacyclovir/In Vivo/Adenovirus/Serotype 5/Herpes Simplex Thymidine Kinase cDNA/Cytokine/Flt3L cDNA/Intratumoral Injection

Chiocca, E. Antonio; Ohio State University; Columbus, Ohio; and Tatter, Stephen B; Wake Forest University; Winston-Salem, North Carolina; *A Non-randomized, Open-label Dose-Finding Trial of Combined Cytotoxic and Immune-Stimulatory Strategy for the Treatment of Resectable Primary Malignant Glioma*.

NIH/OBA Receipt Date: 07-14-09. Not Selected for RAC Public Review: 08-04-09

0907-991 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Hepatic Metastases/Immunotherapy/In Vivo/Salmonella enterica Typhimurium/Human IL-2 cDNA/Oral Administration

Greeno, Edward W.; and Saltzman, Daniel; University of Minnesota; Minneapolis, Minnesota; *A Phase I Study of an IL-2 Expressing, Attenuated Salmonella enterica Typhimurium in Patients with Unresectable Hepatic Spread from any Non-Hematologic Primary Cancer*.

NIH/OBA Receipt Date: 07-21-09. Publicly Reviewed at the September 2009 RAC meeting

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0907-992 (Open) Gene Therapy/Phase I/II/Cancer/Solid tumor/Breast Cancer/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/p53 cDNA/Intradermal Injection

Soliman, Hatem; H. Lee Moffitt Cancer Center and Research Institute, University of South Florida; Tampa, Florida; *A Phase 1/2 Study of Ad.p53 DC Vaccine in Combination with 1-methyl-D-tryptophan in Metastatic Invasive Breast Cancer*.

NIH/OBA Receipt Date: 07-31-09. Not Selected for RAC Public Review: 08-24-09

0908-993 (Open) Gene Therapy/Phase I/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Zheng, Lei; Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins; Baltimore, Maryland; *A Safety and Feasibility Trial of Boost Vaccinations of a Lethally Irradiated, Allogeneic Pancreatic Tumor Cell Vaccine Transfected with the GM-CSF Gene Given Alone or in Combination with Either a Single Intravenous Dose or Daily Metronomic Oral Doses of Cyclophosphamide for the Treatment of Surgically Resected Pancreatic Adenocarcinoma*.

NIH/OBA Receipt Date: 08-03-09. Not Selected for RAC Public Review: 08-25-09

0908-994 (Open) Gene Therapy/Phase I/Cancer/Medulloblastoma/Primitive Neuroectodermal Tumors/Immunotherapy/In Vivo/RNA Transfer/Intradermal and Intravenous Injections

Mitchell, Duane A.; Duke University Medical Center; Durham, North Carolina; *Re-MATCH: **Re**current **M**edulloblastoma and Primitive Neuroectodermal Tumor **A**doptive **T** Cell Therapy during Recovery from Myeloablative **C**hemotherapy and **H**ematopoietic Stem Cell Transplantation*.

NIH/OBA Receipt Date: 08-05-09. Not Selected for RAC Public Review: 08-28-09

0908-995 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Solid Tumors/Vaccinia Virus/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) and Humanized *Escherichia coli β*-galactosidase cDNAs/Intratumoral Injection

Cripe, Timothy; Cincinnati Children's Hospital Medical Center; Cincinnati, Ohio; and Louis, Chrystal; Baylor College of Medicine; Houston, Texas; *A Phase I, Open-Label, Dose-Escalation Trial of JX-594 (Thymidine Kinase-Inactivated Vaccinia Virus Plus GM-CSF) Administered by Intratumoral Injection in Pediatric Patients with Unresectable Refractory Solid Tumors*. Sponsor: Jennerex

NIH/OBA Receipt Date: 08-06-09. Publicly Reviewed at the December 2009 RAC meeting

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0908-996 (Closed) Gene Therapy/Phase I/Cancer/Colorectal/Vaccinia Virus/Vector-Directed Tumor Lysis/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) and Humanized *Escherichia coli β*-galactosidase cDNAs/Intratumoral Injection

Auer, Rebecca; Ottawa Hospital Research Institute; Ottawa, Ontario; *A Phase II Study of Neoadjuvant JX-594 (Thymidine Kinase-Inactivated Vaccinia Virus Plus GM-CSF) Administered by Intratumoral Injection followed by Surgical Resection in Patients with Metastatic Colorectal Tumors within the Liver (JX594-IT-HEP012)*. Sponsor: Jennerex

NIH/OBA Receipt Date: 08-25-09. Not Selected for RAC Public Review: 09-16-09
Closed: 07-13-12

0908-997 (Open) Gene Therapy/Phase II/Cancer/Multiple Myeloma/Immunotherapy/In Vitro/Allogeneic K562 Cells/GM-CSF cDNA/Intradermal Injections

Borrello, Ivan; The Sidney Kimmel Comprehensive Cancer Center, Johns Hopkins Medical Institutions; Baltimore, Maryland; *Randomized Trial of Activated Marrow Infiltrating Lymphocytes Alone or in Conjunction with an Allogeneic GM-CSF based Myeloma Cellular Vaccine in the Autologous Transplant Setting in Multiple Myeloma*.

NIH/OBA Receipt Date: 08-28-09. Not Selected for RAC Public Review: 09-21-09

0909-998 (Open) Gene Therapy/Phase I/Cancer/B Cell Malignancies /Immunotherapy/In Vitro/Allogeneic T Lymphocytes/Retrovirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Bishop Michael; National Institutes of Health; Bethesda, Maryland; *Administration of Anti-CD19-Chimeric-Antigen-Receptor-Transduced T cells from the Original Transplant Donor to Patients with Recurrent or Persistent B-cell Malignancies after Allogeneic Stem Cell Transplantation*.

NIH/OBA Receipt Date: 09-08-09. Not Selected for RAC Public Review: 09-29-09

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0909-999 (Open) Gene Therapy/Phase I/Other Diseases/Peripheral Artery Disease/In Vivo/plasmid DNA/Hepatocyte Growth Factor cDNA/intramuscular injection

Kessler, John A.; Feinberg School of Medicine; Northwestern University; Chicago, Illinois; and Christiansen, Mark P.; Diablo Clinical Research, Inc.; Walnut Creek, California; *A Phase I/II, Open Label, Dose-Escalation Study to Assess the Safety and Tolerability of VM202 in Patients with Painful Diabetic Peripheral Neuropathy*. Sponsor: ViroMed, Co., Ltd.

NIH/OBA Receipt Date: 09-08-09. Not Selected for RAC Public Review: 09-29-09

0909-1000 (Closed) Gene Therapy/Phase I/Other Disorders/Non-Malignant Disorders/Elimination of Graft versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/Inducible Caspase 9 Suicide Gene/ AP1903 or AP20187/Intravenous Infusion

Krance, Robert A.; Baylor College of Medicine; Houston, Texas; *Pilot Study to Evaluate Allodepleted T Cells Transduced with Inducible Caspase 9 Suicide Gene after Haploidentical Stem Cell Transplantation for Non Malignant Disorders (CASPIAN)*.

NIH/OBA Receipt Date: 09-08-09. Not Selected for RAC Public Review: 02-04-10
Closed, never initiated: 4-30-12

0909-1001 (Open) Gene Therapy/Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Tumor Necrosis Factor (TNF) cDNA/Intraprostatic Injection

Shevrin, Daniel; Northwestern University; NorthShore University HealthSystem; Evanston, Illinois; Liauw, Stanley; The Pritzker School of Medicine; The University of Chicago; Chicago, Illinois; and MacVicar, Gary; Northwestern University Feinberg School of Medicine; Chicago, Illinois; *A Phase I Safety and Tolerability Trial of Radiotherapy, Androgen Ablation, and Intratumoral Injections of Ad_{GV}EGR.TNF.11D (TNFerade™ Biologic) for Patients with Locally Advanced Prostate Cancer*. Sponsor: GenVec, Inc.

NIH/OBA Receipt Date: 09-16-09. Not Selected for RAC Public Review: 10-14-09

0910-1002 (Open; RAC reviewed with recommendations) Gene Therapy/ Phase II/Monogenic Disease/Alpha-1 Antitrypsin Deficiency/In Vivo/Adeno-associated Virus/Serotype 1/ Alpha-1 Antitrypsin cDNA/Intramuscular Injection

Flotte, Terence R.; University of Massachusetts Medical School; Worcester, Massachusetts; Trapnell, Bruce C.; Children's Hospital Medical Center; Cincinnati, Ohio; Sandhaus, Robert A.; National Jewish Medical and Research Center; Denver, Colorado; and McElvaney, Noel G.; Royal College of Surgeons in Ireland; Beaumont Hospital; Dublin 2, Ireland; *A Multiple-Site, Phase 2, Safety and Efficacy Trial of a Recombinant Adeno associated Virus Vector Expressing Alpha 1 Antitrypsin (rAAV1-CB-hAAT) in Patients with Alpha 1 Antitrypsin Deficiency*. Sponsor: Applied Genetic Technologies Corporation

NIH/OBA Receipt Date: 10-02-09. Publicly Reviewed at the December 2009 RAC meeting

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0910-1003 (Open) Gene Therapy/Phase I/Cancer/B-Lineage Malignancies/Immunotherapy/In Vitro/HLA Matched Allogeneic T Lymphocytes/*Sleeping Beauty* (*SB*) Transposon/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Cooper, Laurence J.N.; and Kebriaei, Partow; University of Texas, M.D. Anderson Cancer Center; Houston, Texas; *CD19-specific T Cell Infusion in Patients with B-Lineage Lymphoid Malignancies after Allogeneic Hematopoietic Stem-Cell Transplantation*.

NIH/OBA Receipt Date: 10-05-09. Not Selected for RAC Public Review: 10-27-09

0910-1004 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Heart Failure/In Vivo/Plasmid/Stromal Cell-Derived Factor (SDF-1) cDNA/Myocardial Administration Using Biocardia Helix Needle Injection Catheter

Losordo, Douglas W.; Northwestern University Feinberg School of Medicine; Chicago, Illinois, Mendelsohn, Farrell O.; Cardiology P.C.; Birmingham, Alabama; Sherman, Warren; Columbia University Medical Center; New York, New York; and Schaer, Gary L.; Rush University Medical Center; Chicago, Illinois; *An Open Label Dose Escalation Study to Evaluate the Safety of a Single Escalating Dose of ACRX-100 Administered by Endomyocardial Injection to Cohorts of Adults with Ischemic Heart Failure*. Sponsor: Juventas Therapeutics, Inc.

NIH/OBA Receipt Date: 10-05-09. Publicly Reviewed at the December 2009 RAC meeting

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0910-1005 (Open; RAC reviewed with recommendations) Gene Therapy/Phase III/Monogenic Diseases/Leber Congenital Amaurosis Type 2/Retinal Disease due to RPE65 Mutations/In Vivo/Adeno-Associated Virus/Serotype 2/RPE65 cDNA/Subretinal injection

Maguire, Albert; Children's Hospital of Philadelphia; Philadelphia, Pennsylvania; and Russell, Stephen; University of Iowa, Iowa City, Iowa; *A Safety and Efficacy Study in Subjects with Leber Congenital Amaurosis (LCA) using Adeno-associated Virus Vector to Deliver the Gene for Human RPE65 to the Retinal Pigment Epithelium (RPE) [AAV2-hRPE65v2-301].*

NIH/OBA Receipt Date: 10-05-09. Publicly Reviewed at the December 2009 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0910-1006 (Open; RAC reviewed with recommendations) Gene Therapy/Gene Therapy/Phase I/II/Monogenic Disease/Severe Combined Immune Deficiency due to Adenosine Deaminase Deficiency (ADA)/In Vitro/Autologous CD34+ Cells from Bone Marrow/Lentivirus/Adenosine Deaminase cDNA/Intravenous Infusion

Kohn, Donald; University of California, Los Angeles; Los Angeles, California; and Candotti, Fabio; National Institutes of Health; Bethesda, Maryland; *Treatment of Subjects with Adenosine Deaminase (ADA) Deficient Severe Combined Immunodeficiency (SCID) with Autologous Bone Marrow CD34+ Stem/Progenitor Cells after Addition of a Normal Human ADA cDNA by the EFS-ADA Lentiviral Vector*.

NIH/OBA Receipt Date: 10-06-09. Publicly Reviewed at the December 2009 RAC meeting

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0910-1007 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vitro/Allogeneic K562 Cell/Plasmid DNA/Granulocyte-macrophage Colony Stimulating Factor cDNA/Intradermal Injection

Schrump, David; National Institutes of Health; Bethesda, Maryland; *Pilot Study of Allogeneic Tumor Cell Vaccine with Metronomic Oral Cyclophosphamide and Celecoxib in Patients Undergoing Resection of Lung and Esophageal Cancers and Malignant Pleural Mesotheliomas*.

NIH/OBA Receipt Date: 10-06-09. Not Selected for RAC Public Review: 10-28-09

0910-1008 (Open) Gene Therapy/Phase I/Others/Diabetic Ulcer/In Vitro/Linear Plasmid/ $VEGF_{165}$/Skin Graft Tissue

Schurr, Michael; University of Wisconsin Hospital and Clinics; Madison, Wisconsin; *$ExpressGraft_{Vascular}$ Human Skin Substitute for the Treatment of Diabetic Foot Ulcers.*

NIH/OBA Receipt Date: 10-06-09. Not Selected for RAC Public Review: 10-29-09

0910-1009 (Open) Gene Therapy/Phase II/Peripheral Artery Disease/In Vivo/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection

Losordo, Douglas W.; Northwestern University Feinberg School of Medicine; Chicago, Illinois; and Ballard, Jeffrey L.; Vascular and Interventional Specialists of Orange County, Inc.; Orange, California; *A Phase II, Double-blind, Randomized, Placebo-controlled, Multicenter Study to Assess the Safety and Efficacy of VM202 in Subjects with Critical Limb Ischemia.* Sponsor: ViroMed Co., Ltd.

NIH/OBA Receipt Date: 10-09-09. Not Selected for RAC Public Review: 11-02-09

0911-1010 (Open) Gene Therapy/Phase I/Cancer/Advanced Refractory Cancer/Immunotherapy/In Vitro/Plasmid DNA/Electroporation/GM-CSF cDNA/ bi-shRNA^{furin}/Intradermal Injections

Barve, Minal; Texas Oncology, P.A.; Dallas, Texas; and Melnyk, Anton M.S., Jr.; Texas Oncology, P.A.; Abilene, Texas; *Phase I Trial of bi-shRNA^{furin} and GMCSF Augmented Autologous Tumor Cell Vaccine for Advanced Cancer (FANG)*. Sponsor: Gradalis, Inc.

NIH/OBA Receipt Date: 11-02-09. Not Selected for RAC Public Review: 11-30-09

0911-1011 (Open) Gene Therapy/Phase II/Cancer/Colorectal/Immunotherapy/In Vivo/Saccharomyces cerevisiae/Mutated Ras Oncoprotein cDNAs/Subcutaneous injections

Marshall, John; Georgetown University Medical Center; Washington, DC; *A Pilot Phase 2 Trial of GI-4000 plus Bevacizumab and either FOLFOX or FOLFIRI in Patients with Newly Diagnosed Ras Mutant Positive Metastatic Colorectal Cancer or Patients with Ras Mutant Positive Colorectal Cancer who have just Completed a First Line Therapy with an Oxaliplatin or Irinotecan Plus Fluoropyrimidine and Bevacizumab Containing Regimen*. Sponsor: GlobeImmune, Inc.

NIH/OBA Receipt Date: 11-16-09. Not Selected for RAC Public Review: 12-11-09

0912-1012 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo Electroporation/Plasmid DNA/Interleukin-12 cDNA/Intratumoral *In Vivo* Electroporation

Daud, Adil; University of California, San Francisco; San Francisco, California; *Randomized Phase II Trial of Intratumoral pIL-12 Electroporation in Advanced Stage Malignant Melanoma*.

NIH/OBA Receipt Date: 12-11-09. Not Selected for RAC Public Review: 1-05-10

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

0912-1013 (Open) Gene Therapy/Phase III/Cancer/Pancreatic Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3)galactosyltransferase/Intradermal Injection

Hardacre, Jeffrey M.; University Hospitals Case Medical Center; Cleveland, Ohio; Chiorean, Elena Gabriela; Indiana University; Indianapolis, Indiana; Cohen, Steven J.; Fox Chase Cancer Center; Philadelphia, Pennsylvania; Espat, N. Joseph; Roger Williams Hospital; Providence, Rhode Island; Hawkins, William; Washington University School of Medicine; St. Louis, Missouri; Fisher, William E.; Baylor College of Medicine; Houston, Texas; Grem, Jean L.; University of Nebraska Medical School; Omaha, Nebraska; Velanovich, Vic; Henry Ford Hospital; Detroit, Michigan; Fisher, George; Stanford University; Stanford, California; Morse, Michael; Duke University Medical Center; Durham, North Carolina; Weekes, Colin Dexter; University of Colorado Cancer Center; Aurora, Colorado; Obel, Jennifer R.; Northshore University Health System; Evanston, Illinois; Lenz, Henz-Josef; University of Southern California; Los Angeles, California; Springett, Gregory; H. Lee Moffitt Cancer Center; Tampa, Florida; Scoggins, Charles Raben; University of Louisville; Louisville, Kentucky; Marshall, John L.; Lombardi Cancer Center; Washington, DC; Pant, Shubham; The University of Oklahoma Health Sciences Center; Oklahoma City, Oklahoma; Mulcahy, Mary; Northwestern University; Chicago, Illinois; Labow, Daniel; Mount Sinai School of Medicine; New York, New York; Kennedy, Eugene Paul; Thomas Jefferson University; Philadelphia, Pennsylvania; Shaw, James E.; Massey Cancer Center; Richmond, Virginia; Hsueh, Chung-Tsen; Loma Linda University Cancer Institute; Loma Linda, California; Schwarz, Roderich E.; University of Texas Southwestern Medical Center; Dallas, Texas; Dumlao, Theresa Liu; University of South Alabama; Mobile, Alabama; Sanoff, Hanna K.; University of Virginia Health System; Charlottesville, Virginia; Sun, Weijing; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; Wang, Yubao; The University of Texas Health Sciences Center at San Antonio; San Antonio, Texas; Conway, W. Charles, II; Ochsner Cancer Institute; New Orleans, Louisiana; Nugent, Francis W., III; Lahey Clinic; Burlington, Massachusetts; Brenner, Warren S.; Boca Raton Hospital; Boca Raton, Florida; Dragovich, Tomislav; University of Arizona College of Medicine; Tucson, Arizona; Baron, Ari, California Pacific Medical Center; San Francisco, California; Moser, Arthur James; University of Pittsburgh; Whiting, Samuel H.; Seattle Cancer Care Alliance; Seattle, Washington; Seng, John, Virginia Piper Cancer Center; Minneapolis, Minnesota; Shen, Perry; Wake Forest University; Winston-Salem, North Carolina; Nadeau, Laura M.; Hematology Oncology Consultants, PC; Royal Oak, Michigan; Fischer, Craig P.; The Methodist Hospital; Houston, Texas; Saif, Wasif Muhammad; Columbia University Medical Center; New York, New York; George, Thomas J.; University of Florida; Gainesville, Florida; Nicholl, Michael B.; University of Missouri; Columbia, Missouri; Nissen, Nicholas N.; Cedars-Sinai Medical Center; Los Angeles, California; Kim, George; Mayo Clinic-Jacksonville; Jacksonville, Florida; Bekaii-Saab, Tanios S.; The Ohio State University Medical Center; Columbus, Ohio; Paul, Kathleen P.; Lynchburg Hematology Oncology Clinic; Lynchburg, Virginia; Mahalingham, Devalingam; The University of Texas Health Sciences Center at San Antonio; San Antonio, Texas; McWilliams, Robert R.; Mayo Clinic; Rochester, Minnesota; Berg, Daniel James; The University of Iowa; Iowa City, Iowa; Ferrone, Cristina R.; Massachusetts General Hospital; Boston, Massachusetts; Cho, Clifford Suhyun; University of Wisconsin; Madison, Wisconsin; Olowokure, Olugbenga; University of Cincinnati; Cincinnati, Ohio; Gusani Niraj; Penn State College of Medicine, Hershey; Hershey, Pennsylvania; Vaccaro, Gina, Oregon Health & Science University; Portland, Oregon; Rich, Randy; Cancer Care & Hematology Specialists of Chicago; Arlington Heights, Illinois; Chung, Vincent; City of Hope Nation Medical Center; Duarte, California; Manges, Robert F.; Investigative Clinical Research of Indiana, LLC; Indianapolis, Indiana; Badgwell, Brian; University of Arkansas; Little Rock, Arkansas; Del Prete, Salvatore; Stamford Hospital; Stamford, Connecticut; D'Andre, Stacy; Sutter Cancer Center; Sacramento, California; Posey, James A, III; University of Alabama at Birmingham; Birmingham, Alabama; Bienvenu, Bryan J., Mary Bird Perkins Cancer Center; Baton Rouge, Louisiana; Lee, Fa-Chyi, University of New Mexico; Albuquerque, New Mexico; Patel, Dhimant; Aurora BayCare Medical Center; Green Bay, Wisconsin; Desai, Darius C.; St. Luke's Cancer Care Associates; Bethlehem, Pennsylvania; Hanna, Nader Nabil; University of Maryland, Baltimore; Baltimore, Maryland; Zeh, Hebert J.; University of Pittsburgh Medical Center; Pittsburgh, Pennsylvania; Adams, Reid Barton; University of Virginia Health System; Charlottesville, Virginia; Taylor, William R.; University of South Alabama; Mobile, Alabama; Lavu, Harish; Thomas Jefferson University; Philadelphia, Pennsylvania; Velanovich, Vic; University of South Florida; Tampa, Florida; Zalupski, Mark Michael; The University of Michigan; Ann Arbor, Michigan; Williamson, Stephen K.; University of Kansas Cancer Center; Kansas City, Kansas; Molina, Manuel A.; Lakeland Regional Cancer; Lakeland, Florida; and Tafur, Isaac; Joe Arrington Cancer Research and Treatment Center; Lubbock, Texas; *A Phase III Study of Chemotherapy and Chemoradiotherapy With or Without HyperAcute®-Pancreatic Cancer Vaccine in Subjects with Surgically Resected Pancreatic Cancer*. Sponsor: NewLink Genetics Corporation.

NIH/OBA Receipt Date: 12-18-09. Not Selected for RAC Public Review: 1-12-10

0912-1014 (Open) Gene Therapy/Phase I/Infectious Disease/Cytomegalovirus (CMV), Epstein-Barr (EBV), Adenovirus Disease/In Vitro/EBV, Ad, and CMV-specific Cytotoxic T Lymphocytes/Plasmid/CMV pp65, IE1, Ad-hexon, Ad-penton, EBV EBNA1, LMP2, BZLF1/Intravenous

Heslop, Helen; Baylor College of Medicine, Texas Children's Hospital; Houston, Texas; *Administration of Rapidly Generated Multivirus-Specific Cytotoxic T-Lymphocytes for the Prophylaxis and Treatment of EBV, CMV and Adenovirus Infection post Allogeneic Stem Cell Transplant (VIRAGE)*.

NIH/OBA Receipt Date: 12-23-09. Not Selected for RAC Public Review: 1-15-10

0912-1015 (Open) Gene Therapy/Phase II/Infectious Disease/Human Immunodeficiency Virus/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Autologous HIV RNA/Plasmids/CD40L cDNA/Intradermal Injection

Jacobson, Jeffrey M.; Drexel University College of Medicine; Philadelphia, Pennsylvania; Stein, David Kidd; Jacobi Medical Center; Bronx, New York; Margolis, David M.; University of North Carolina at Chapel Hill; Chapel Hill, North Carolina; Asmuth, David Michael; University of California Davis Medical Center; Sacramento, California; Routy, Jean Pierre; McGill University Health Centre; Montreal, Canada; Hicks, Charles; Duke University Medical Center; Durham, North Carolina; Vezina, Sylvie; Clinique medical l'Actuel; Montreal, Canada; and Angel, Jonathan B.; Ottawa Hospital; Ottawa, Ontario; *A Randomized, Double Blind, Phase 2B Study Testing the Efficacy and Safety of AGS-004 on Host Control of HIV Replication during Analytical Treatment Interruption*. Sponsor: Argos Therapeutics, Inc.

NIH/OBA Receipt Date: 12-23-09. Not Selected for RAC Public Review: 2-01-10

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0912-1016 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Other Diseases-disorders/Degenerative Arthritis/In Vitro/ Allogeneic Human Chondrocytes/Retrovirus/Transforming Growth Factor-β1 (TGF-β1)/Intra-articular Administration

Mont, Michael A.; Center for Joint Preservation and Replacement; Sinai Hospital of Baltimore; Baltimore, Maryland; Romness, David William; Commonwealth Orthopaedics and Rehabilitation; Arlington, Virginia; Figueroa, Debra P.; Pinellas Park, Florida; Parvizi, Javad; Jefferson Medical College; Philadelphia, Pennsylvania; and Cherry, Kenneth; University Orthopedics Center; State College, Pennsylvania; *A Phase II Study to Determine the Efficacy and Safety of Allogeneic Human Chrondrocytes Expressing TGF- β1 in Patients with Grade 3 Chronic Degenerative Joint Disease of the Knee*. Sponsor: TissueGene, Inc.

NIH/OBA Receipt Date: 12-24-09. Publicly Reviewed at the March 2010 RAC meeting

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1001-1017 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous Lymphocytes/Adenovirus/Serotype 5/cDNA of Engineered Zinc Finger Nucleases (ZFNs) Targeting the Human CCR5 Locus (SB-728)/Intravenous

Mitsuyasu, Ronald; University of California, Los Angeles; Los Angeles, California; and Connick, Elizabeth; University of Colorado, Denver; Denver, Colorado; *A Phase 1 Single and Repeat-Dosing Study of Autologous T-Cells Genetically Modified at the CCR5 Gene by Zinc Finger Nucleases SB-728 in HIV-Infected Subjects who have Exhibited Suboptimal CD4+ T-Cell Gains During Long-Term Antiretroviral Therapy*. Sponsor: Sangamo Biosciences, Inc.

NIH/OBA Receipt Date: 1-06-10. Not Selected for RAC Public Review: 2-03-10

1001-1018 (Under review) Gene Therapy/Phase I/Cancer/Glioma/Pro-Drug/In Vitro/Autologous Mesenchymal Stromal Cells/Plasmid/Yeast Cytosine Deaminase (CD) cDNA/Intracerebral Administration

Black, Peter; Brigham and Women's Hospital; Harvard Medical School; Boston, Massachusetts; *A Pilot Study of Cytosine Deaminase -Expressing Mesenchymal Stromal Cells in Recurrent Glioblastoma*.

NIH/OBA Receipt Date: 1-12-10.

1001-1019 (Open) Gene Therapy/Phase I/Thyroid Cancer/In Vivo/Adenovirus/Serotype 5/Fas-TNF Receptor Chimera Transgene/Intravenous Injection

Bible, Keith; Mayo Clinic Cancer Center; Rochester, Minnesota; and Smallridge, Robert C.; Mayo Clinic-Jacksonville; Jacksonville, Florida; *A Phase II, Open-Label Study to Assess the Safety and Efficacy of a Single Intravenous (IV) Dose of VB-111 in Subjects with Advanced Differentiated Thyroid Cancer (DTC)*. Sponsor: Vascular Biogenics Ltd.

NIH/OBA Receipt Date: 1-13-10. Not Selected for RAC Public Review: 2-04-10

1001-1020 (Closed; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Osteosarcoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/Chimeric Antigen Receptor- HER2- CD28 endodomain/Intravenous Injections

Ahmed, Nabil; Texas Children's Hospital; Houston, Texas; *Administration of EBV-specific Cytotoxic T Cells Expressing HER2 Chimeric Antigen Receptor to Subjects with Advanced Osteosarcoma (ECHO)*.

NIH/OBA Receipt Date: 1-12-10. Publicly Reviewed at the March 2010 RAC meeting
Closed: 03/21/2012

1001-1021 (Open) Gene Therapy/Phase I/Cancer/Squamous Cell Carcinoma of the Head and Neck/In Vivo/Measles Virus/Sodium Iodide Symporter (NIS) cDNA/Intratumoral Administration

Scott Okuno; Mayo Clinic; Rochester, Minnesota; *Phase I Trial of Intratumoral Administration of a NIS-Expressing Derivative Manufactured from a Genetically Engineered Strain of Measles Virus in Patients with Recurrent/metastatic Squamous Cell Carcinoma of the Head and Neck*.

NIH/OBA Receipt Date: 1-12-10. Not Selected for RAC Public Review: 2-04-10

1001-1022 (Open) Gene Therapy/Phase I/Cancer/B-Lineage Malignancies/Immunotherapy/In Vitro/Allogeneic Umbilical Cord Blood-derived Lymphocytes/Sleeping Beauty (SB) Transposon/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Cooper, Laurence; and Kelly, Susan; MD Anderson Cancer Center; Houston, Texas; *Adoptive Immunotherapy for CD19+ B-cell Malignancies using Sleeping Beauty Transposition to Express a CD19-specific Chimeric Antigen Receptor in Allogeneic Neonatal Ex Vivo expanded T cells*.

NIH/OBA Receipt Date: 1-12-10. Not Selected for RAC Public Review: 2-05-10

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1001-1023 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/ Sickle Cell Disease/In Vitro/Autologous CD34+ Cells/Lentivirus/Human β-Globin with γ Globin Exons Gene/Intravenous

Malik, Punam; Cincinnati Children's Hospital Medical Center; Cincinnatti, Ohio; *Gene Transfer for Patients with Sickle Cell Disease Using a Gamma Globin Lentivirus Vector: An Open Label Phase I/II Pilot Study*.

NIH/OBA Receipt Date: 1-12-10. Publicly Reviewed at the March 2010 RAC meeting

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1001-1024 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Tumor infiltrating T lymphocytes CXCR2 cDNA/NGFR cDNA/Intravenous Injections

Hwu, Patrick; MD Anderson Cancer Center; Houston, Texas; *Lymphodepletion Plus Adoptive Cell Transfer with CXCR2 and NGFR Transduced T-Cells Followed by High Dose Interleukin-2 in Patients with Metastatic Melanoma*.

NIH/OBA Receipt Date: 1-12-10. Publicly Reviewed at the March 2010 RAC meeting

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1001-1025 (Open) Gene Therapy/Phase I/Cancer/CD19+ Acute Lymphoblastic Leukemia Relapsed Post Allogeneic Stem Cell Transplantation/Immunotherapy/In Vitro/Autologous CD3+ T Lymphocytes/Lentivirus/CD19 antigen specific-Zeta T Cell Receptor/Intravenous Injections

Porter, David; Hospital of the University of Pennsylvania; Philadelphia, Pennsylvania; *Pilot Study of Donor Lymphocyte Infusions Using Donor T Cells Engineered to Contain Anti-CD19 Attached to TCR-ζ and 4-1BB Signaling Domains in Patients with Relapsed CD19+ ALL After Allogeneic Stem Cell Transplantation*.

NIH/OBA Receipt Date: 1-12-10. Not Selected for RAC Public Review: 2-05-10

1001-1026 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Prostate/Apoptosis Induction/In Vivo/Adenovirus/REIC-Dkk-3 cDNA/Intratumoral Injection

Hall, Simon J; Mount Sinai School of Medicine; New York, New York; *A Phase I Neoadjuvant Study of In-situ REIC/Dkk-3 Therapy Followed by Prostatectomy in Patients with High Risk Localized Prostate Cancer*. Sponsor: Momotaro-Gene.

NIH/OBA Receipt Date: 1-19-10. Publicly Reviewed at the March 2010 RAC meeting

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1002-1027 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag, Pol and Env cDNAs/Intramuscular Injections followed by Electroporation

Tebas, Pablo; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; *A Phase I, Open Label Study to Evaluate the Safety, Tolerability, and Immunogenicity of PENNVAX™-B (gag, pol, env) + Electroporation in HIV-1 Infected Adult Participants.* Sponsor: VGX Pharmaceuticals.

NIH/OBA Receipt Date: 2-02-10. Not Selected for RAC Public Review: 3-02-10

1004-1028 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Cancer/Chronic Lymphocytic Leukemia, Small Lymphocytic Lymphoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/CD154/Intranodal Injection

Castro, Januario E.; University of California San Diego; La Jolla, California; *A Phase II Study of Repeat Intranodal Injections of Adenovirus-CD154 (Ad-ISF35) in Patients with Chronic Lymphocytic Leukemia / Small Lymphocytic Lymphoma*.

NIH/OBA Receipt Date: 4-23-10. Publicly Reviewed at the June 2010 RAC meeting

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1002-1029 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Cancer/ Non-Hodgkin's Lymphoma (Follicular, Diffuse Large Cell Mantle Cell, and Small Lymphocytic Leukemia; Chronic Lymphocytic Leukemia)/Immunotherapy/In Vivo/Adenovirus/Serotype 5/CD154/Intranodal Injection

Castro, Januario E.; University of California San Diego; La Jolla, California; *A Phase II Study of Repeat Intranodal Injections of Adenovirus-CD154 (Ad-ISF35) in Patients with Non-Hodgkin's Lymphoma (Follicular, Diffuse Large Cell Mantle Cell, and Small Lymphocytic Leukemia / Chronic Lymphocytic Leukemia)*.

NIH/OBA Receipt Date: 02-26-10. Publicly Reviewed at the June 2010 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1002-1030 (Open) Gene Therapy/Phase II/Other/Diabetic Peripheral Neuropathy/In Vivo/Plasmid DNA/ZPF-TF cDNA (Zinc Finger DNA Binding Protein)/Intramuscular Injection

Brannagan, Thomas H., III; The Neurological Institute; New York, New York; Mendelson, John Edward; California Pacific Medical Center, Research Institute; San Francisco, California Beydoun, Said; University of Southern California; Los Angeles, California; Rendell, Mark S.; Creighton University School of Medicine; Omaha, Nebraska; Sang, Christine; Brigham and Women's Hospital; Boston, Massachusetts; and Pulley, Michael T.; University of Florida Health Sciences Center Jacksonville; Jacksonville, Florida; *A Phase 2b Repeat Dosing Clinical Trial of SB-509 in Subjects with Moderately Severe Diabetic Neuropathy*. Sponsor: Sangamo Biosciences, Inc.

NIH/OBA Receipt Date: 2-24-10. Not Selected for RAC Public Review: 3-17-10

1003-1031 (Open) Gene Therapy/Phase I/Cancer/Mesothelioma or Pleural Malignancies/Immunotherapy/In Vivo/Adenovirus/Serotype 5/Interferon-alpha 2b/Intrapleural Administration

Sterman, Daniel H.; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; *A Pilot and Feasibility Trial Evaluating Two Different Chemotherapy Regimens in Combination with Intrapleural Adenoviral-Mediated Interferon-Alpha (SCH 721015, Ad.hIFN-α2b) Gene Transfer for Malignant Pleural Mesothelioma*

NIH/OBA Receipt Date: 3-10-10. Not Selected for RAC Public Review: 3-31-10

1003-1032 (Closed) Gene Therapy/Phase III/Cancer/Squamous Cell Carcinoma of the Head and Neck/Herpes Simplex Virus Type-1/Vector-Directed Tumor Lysis/Granulocyte-macrophage Colony Stimulating Factor (GM-CSF)/Intratumoral Injection

Posner, Marshall, R.; Dana-Farber Cancer Institute; Boston, Massachusetts; Gabrail, Nashat Y.; Gabrail Cancer Center Research; Canton, Ohio; Feeney, Kendra J.; Thomas Jefferson University; Philadelphia, Pennsylvania; Hauke, Ralph; Nebraska Methodist Hospital; Omaha, Nebraska; McWilliams, Robert R.; Mayo Clinic; Rochester, Minnesota; Pendergrass, Kelly B.; Kansas City Cancer Center; Kansas City, Missouri; Ross, Merrick I.; The University of Texas MD Anderson Cancer Center; Houston, Texas; DiNardo, Laurence J.; Virginia Commonwealth University; Richmond, Virginia; Rabinowits, Guilherme; University of Louisville; Louisville, Kentucky; Schultz, Stephen M.; Investigative Clinical Research of Indiana, LLC; Indianapolis, Indiana; Shirai, Keisuki; Medical University of South Carolina; Charleston, South Carolina; Brumund, Kevin T.; Moores UCSD Cancer Center; La Jolla, California; and Guthrie, Troy H., Jr.; Baptist Cancer Institute; Jacksonville, Florida; *A Phase 3 Randomized Trial of Concurrent Cisplatin and Radiotherapy With or Without OncoVEX^{GM-CSF} in Previously Untreated Patients with Locally Advanced Squamous Cell Carcinoma of the Head and Neck*. Sponsor: BioVex, Inc.

NIH/OBA Receipt Date: 3-12-10. Not Selected for RAC Public Review: 4-02-10
Closed to new enrollment: 08-04-11

1003-1033 (Open) Gene Therapy/Phase I/Cancer/Mesothelioma/Vector-Directed Cell Lysis/In Vivo/Measles Virus/Sodium Iodide Symporter (NIS) cDNA/Intrapleural Administration

Kratzke, Robert A.; University of Minnesota; Minneapolis, Minnesota; and Peikert, Tobias; Mayo Clinic; Rochester, Minnesota; *A phase I Trial of Oncolytic Measles Virotherapy in Malignant Pleural Mesothelioma*.

NIH/OBA Receipt Date: 3-18-10. Not Selected for RAC Public Review: 4-09-10

1004-1034 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Hodgkin's Lymphoma/Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/Autologous Epstein Barr Virus cytotoxic T Lymphocytes/Retrovirus/CD30 antigen specific-Zeta T Cell Receptor/Intravenous Injections

Heslop, Helen; Baylor College of Medicine; Houston, Texas; *Phase I Study of the Administration of EBV CTLs Expressing CD30 Chimeric Receptors for Relapsed CD30+ Hodgkin's Lymphoma and CD30+ Non-Hodgkin's Lymphoma (CAR CD30)*.

NIH/OBA Receipt Date: 4-05-10. Publicly Reviewed at the June 2010 RAC meeting

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1004-1035 (Open) Gene Therapy/Phase I/Cancer/Bladder/Immunotherapy/In Vivo/Adenovirus Serotype 5/Interferon Alpha-2b cDNA/Intravesical Administration

Dinney, Colin; University of Texas, MD Anderson Cancer Center; Houston, Texas; *Phase 1B Intravesical Administration of SCH 721015 in Patients with Transitional Cell Carcinoma of the Bladder*.

NIH/OBA Receipt Date: 4-08-10. Not Selected for RAC Public Review: 4-29-10

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1004-1036 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Metastatic cancer (melanoma and renal)/Immunotherapy/In Vitro/Autologous CD8+ Peripheral Blood Lymphocytes/Retrovirus/VEGFR antigen specific-Zeta T Cell Receptor/Intravenous Injections

Rosenberg Steven; National Institutes of Health; Bethesda, Maryland; *Phase I/II Study of Metastatic Cancer Using Lymphodepleting Conditioning followed by Infusion of Anti-VEGFR2 Gene Engineered CD8+ Lymphocytes*.

NIH/OBA Receipt Date: 4-09-10. Publicly Reviewed at the June 2010 RAC meeting

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1004-1037 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Metastatic Melanoma/Immunotherapy/In Vitro/Autologous CD8+ Tumor Infiltrating Lymphocytes/Retrovirus/Interleukin-12/Intravenous Injections

Rosenberg Steven; National Institutes of Health; Bethesda, Maryland; *Phase I/II Study of Metastatic Melanoma Using Lymphodepleting Conditioning Followed by Infusion of CD8 Enriched Tumor Infiltrating Lymphocytes Genetically Engineered to Express IL-12*.

NIH/OBA Receipt Date: 4-09-10. Publicly Reviewed at the June 2010 RAC meeting

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1004-1038 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/In Vivo/Plasmid/HIV-1 Gag/Pol and Nef/Tat/Vif/Env cDNAs/Interleukine-12 cDNA/Intramuscular Injections

Tebas, Pablo; University of Pennsylvania; Philadelphia, Pennsylvania; Luetkemeyer, Anne; University of California, San Francisco; San Francisco, California; Zolopa, Andrew; Stanford University Medical Center; Stanford, California; Luque, Amneris Esther; University of Rochester; Rochester, New York; Sax, Paul E.; Harvard University, Brigham and Women's Hospital; Boston, Massachusetts; Gandhi, Rajesh; Massachusetts General Hospital, Boston, Massachusetts; Macatangay, Bernard; University of Pittsburgh; Pittsburgh, Pennsylvania; Davis, Charles E.; University of Maryland, Baltimore; Baltimore, Maryland; Fichtenbaum, Carl J.; University of Cincinnati; Cincinnati, Ohio; and Onen, Nur F.; Washington University School of Medicine; Saint Louis, Missouri; Mitsuysau, Ronald T.; University of California Los Angeles; Los Angeles, California; and Arduino, Roberto C.; The University of Texas Houston Health Sciences Center; Houston, Texas; Campbell, Thomas; University of Colorado, Denver; Denver, Colorado; *A Phase I Randomized, Partially Double-Blind, Placebo-Controlled, Dose-Escalation Study to Evaluate the Safety and Immunogenicity of a Cytokine Enhanced HIV1 Multiantigen pDNA Vaccine Delivered Intramuscularly (IM) or IM in Combination with In Vivo Electroporation (IM/EP) in HIV1 Infected Adults Receiving HAART*. Sponsor: Profectus BioSciences.

NIH/OBA Receipt Date: 4-12-10. Not Selected for RAC Public Review: 5-04-10

1004-1039 (Open) Gene Therapy/Phase II/Cancer/Prostate/Immunotherapy/In Vivo/Vaccinia Virus/Fowlpox Virus/Prostate Specific Antigen (PSA)/B7.1 (CD80)/ICAM-1/LFA-3/GM-CSF/Subcutaneous Injection

McNeel, Douglas; University of Wisconsin; Madison, Wisconsin; and Kohli, Manish; Mayo Clinic; Rochester, Minnesota; and Ferrari, Anna C.; New York University Cancer Center; New York, New York; Drake, Charles G.; John Hopkins; Baltimore, Maryland; Larned, Zoe; Ochsner Clinic Foundation; New Orleans, Louisiana; MacVicar, Gary; Northwestern University; Chicago, Illinois; Bubley, Glenn; Beth Israel Deaconess Medical Center, Boston; Boston, Massachusetts; and Graham, David L.; Carle Foundation; Urbana, Illinois; *Randomized Phase II Trial of Docetaxel with or without PSA-TRICOM Vaccine in Patients with Castrate-Resistant Metastatic Prostate Cancer*. Sponsor: NCI Clinical Trials Evaluation Program (CTEP).

NIH/OBA Receipt Date: 4-14-10. Not Selected for RAC Public Review: 5-12-10

1004-1040 (Open) Gene Therapy/Phase I/Cancer/Cervical/Immunotherapy/In Vivo/Plasmid DNA/HPV16 E6-E7 Fusion Protein cDNA/ HPV18 E6-E7 Fusion Protein cDNA/Intramuscular Injection in combination with Electroporation

Parker, R. Lamar, Jr.; Lyndhurst Clinical Research; Winston-Salem, North Carolina; Sunyecz, John A.; Laurel Highlands OBGYN, P.C.; Hopwood, Pennsylvania; and Morales-Ramirez, Javier O.; Clinical Research Puerto Rico; San Juan, Puerto Rico; *Phase I, Open-Label Study to Evaluate the Safety, Tolerability and Immunogenicity of a Fourth Dose of Human Papillomavirus (HPV) DNA Plasmid (VGX-3100) + Electroporation (EP) in Adult Females Previously Immunized with VGX-3100*. Sponsor: VGX Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 4-15-10. Not Selected for RAC Public Review: 5-06-10

1004-1041 (Open) Non-therapeutic (Healthy Volunteers)/Phase I/ /Human Immunodeficiency Virus/In Vivo/Plasmid/Vesicular Stomatis Virus (VSV)/HIV-1 Gag/Pol and Nef/Tat/Vif/Env cDNAs/Interleukine-12 cDNA/Intramuscular Injections

Hay, Christine; University of Rochester; Rochester, New York; *A Phase 1 trial to Evaluate the Safety and Immunogenicity of an IL-12 pDNA Enhanced HIV-1 Multiantigen pDNA Vaccine Delivered Intramuscularly with Electroporation, as a Prime for an HIV-1 rVSV Vaccine Boost, in Healthy HIV Uninfected Adult Participants*. Sponsor: HIV Vaccine Trials Network

NIH/OBA Receipt Date: 4-15-10. Not Selected for RAC Public Review: 5-12-10

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1005-1042 (Open) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Allogeneic Neuroblastoma Cell Lines/ Retrovirus/Cytokine/Interleukin-2/Plasmid/Electroporation/Lymphotactin/Subcutaneous Injections

Louis, Chrystal; Baylor College of Medicine; Houston, Texas; *A Phase I/II Study using Allogeneic Tumor Cell Vaccination with Oral Metronomic Cytoxan in Patients with High-Risk Neuroblastoma (ATOMIC)*.

NIH/OBA Receipt Date: 5-18-10. Not Selected for RAC Public Review: 6-09-10

1006-1043 (Open) Gene Therapy/Phase II/Cancer/Prostate Cancer/Immunotherapy/In Vitro/Vaccinia Virus/5T4 cDNA/Intramuscular Injection

Doshi, Gurjyot K.; University of Texas Health Science Center; Houston, Texas; Geils, George F., Sr.; Charleston Hematology-Oncology, PA; Charleston, South Carolina; Srinivas, Sandhya; Stanford University School of Medicine; Stanford, California; Ansari, Maria; The University of Texas, Houston Health Sciences Center; Houston, Texas; Gabrail, Nashat Y.; Gabrail Cancer Center; Canton, Ohio; Anna C.; New York University Cancer Center; New York, New York; Chu, Franklin M.; San Bernardino Urological Associates; San Bernardino, California; Showel, John L.; Rush University Medical Center; Chicago, Illinois; Lingamurthy, Manjesh; Holy Cross Hospital; Fort Lauderdale, Florida; and Nordquist, Luke T.; Urology Cancer Center; Omaha, Nebraska; *A Randomized Phase II Study to Assess the Activity of TroVax® (MVA-5T4) Plus Docetaxel Versus Docetaxel Alone in Subjects with Progressive Hormone Refractory Prostate Cancer*. Sponsor: Oxford BioMedica, Ltd.

NIH/OBA Receipt Date: 6-15-10. Not Selected for RAC Public Review: 7-13-10

1006-1044 (Open) Gene Therapy/Phase II/Cancer/T Cell Lymphoma/Mycosis Fungoides/Immunotherapy/In Vivo/Plasmid DNA/Interleukin-12 cDNA/Electroporation/Intratumoral Injection

Ai, Weiyun; University of California, San Francisco; San Francisco, California; *Phase II Trial of Intratumoral IL12 Plasmid Electroporation in Cutaneous Lymphoma*.

NIH/OBA Receipt Date: 6-17-10. Not Selected for RAC Public Review: 7-09-10

1007-1045 (Open)/Phase II/Cancer/Ovarian, Fallopian Tube, or Primary Peritoneal/Immunotherapy/In Vivo/DNA Complex with PEG-PEI-Cholesterol/Interleukin-12 cDNA/Intraperitoneal Injection

Alvarez, Ronald D.; University of Alabama School of Medicine; Birmingham, Alabama; Mannel, Robert; University of Oklahoma Health Sciences Center; Oklahoma City, Oklahoma; Werner, Theresa; The University of Utah; Salk Lake City, Utah; Waggoner, Steven; Case Western Reserve University; Cleveland, Ohio; Davidson, Susan A.; University of Colorado, Denver; Denver, Colorado; Kumar, Pallavi; Sinai Hospital of Baltimore; Baltimore, Maryland; De Geest, Koen; The University of Iowa; Iowa City, Iowa; Thigpen, J. Tate; University of Mississippi Medical Center; Jackson, Mississippi; Muller, Carolyn Y.; University of New Mexico; Albuquerque, New Mexico; Myers, Tashana Keisha Nicole; Baystate Medical Center; Springfield, Massachusetts; *A Phase II Evaluation of Intraperitoneal EGEN-001 (IL-12 Plasmid Formulated with PEG-PEI-Cholesterol Lipopolymer) in the Treatment of Persistent or Recurrent Epithelial Ovarian, Fallopian Tube or Primary Peritoneal Cancer*. Sponsor: Gynecologic Oncology Group.

NIH/OBA Receipt Date: 7-14-10. Not Selected for RAC Public Review: 8-04-10

1007-1046 (Withdrawn from review) Gene Therapy/Phase I/Cancer/Glioblastoma Multiforme/Immunotherapy/In Vtro/Allogeneic Virus-Specific Cytotoxic T Lymphocytes/Adenovirus/CMV pp65 Gene/Intravenous

Ahmed, Nabil; Baylor College of Medicine; Houston, Texas; *Phase I/II Administration of CMV-Specific Cytotoxic T cells in Patients with Glioblastoma Multiforme (COGLI)*.

NIH/OBA Receipt Date: 7-16-10.

1007-1047 (Open) Gene Therapy/Phase I/Monogenic Diseases/Leber Congenital Amaurosis Type 2/Retinal Disease due to RPE65 Mutations/In Vivo/Adeno-Associated Virus/Serotype 2/RPE65 cDNA/Subretinal injection

Maguire, Albert; Children's Hospital of Philadelphia; Philadelphia, Pennsylvania; *A Follow-On Study to Evaluate the Safety of Re-Administration of Adeno-Associated Viral Vector Containing the Gene for Human RPE65 [AAV2-hRPE65v2] to the Contralateral Eye in Subjects with Leber Congenital Amaurosis (LCA) Previously Enrolled in a Phase 1 Study*.

NIH/OBA Receipt Date: 7-19-10. Not Selected for RAC Public Review: 8-09-10

1007-1048 (Open) Gene Therapy/Phase I/Cancer/Merkel Cell Carcinoma/Immunotherapy/In Vivo Electroporation/Plasmid DNA/Interleukin-12 cDNA/Intratumoral *in vivo* Electroporation

Bhatia, Shailender; University of Washington; Seattle, Washington; Usmani, Saad; University of Arkansas for Medical Sciences; Little Rock, Arkansas; and Hamadani, Mehdi; West Virginia University; Morgan, West Virginia; *A Multicenter Pharmacodynamic Study of Intratumoral Injection of Interleukin-12 Plasmid and In Vivo Electroporation in Patients with Merkel Cell Carcinoma*.

NIH/OBA Receipt Date: 7-20-10. Not Selected for RAC Public Review: 8-10-10

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1007-1049 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Multiple Myeloma/Apoptosis Induction/Plasmid DNA/eIF5AK50R Gene/siRNA that Targets the Endogenous eIF5A/Intravenous Infusions

Lust, John A.; Mayo Clinic; Rochester, Minnesota; *Phase 1/2 Open-Label, Single-Center, Multiple-Dose, Dose-Escalation Study to Evaluate the Safety and Tolerability of SNSO1 -T Administered by Intravenous Infusion in Patients with Relapsed or Refractory Multiple Myeloma*. Sponsor: Senesco Technologies, Inc.

NIH/OBA Receipt Date: 7-21-10. Publicly Reviewed at the September 2010 RAC meeting

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1007-1050 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Lymphoplasmacytic Lymphoma/Immunotherapy/In Vivo/Plasmid DNA/ Macrophage-Inflammatory Protein 3α/Subject-specific Idiotype Single Chain (scFv)/Intramuscular Injection

Thomas, Sheeba; University of Texas, M.D. Anderson Cancer Center; Houston, Texas; *Phase I Study of an Active Immunotherapy for Asymptomatic Phase Lymphoplasmacytic Lymphoma with DNA Vaccines Encoding Antigen-Chemokine Fusion*.

NIH/OBA Receipt Date: 7-21-10. Publicly Reviewed at the September 2010 RAC meeting

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1007-1051 (Open) Gene Therapy/Phase I/Glioblastoma multiforme/In Vivo/Adenovirus/Serotype 5/Fas-TNF Receptor Chimera Transgene/Intravenous Injection

Wen, Patrick; Dana Farber/Brigham and Women's Cancer Center; Boston, Massachusetts; Brenner, Andrew; The University of Texas Health Sciences Center at San Antonio; San Antonio, Texas; and Vredenburgh, James Joseph; Duke University Medical Center; Durham, North Carolina; *A Phase I/II Single-Arm Open-Label Multicenter Study of VB-111 in Patients with Recurrent Glioblastoma Multiforme*. Sponsor: Vascular Biogenics Ltd.

NIH/OBA Receipt Date: 7-22-10. Not Selected for RAC Public Review: 8-12-10

1007-1052 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/Wiskott-Aldrich Syndrome (WAS)/In Vitro/Autologous CD34+ Cells from Bone Marrow/Lentiviral Vector/WAS Protein cDNA/Intravenous Infusion

Pai, Sung-Yun; Division of Hematology/Oncology Children's Hospital Boston, Harvard Medical School; Boston Massachusetts; *Pilot and Feasibility Study of Hematopoietic Stem Cell Gene Transfer for Wiskott-Aldrich Syndrome*.

NIH/OBA Receipt Date: 7-22-10. Publicly Reviewed at the September 2010 RAC meeting

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1007-1053 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other Diseases-Disorders/Autoimmune Disease/Myasthenia Gravis (MG)/Immunotherapy/In Vitro/Plasmid/Human Nicotinic Acetylcholine Receptor (AChR) Alpha Chain cDNA/Intramuscular Injection

Wolfe, Gil; University of Texas Southwestern Medical School; Dallas, Texas; *A Phase 1/2 Randomized, Blinded, Placebo-Controlled, Sequential Dose Escalation Study of the Safety and Pharmacodynamics of BHT-3034, an Acetylcholine Receptor Tolerizing Plasmid*. Sponsor: Bayhill Therapeutics, Inc.

NIH/OBA Receipt Date: 7-22-10. Publicly Reviewed at the September 2010 RAC meeting

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1007-1054 (Open) Gene Therapy/Phase III/Cancer/Leukemia/Pro-Drug/Elimination of Graft-vs.-Host Disease/In Vitro/Allogeneic T Lymphocytes/Retrovirus/Herpes Simplex Virus Thymidine Kinase cDNA/Ganciclovir/Nerve Growth Factor Receptor cDNA/Intravenous Infusion

Mehta, Jayesh; Northwestern University; Chicago, Illinois; *Randomized Phase III trial of Haploidentical HCT with or without an Add Back Strategy of HSV-Tk donor Lymphocytes in Patients with High Risk Acute Leukemia*. Sponsor: MolMed, S.p.A.

NIH/OBA Receipt Date: 7-22-10. Not Selected for RAC Public Review: 8-12-10

1007-1055 (Open) Gene Therapy/Phase II/Other Disorders/Autoimmune Disorder/Type I Diabetes Mellitus/Immunotherapy/In Vivo/Plasmid/Human Proinsulin Protein (h-proIns)/Intramuscular Injection

Gottlieb, Peter A.; University of Colorado Health Science Center; Aurora, Colorado; *A Phase II, Randomized Double-Blind, Placebo-Controlled Trial of the Safety and Efficacy of BHT-3021 (A Pro-Insulin Tolerizing Plasmid) in Patients Aged 5−18 with Recent Onset Type 1 Diabetes Mellitus*. Sponsor: Genetech, Inc.

NIH/OBA Receipt Date: 7-22-10. Not Selected for RAC Public Review: 8-12-10
Closed: 01-11-11, trial never initiated

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1007-1056 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Advanced Myeloma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Lentivirus/Alpha and Beta cDNAs/High Affinity T Cell Receptor Specific for MAGE-A3 or NY-ESO-1/Intravenous Infusion

Rapoport, Aaron; University of Maryland; Baltimore, Maryland; and Stadtmauer, Edward; University of Pennsylvania; Philadelphia, Pennsylvania; *A Phase I, Dual Cohort, Two-site, Clinical Trial Evaluating the Safety and Activity of Redirected Autologous T Cells Expressing a High Affinity TCR Specific for MAGEA3/6 or NYESO1 Administered Post ASCT in Patients with Advanced Myeloma*.

NIH/OBA Receipt Date: 7-22-10. Publicly Reviewed at the September 2010 RAC meeting

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1007-1057 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Metastatic Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Lentivirus/Alpha and Beta cDNAs/High Affinity T Cell Receptor Specific for MAGE-A3 or NY-ESO-1/Intravenous Infusion

Linette, Gerald P.; Washington University School of Medicine; St. Louis, Missouri; *Phase I Study to Assess the Safety and Activity of Enhanced TCR Transduced Autologous T cells against Cancer-Testis Antigens in Metastatic Melanoma*.

NIH/OBA Receipt Date: 7-22-10. Publicly Reviewed at the September 2010 RAC meeting

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1007-1058 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/mRNA Cleavage/In Vitro/Autologous CD4+ T Cells/Retrovirus/E. coli MazF Gene/Endoribonuclease/Intravenous Infusion

Jacobson, Jeffrey; Drexel University College of Medicine; Philadelphia, Pennsylvania; *A Phase I, Open Label, Dual Cohort, Single Center Study to Evaluate the Safety, Tolerability and Immunogenicity of Autologous CD4 T Cells Modified with a Retroviral Vector Expressing the MazF Endoribonuclease Gene in Patients with HIV*.

NIH/OBA Receipt Date: 7-22-10. Not Selected for RAC Public Review: 11-3-10

1007-1059 (Open) Gene Therapy/Phase I/Cancer/Unmutated IgV_H Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Brentjens, Renier; Memorial Sloan-Kettering Cancer Center, New York, New York; *A Phase I Trial of Pentostatin, Cyclophosphamide and Rituximab (PCR) Followed by Consolidation with Autologous T Cells Genetically Targeted to the B Cell Specific Antigen CD19 for Patients with Unmutated IgV_H Chronic Lymphocytic Leukemia*.

NIH/OBA Receipt Date: 7-22-10. Not Selected for RAC Public Review: 8-12-10

1007-1060 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/tumor Cells/Adenovirus/Interleukin-12 cDNA/ RheoSwitch® Therapeutic System/Intratumoral Injection

O'Day, Stephen; The Angeles Clinic and Research Center; Los Angeles, California; Hamid, Omid; The Angeles Clinic and Research Center; Santa Monica, California; Starodub, Alexander; Indiana University Health Goshen Cancer Center; Goshen, Indiana; Whitman, Eric D.; Atlantic Melanoma Center; Morristown, New Jersey; Schwartzentruber, Douglas; Goshen Center for Cancer Care; Goshen, Indiana; and Linette, Gerald; Washington University; St. Louis, Missouri; *Phase 1b Open Label, Single Arm, Multicenter Trial to Evaluate the Safety, Tolerance, Response Rate and Immunological and Other Biological Effects of Repeated Intratumoral Injections of Ad-RTS-IL-12 Vector Engineered to Express hIL-12 in Response to an Oral Activator Ligand, Both Administered in Intra-Patient Escalating Doses in Patients With Unresectable Stage III C or IV Malignant Melanoma*. Sponsor: Intrexon Co.

NIH/OBA Receipt Date: 7-22-10. Not Selected for RAC Public Review: 8-12-10

1007-1061 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other/Age-related Macular Degeneration (AMD)/Lentivirus/Endostatin Angiostatin cDNAs/ Subretinal Injection

Campochiaro, Peter A.; Johns Hopkins University School of Medicine; Baltimore, Maryland; *A Phase I Dose Escalation Safety Study of Subretinally Injected RetinoStat®, a Lentiviral Vector Expressing Endostatin and Angiostatin, in Patients with Advanced Neovascular Age-Related Macular Degeneration*. Sponsor: Oxford BioMedica UK Ltd

NIH/OBA Receipt Date: 7-22-10. Publicly Reviewed at the September 2010 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1008-1062 (Open) Gene Therapy/Phase I/Cancer/Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Lentivirus/CD19 Antigen Specific Chimeric Antigen Receptor (CAR)/Intravenous Injections

Popplewell, Leslie; City of Hope; Duarte, California; *Phase I/II Study of Cellular Immunotherapy Using Central Memory-Enriched CD8+ T Cells Lentivirally Transduced to Express a CD19-Specific Chimeric Immunoreceptor Following Peripheral Blood Stem Cell Transplantation for Patients with High-Risk Intermediate Grade B-Lineage Non-Hodgkin Lymphoma*.

NIH/OBA Receipt Date: 8-2-10. Not Selected for RAC Public Review: 8-23-10

1008-1063 (Open) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/GD-2-Specific scFvFc-Zeta T Cell Receptor/Intravenous Injections

Myers, Doug; University of Missouri; Kansas City, Missouri; *Phase I Study of Donor Derived, Multi-Virus-Specific, Cytotoxic T-Lymphocytes Redirected to the Tumor Marker GD2 in Patients with Relapsed/Refractory Neuroblastoma Post-Allogeneic Hematopoietic Stem Cell Transplantation with a Sub-Myeloablative Conditioning Regimen*.

NIH/OBA Receipt Date: 8-26-10. Not Selected for RAC Public Review: 9-17-10

1008-1064 (Open) Gene Therapy/Phase I/Monogenic disease/Late Infantile Neuronal Ceroid Lipofuscinoses/In Vivo/Brain/Adeno-Associated Virus 10/CLN2 cDNA/Intraparenchymal injection

Crystal, Ronald G; Weill Cornell Medical College; New York, New York; *Direct CNS Administration of a Replication Deficient Adeno-Associated Virus Gene Transfer Vector Serotype rh.10 Expressing the Human CLN2 cDNA to Children with Late Infantile Neuronal Ceroid Lipofuscinosis Using a Modified Administration Method*.

NIH/OBA Receipt Date: 8-27-10. Not Selected for RAC Public Review: 9-20-10

1009-1065 (Open) Gene Therapy/Phase II/Cancer/Metastatic Cancer/Immunotherapy/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/Alpha and Beta cDNAs/T Cell Receptor Specific for MAGE-A3/12/Intravenous Infusions

Rosenberg, Steven; National Institutes of Health; Bethesda, Maryland; *Phase II Study of Metastatic Cancer that Expresses MAGE-A3/12 Using Lymphodepleting Conditioning Followed by Infusion of Anti-MAGE-A3/12 TCR-Gene Engineered Lymphocytes*.

NIH/OBA Receipt Date: 9-2-10. Not Selected for RAC Public Review: 9-24-10

1009-1066 (Open) Gene Therapy/Phase I/Cancer/Hodgkin's Lymphoma/Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/Autologous or Syngeneic Activated T Lymphocytes/Retrovirus/CD30 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Heslop, Helen; Baylor College of Medicine; Houston, Texas; *Phase I Study of the Administration of T lymphocytes Expressing the CD30 Chimeric Antigen Receptor for Relapsed CD30+ Hodgkin's Lymphoma and CD30+ Non-Hodgkin's Lymphoma*.

NIH/OBA Receipt Date: 9-24-10. Not Selected for RAC Public Review: 10-19-10

1010-1067 (Open) Gene Therapy/Phase I/Monogenic Disease/β-Thalassemia/In Vitro/Autologous CD34+ Cells/Lentivirus/Human γ-Globin Gene/Intravenous

Persons, Derek; St. Jude Children's Research Hospital; Memphis, Tennessee; *A Pilot Feasibility Study of Gene Transfer for Patients with β-thalassemia Using a Self-Inactivating γ Globin Lentiviral Vector to Transduce Autologous CD34+ Hematopoietic Cells*.

NIH/OBA Receipt Date: 10-5-10. Not Selected for RAC Public Review: 10-27-10

1010-1068 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/Galactosialidosis/In Vivo/Adeno-Associated Virus 2-8/Protective Protein Cathepsin A/Intravenous

Nienhuis, Arthur; St Jude Children's Research Hospital; Memphis, Tennessee; *An Open Label Dose-Escalation Study of a Self Complementary Adeno-Associated Viral Vector (scAAV2/8-LP1-hPPCA) for Gene Transfer in Subjects with Galactosialidosis*.

NIH/OBA Receipt Date: 10-7-10. Publicly Reviewed at the December 2010 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1010-1069 (Open) Gene Therapy/Expanded Access/Cancer/Non-Small Cell Lung Cancer/Antisense/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid DNA/TGF-β/Intradermal Injection

Brandon, Donald M.; Innovative Research Center of California; and DiCarlo, Brian A.; Central Coast Medical Oncology Corporation; Santa Maria, California; *Expanded Access Study of Lucanix® (belagenpumatucel-L) Combination Therapy in Advanced Non-small Cell Lung Cancer*. Sponsor: NovaRx Corp

NIH/OBA Receipt Date: 10-7-10. Not Selected for RAC Public Review: 11-1-10

1010-1070 (Open) Gene Therapy/Phase I/Other Diseases-Disorders/Peripheral Artery Disease, Critical Limb Ischemia/In Vivo/Plasmid/Stromal Cell-Derived Factor (SDF-1) cDNA/Intramuscular injection

Kibbe, Melina R.; Northwestern Memorial Hospital; Chicago, Illinois; *A Phase I/II Randomized Double-Blind, Placebo Controlled Dose Escalation Study to Evaluate the Safety and Efficacy of JVS-100 Administered by Direct Intramuscular Injection to Cohorts of Adults with Critical Limb Ischemia*. Sponsor: Juventas Therapeutics, Inc.

NIH/OBA Receipt Date: 10-12-10. Not Selected for RAC Public Review: 11-2-10

1010-1071 (Open) Gene Therapy/Phase I/Cancer/Synovial Sarcoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Lentivirus/T Cell Receptor alpha and beta cDNAs Chains/High Affinity T Cell Receptor Specific for NY-ESO-1/Intravenous Infusion

Mackall, Crystal; and Linette, Gerald P.; Washington University School of Medicine; St. Louis, Missouri; *A Pilot Study of Genetically Engineered NY-ESO-1 Specific (c259) T Cells in HLA-A2+ Patients with Synovial Sarcoma*.

NIH/OBA Receipt Date: 10-12-10. Not Selected for RAC Public Review: 11-2-10

1010-1072 (Open) Gene Therapy/Phase I/Cancer/Mesothelioma/Immunotherapy/In Vitro/mRNA/α-mesothelin-scFv with Signaling Domains Comprised of TCRζ, CD28, and 4-1BB cDNA/Intravenous Injection

Haas, Andrew; University of Pennsylvania Medical Center; Philadelphia, Pennsylvania; *Phase I Clinical Trial of Autologous Mesothelin Redirected T Cells*.

NIH/OBA Receipt Date: 10-12-10. Not Selected for RAC Public Review: 11-2-10

1010-1073 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Monogenic Disease/Childhood Cerebral Adrenoleukodystrophy/In Vitro/Autologous CD34+ Cells/Lentivirus/ABCD-1 Gene (ALD protein)/Intravenous

Williams, David; Children's Hospital Boston; Boston, Massachusetts; *An Open Label, Non-Randomized, Single Dose, Multi-Center Phase 2/3 Study of the Safety and Efficacy of Lenti-D Modified Autologous Stem Cells (Lenti-D Drug Product) for the Treatment of Subjects with Childhood Cerebral Adrenoleukodystrophy (CCALD)*. Sponsor: bluebird bio, Inc.

NIH/OBA Receipt Date: 10-12-10. Publicly Reviewed at the December 2010 RAC meeting

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1010-1074 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Diseases/Becker Muscular Dystrophy, Sporadic Inclusion Body Myositis/In Vivo/Adeno-Associated Virus/Serotype 1/Follistatin Gene/Intramuscular Injection

Mendell, Jerry R.; The Research Institute at Nationwide Children's Hospital; Columbus, Ohio; *Phase I Clinical Intramuscular Gene Therapy of rAAV.FS344 Trial to Patients with Becker Muscular Dystrophy and Sporadic Inclusion Body Myositis.* Sponsors: Parent Project for Muscular Dystrophy and The Myositis Association

NIH/OBA Receipt Date: 10-12-10. Publicly Reviewed at the December 2010 RAC meeting

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1010-1075 (Open) Gene Therapy/Phase I/Cancer/Carcinoma pancreas /Immunotherapy/In Vivo/Listeria monocytogenes/Mesothelin cDNA/Intravenous Injection/Autologous Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)/Intradermal injections

Le, Dung; The Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins; Baltimore, Maryland; Crocenzi, Todd S.; Providence Portland Medical Center; Portland, Oregon; Greten, Tim F.; National Institutes of Health; Bethesda, Maryland; Morse, Michael A.; Duke University Medical Center; Durham, North Carolina; Wang-Gillam; Andrea Washington University School of Medicine; St. Louis, Missouri; Picozzi, Vincent J.; Virginia Mason Medical Center; Seattle, Washington; Fine, Robert L.; Columbia University Medical Center; New York, New York; Zeh, Herbert J.; University of Pittsburgh Medical Center; Pittsburgh, Pennsylvania; Lauer Andreas K.; Oregon Health & Science University; Portland, Oregon; Cohen, Deirdre J.; New York University; New York, New York; and Springett Gregory; H. Lee Moffitt Cancer Center & Research Institute; Tampa, Florida; *A Phase 2, Randomized, Multicenter, Open-Label Study of the Efficacy and Immune Response of the Sequential Administration of GVAX Pancreas Vaccine (with Cyclophosphamide) Alone or Followed by CRS-207 Adults with Metastatic Pancreatic Adenocarcinoma (Protocol ADU-CL-01)*. Sponsor: Aduro BioTech

NIH/OBA Receipt Date: 10-12-10. Not Selected for RAC Public Review: 11-2-10

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1010-1076 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vitro/Lentivirus/α-folate Receptor-scFv with Signaling Domains Comprised of TCRζ, and 4-1BB cDNA/Intravenous Injection

Coukos, George; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; *Phase I Clinical Trial of Autologous Alpha-Folate Receptor Redirected T Cells Administered Intravenously in Ovarian Cancer Patients*.

NIH/OBA Receipt Date: 10-12-10. Publicly Reviewed at the December 2010 RAC meeting

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1010-1077 (Withdrawn from review; Replaced by 1102-1087) Gene Therapy/Phase II/Cancer/Stage IV Adenocarcinoma of the Lung/Immunotherapy/In Vitro/Adenovirus/Serotype 5/CCL-21 cDNA/Intramuscular Injection

Gray, Jhanelle; Moffitt Cancer Center; Tampa, Florida; *A Randomized Phase ll Trial Using a GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) Vaccine in Combination with CCL21 for Patients with Stage IV Adenocarcinoma of the Lung*.

NIH/OBA Receipt Date: 10-18-10.

1011-1078 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta cDNAs Chains/Intravenous Infusion

Ribas, Antoni; University of California, Los Angeles; Los Angeles, California; *MART-1 F5 TCR Engineered Adoptive Cell Transfer Therapy with CTLA4 Blockade*.

NIH/OBA Receipt Date: 10-27-10. Not Selected for RAC Public Review: 11-23-10

1101-1079 (Open) Gene Therapy/Phase I/Cancer/Colorectal, Gastric, or Pancreatic/Immunotherapy/In Vivo/Vaccinia (MVA)/p53 cDNA/Subcutaneous Injections

Ellenhorn, Joshua D.I.; and Chung, Vincent; City of Hope; Duarte, California; *Phase I Study of an MVA Vaccine Targeting p53 in Cancer*.

NIH/OBA Receipt Date: 11-09-10. Not Selected for RAC Public Review: 12-10-10

1101-1080 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous Lymphocytes/Adenovirus/Serotype 5/cDNA of Engineered Zinc Finger Nucleases (ZFNs) Targeting the Human CCR5 Locus (SB-728)/Intravenous

Mitsuyasu, Ronald; University of California, Los Angeles; Los Angeles, California; *A Phase 1/2, Open Label, Single Infusion Study of Autologous T Cells Genetically Modified at the CCR5 Gene by Zinc Finger Nucleases (SB-728-T) in HIV Infected Subjects*. Sponsor: Sangamo Biosciences, Inc.

NIH/OBA Receipt Date: 11-16-10. Not Selected for RAC Public Review: 12-15-10

1101-1081 (Open) Gene Therapy/Phase II/Cancer/Stage IIIc Ovarian Cancer/Immunotherapy/In Vitro/Plasmid DNA/Electroporation/GM-CSF cDNA/bi-shRNA^{furin}/Intradermal Injections

Barve, Minal; Texas Oncology, P.A.; Dallas, *Texas; Randomized Phase II Trial of Adjuvant bi-shRNA^{furin} and GMCSF Augmented Autologous Tumor Cell Vaccine (FANG™) for High Risk Stage IIIc Ovarian Cancer*. Sponsor: Gradalis, Inc.

NIH/OBA Receipt Date: 11-23-10. Not Selected for RAC Public Review: 12-22-10

1012-1082 (Open) Gene Therapy/Phase II/Cancer/Cervical Cancer/Immunotherapy/In Vivo/*Listeria monocytogenes*/Human Papilloma Virus E7 Gene/Intravenous Administration

Huh, Warner K.; University of Alabama at Birmingham; Birmingham, Alabama; Mannel, Robert; University of Oklahoma Health Sciences Center; Oklahoma City, Oklahoma; Boardman, Cecelia H.; Virginia Commonwealth University; Richmond, Virginia; Burke, James J., II; Memorial Health University Medical Center; Savannah, Georgia; and Mutch, David G.; Washington University School of Medicine; St. Louis, Missouri; *A Phase II Evaluation of ADXS11-001 (NSC #752718, IND # 13,712) in the Treatment of Persistent or Recurrent Squamous or Non-Squamous Cell Carcinoma of the Cervix*. Sponsor: Advaxis Inc.

NIH/OBA Receipt Date: 12-15-10. Not Selected for RAC Public Review: 01-12-11

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1012-1083 (Open) Gene Therapy/Phase III/Cancer/Prostate/ Pro-Drug/Valacyclovir/In Vivo/Adenovirus/ Herpes Simplex Thymidine Kinase cDNA/Intratumoral Injection

Buyyounuouski, Mark K.; Fox Chase Cancer Center; Philadelphia, Pennsylvania; DeWeese, Theodore L.; Johns Hopkins University; Baltimore, Maryland; Blute, Michael L.; U Mass Memorial Medical Center; Worcester, Massachusetts; Zelefsky, Michael J.; Memorial Sloan-Kettering Cancer Center; New York, New York; McLeod, David G.; Walter Reed Army Medical Center; Washington, DC; Schroeder, Thomas M.; University of New Mexico Cancer Center; Albuquerque, New Mexico; Sukin, Steven, Texas Urology Specialists; Tomball, Texas; and Beyer, David C.; Arizona Oncology Services Foundation; Phoenix, Arizona; *A Randomized Controlled Trial of ProstAtak™ as Adjuvant to Up-Font Radiation Therapy for Localized Prostate Cancer*. Sponsor: Advantagene, Inc.

NIH/OBA Receipt Date: 12-16-10. Not Selected for RAC Public Review: 01-10-11

1012-1084 (Open) Gene Therapy/Phase I/Cancer/Immunotherapy/In Vitro/Allogeneic K562 Cell/Plasmid DNA/Granulocyte-macrophage Colony Stimulating Factor cDNA/Intradermal Injection

Schrump, David; National Institutes of Health; Bethesda, Maryland; *Adjuvant Allogeneic Tumor Cell Vaccine with Metronomic Oral Cyclophosphamide and Celecoxib in Patients Undergoing Resection of Sarcomas, Melanomas, Germ Cell Tumors, or Epithelial Malignancies Metastatic to Lungs, Pleura, or Mediastinum*.

NIH/OBA Receipt Date: 12-23-10. Not Selected for RAC Public Review: 01-18-11

1012-1085 (Open) Gene Therapy/Phase I/Monogenic Disease/Stargardt's Macular Degeneration (SMD)/Lentivirus/Retina-Specific ABC Transporter (ABCR) cDNA/Subretinal Injection

Francis, Peter J.; Oregon Health and Science University; Portland, Oregon; and Wilson, David J.; Casey Eye Institute; Portland, Oregon; *A Phase I/IIa Dose Escalation Safety Study of Subretinally Injected StarGen™, Administered to Patients with Stargardt's Macular Degeneration*. Sponsor: Oxford BioMedica (UK) Ltd

NIH/OBA Receipt Date: 12-23-10. Not Selected for RAC Public Review: 01-21-11

1101-1086 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Peripheral Blood T Lymphocytes/Lentivirus/Alpha and Beta cDNAs/T Cell Receptor Specific for Tyrosinase/Intravenous Infusions

Nishimura, Michael I.; Loyola University School of Medicine; Maywood, Illinois; *Transfer of Genetically Engineered Lymphocytes in Melanoma Patients: A Phase I Dose Escalation Study*. Sponsor: Lentigen Corporation

NIH/OBA Receipt Date: 01-10-11. Not Selected for RAC Public Review: 02-01-11

1101-1087 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Stage IV Adenocarcinoma of the Lung/Immunotherapy/In Vitro/Adenovirus/Serotype 5/CCL-21 cDNA/Intramuscular Injection

Gray, Jhanelle; Moffitt Cancer Center; Tampa, Florida; *A Randomized Phase I/II Trial Using a GM-CSF-Producing and CD40L-Expressing Bystander Cell Line (GM.CD40L) Vaccine in Combination with CCL21 for Patients with Stage IV Adenocarcinoma of the Lung*.

NIH/OBA Receipt Date: 01-11-11. Publicly Reviewed at the March 2011 RAC meeting

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1101-1088 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Malignant Pleural Effusions/Vaccinia Virus/Vector-Directed Tumor Lysis/*Renilla* Luciferase-Green Fluorescent Protein Fusion RUC-GFP, β-galactosidase and β-glucuronidase cDNAs/Intrapleural Administration

Rusch, Valerie; Memorial Sloan-Kettering Cancer Center; New York, New York; *Phase I Study of Intra-pleural Administration of GL-ONC1, a Genetically Modified Vaccinia Virus, in Patients with Malignant Pleural Effusion: Primary, Metastases, and Mesothelioma*. Sponsor: Genelux Corporation.

NIH/OBA Receipt Date: 01-31-11. Publicly Reviewed at the June 2011 RAC meeting

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1101-1089 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Locoregionally Advanced Head and Neck Carcinoma/Vaccinia Virus/Vector-Directed Tumor Lysis/*Renilla* Luciferase-Green Fluorescent Protein Fusion RUC-GFP, β-galactosidase and β-glucuronidase cDNAs/Intravenous Injection

Mell, Loren K.; University of California, San Diego; La Jolla, California; *Phase I Trial of Attenuated Vaccinia Virus (GL-ONC1) Delivered Intravenously with Concurrent Cisplatin and Radiotherapy in Patients with Locoregionally Advanced Head and Neck Carcinoma*. Sponsor: Genelux Corporation.

NIH/OBA Receipt Date: 01-31-11. Publicly Reviewed at the June 2011 RAC meeting

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1102-1090 (Open) Gene Therapy/Phase I/Cancer/Peritoneal Carcinoma of Colorectal Cancer/Immunotherapy/In Vivo/DNA Complex with PEG-PEI-Cholesterol/Interleukin-12 cDNA/Intraperitoneal Injection

Brown, Charles Komen; Cancer Treatment Centers of America at Midwestern Regional Medical Center; Zion, Illinois; *A Phase I/II Study of the Safety and Biological Activity of Intraperitoneal EGEN-001 (IL-12 Plasmid Formulated with PEG-PEI-Cholesterol Lipopolymer) Administered Alone and in Combination with Standard Chemotherapy in Colorectal Peritoneal Carcinomatosis Patients Who Had Previously Received Cytoreductive Surgery plus HIPEC Therapy*. Sponsor: EGEN, Inc.

NIH/OBA Receipt Date: 02-18-11. Not Selected for RAC Public Review: 03-14-11

1102-1091 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Acute Lymphocytic Leukemia/Immunotherapy/In Vitro/Allogeneic Epstein Bar virus-specific T Lymphocytes/Retrovirus/CD19 antigen specific-Zeta T Cell Receptor/Intravenous Injections

Kernan, Nancy A.; Memorial Sloan-Kettering Cancer Center; New York, New York; *A Phase I Dose Escalation Trial Using In Vitro Expanded Allogeneic Epstein-Barr Virus Specific Cytotoxic T-Lymphocytes (EBV-CTLs) Genetically Targeted to the B-Cell Specific Antigen CD19 for In Vivo Treatment Of Residual Or Relapsed Acute Lymphoblastic Leukemia After Allogeneic Hematopoietic Progenitor Cell Transplantation*.

NIH/OBA Receipt Date: 02-18-11. Publicly Reviewed at the June 2011 RAC meeting

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1102-1092 (Open) Gene Therapy/Phase I/Cancer/Hematological Malignancies/Elimination of Graft versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/Inducible Caspase 9 Suicide Gene/AP1903/Intravenous

Brenner, Malcolm and Heslop, Helen; Baylor College of Medicine; Houston, Texas; *Administration of Haploidentical Donor T Cells Gene Transduced with the Inducible Caspase-9 Suicide Gene (DOTTI)*.

NIH/OBA Receipt Date: 02-22-11. Not Selected for RAC Public Review: 03-15-11

1102-1093 (Open) Gene Therapy/Phase II/Cancer/Cervical/Immunotherapy/In Vivo/Plasmid DNA/HPV16 E6-E7 Fusion Protein cDNA/HPV18 E6-E7 Fusion Protein cDNA/Intramuscular Injection in Combination with Electroporation

Trimble, Cornelia; Johns Hopkins Medical Institutions; Baltimore, Maryland; Cestero, Ramon M.; Arrowhead Regional Medical Center; Colton, California; Parker, Robert L., Jr.; Lyndhurst Gynecologic Associates; Winston-Salem, North Carolina; Kells, David N.; Boojum Obstetrics and Oncology; Chandler, Arizona; Graul, Elizabeth S.; Jean Brown Research, Phase II Women's Center; Murray, Utah; Odom, Albert E.; South Carolina Clinical Research; Columbia, South Carolina; Garcia, Francisco A.; University of Arizona; Tucson, Arizona; Aqua, Keith A.; Visions Clinical Research; Boynton Beach, Florida; Goldberg, Cynthia C.; Visions Clinical Research – Tucson; Tucson, Arizona; Huey, James; HWC Women's Research Center; Englewood, Ohio; Chu, Christina S.; University of Pennsylvania Health System; Smith-Nguyen, Gioi; Grossmont Center for Clinical Research; La Mesa, California; Rice, James P.; Valley Women's Clinic; Renton, West Virginia; Kirkpatrick, Helena P.; Magnolia OB/GYN Research Center; Myrtle Beach, South Carolina; Einstein, Mark H.; Montefiore Medical Center; Bronx, New York; Smith, Katie M.; The University of Oklahoma; Oklahoma City, Oklahoma; Swor, G. Michael; Physician Care Clinical Research; Sarasota, Florida; Chatwani, Ashwin; Temple University; Philadelphia, Pennsylvania; Edwards, Lance Suffolk Obstetrics & Gynecology; Port Jefferson, New York; Twede, Michael L.; Salt Lake Women's Center, PC; Sandy, Utah; Zedler, Peter A. Virginia Women's Center; Richmond, Virginia; Harris, Micah S.; Women's Health Research; Phoenix, Arizona; Beyerlein, Richard A.; Pacific Women's Center, LLC; Eugene, Oregon; Grube, Jennifer; Red Rocks OB/GYN; Lakewood, Colorado; Hardy, Ronald D.; North Spokane Women's Health; Spokane, Washington; Young, David B.; Central Utah Clinic Women's Center; Pleasant Grove, Utah; Romaguera, Josefina; University of Puerto Rico School of Medicine; San Juan, Puerto Rico; and Osman, Khadra M.; KO Clinical Research, LLC; Ft. Lauderdale, Florida; *Phase II Placebo-controlled Study of VGX-3100, (HPV16 E6/E7, HPV18 E6/E7 DNA Vaccine) Delivered IM Followed By Electroporation (Ep) With Cellectra®-5p For The Treatment Of Biopsy-Proven Cin 2/3 Or Cin 3 With Documented HPV 16 or 18*. Sponsor: Inovio Pharmaceuticals, Inc.

NIH/OBA Receipt Date: 02-28-11. Not Selected for RAC Public Review: 03-21-11

1103-1094 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Allogeneic Melanoma Tumor Cells/Lethally Irradiated/Genomic DNA (no vector)/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor cDNA/Intradermal Injections

Sharfman, William H.; The Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins; Lutherville, Maryland; *A Feasibility and Toxicity Study of a GM-CSF Secreting Allogeneic Melanoma Vaccine Administered Alone or in Combination with Cyclophosphamide in Subjects with Surgically Resected At-risk Melanoma*.

NIH/OBA Receipt Date: 03-02-11. Not Selected for RAC Public Review: 03-23-11

1103-1095 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Glioma/Immunotherapy/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/T Cell Receptor Specific for Endothelial Growth Factor Receptor variant III (EGFRvIII)/Intravenous Infusions

Rosenberg, Steven; National Institutes of Health; Bethesda, Maryland; *A Phase I/II Study of the Safety and Feasibility of Administering T Cells Expressing Anti-EGFRvIII Chimeric Antigen Receptor to Patients with Malignant Gliomas Expressing EGFRvIII*.

NIH/OBA Receipt Date: 03-08-11. Publicly Reviewed at the June 2011 RAC meeting

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1103-1096 (Open) Gene Therapy/Phase I/Cancer/HER2+ Cancers/Immunotherapy/In Vivo/ Adenovirus Vector/Human Epidermal Growth Factor Receptor-2/Subcutaneous Injection

Morse, Michael A.; Duke University; Durham, North Carolina; *A Phase I/II Study to Evaluate the Antitumor Activity and Safety of Etbx-021, an Adenoviral Vector Encoding the Her2 Extracellular Domain and Transmembrane Region, in Patients with Locally Advanced or Metastatic Human Epidermal Growth Factor Receptor 2-Positive (Her2+) Cancers Including Breast Cancer*. Sponsor: Etubics Corporation.

NIH/OBA Receipt Date: 03-08-11. Not Selected for RAC Public Review: 04-01-11

1103-1097 (Open) Gene Therapy/Phase I/Cancer/NY-ESO-1 Expressing Metastatic Cancer/Immunotherapy/In Vitro/Autologous CD8+ Tumor Infiltrating Lymphocytes/Retrovirus/T Cell Receptor alpha and beta cDNAs Chains/Interleukin-12 cDNA/Intravenous Injections.

Rosenberg, Steven; National Institutes of Health; Bethesda, Maryland; *Phase I/II Study of Metastatic Cancer that Expresses NY-ESO-1 Using Lymphodepleting Conditioning Followed by Infusion of Gene Engineered Lymphocytes Cotransduced with Genes Encoding IL-12 and Anti-NY ESO-1 TCR*.

NIH/OBA Receipt Date: 03-15-11. Not Selected for RAC Public Review: 04-05-11

1103-1098 (Open) Gene Therapy/Phase I/Cardiovascular Disease/Ischemic Heart Disease Heart Failure/Non-viral Gene Therapy/In Vivo/ Plasmid DNA/Stromal Cell-Derived Factor-1/ Retrograde Infusion

Patel, Amit; University of Utah; Salt Lake City, Utah; *A Phase Ib, Open-Label Dose-Escalation Study to Evaluate the Safety of JVS-100 Administered by Retrograde Delivery to Cohorts of Adults with Ischemic Heart Failure*. Sponsor: Juventas Therapeutics, Inc.

NIH/OBA Receipt Date: 03-21-11. Not Selected for RAC Public Review: 04-11-11

1103-1099 (Open) Gene Therapy/Phase I/Other Diseases/Coronary Artery Disease/Atherosclerosis/ Myocardial Ischemia/Plasmid DNA/Hepatocyte Growth Factor/ Intramyocardial Injections

Losordo, Douglas; Northwestern University; Chicago, Illinois; *A Phase I/II, Open Label, Dose-Escalation Study to Assess the Safety and Tolerability of VM202 in Subjects with Chronic Refractory Myocardial Ischemia*. Sponsor: ViroMed Co., Ltd.

NIH/OBA Receipt Date: 03-21-11. Not Selected for RAC Public Review: 04-11-11

1104-1100 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Malignant Gliomas/Immunotherapy/In Vivo/Cytotoxic Agent/HSV-1/ Peritumoral Injection

Chiocca, E. A.; The Ohio State University; Columbus, Ohio; *A Phase I Study of the Treatment of Recurrent Malignant Glioma with rQNestin34.5v2, a Genetically Engineered HSV-1 Virus, and immunomodulation with Cyclophosphamide*.

NIH/OBA Receipt Date: 04-01-11. Publicly Reviewed at the June 2011 RAC meeting

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1104-1101 (Open; RAC reviewed with recommendations) Gene Therapy/Phase III/Cancer/Prostate/Immunotherapy/In Vivo/Viral vector/Prostate, Prostate Cancer Specific Antigens/Subcutaneous Injections

Gulley, James L.; National Institutes of Health; Bethesda, Maryland; Zhou, Shaw; Pinellas Urology, Inc.; St. Petersburg, Florida; Hodge, Gameel B., Jr.; Lakeland Regional Cancer Center; Lakeland, Florida; Bidair, Mohammed; San Diego Clinical Trials; San Diego, California; Gitelman, Marc C.; South Florida Medical Research; Miami, Florida; Gabrail, Nashat Y.; Gabrail Cancer Center; Canton, Ohio; Henderson, Ralph J.; Regional Urology, LLC; Shreveport, Louisiana; Concepcon Raaoul S.; Urology Associates, P.C.; Nashville, Tennessee; Gambla, Michael T.; Central Ohio Urology Group; Columbus, Ohio; Sieber, Paul R. Urological Associates of Lancaster; Lancaster, Pennsylvania; Ruiz, Henry E.; Urology Associates of South Texas; McAllen, Texas; Kaminetsky, Jed C.; University Urology Associates; New York, New York; Chu, Franklin M.; San Bernardino Urological Associates; San Bernardino, California; Clark, William R.; Alaska Clinical Research Center, LLC; Anchorage, Alaska; Richards, Donald A.; Texas Oncology – Tyler; Tyler, Texas; Cartwright, Thomas H.; Ocala Oncology Center; Ocala, Florida; Shore, Neal D. Carolina Urologic Research Center; Myrtle Beach, South Carolina; Green, Robert J.; Palm Beach Cancer Institute; West Palm Beach, Florida; Lipsitz, David U. NorthEast Urology Research; Concord, North Carolina; Anderson, Thomas C. Texas Oncology – Bedford; Bedford, Texas; Goldfischer, Evan Robert; Premier Medical Group of the Hudson Valley; Poughkeepsie, New York; Shah, Shreya; Alta Bates Summit Comprehensive Cancer Center; Berkley, California; Oselinsky, David; Mount Nittany Medical Center Health Services, Inc.; State College, Pennsylvania; Mehta, Amit R.; Regional Cancer Care; Durham, North Carolina; Merrick, Gregory S.; Wheeling Jesuit University; Wheeling, West Virginia; Kantoff, Philip W.; Dana-Farber Cancer Institute; Boston, Massachusetts; Fiorillo, Joseph A. Willamette Valley Cancer Institute and Research Center; Eugene, Oregon; Nordquist, Luke T.; Urology Cancer Center and GU Research Network; Omaha, Nebraska; Clark, Randil L.; North Idaho Urology; Coeur D'Alene, Idaho; Miller, Alan K.; Manatee Medical Research Institute, LLC; Bradenton, Florida; Godschalk, Michael; Hunter Holmes McGuire Medical Center; Richmond, Virginia; Corman, John; Virginia Mason Medical Center; Seattle, Washington; Jacobson, Jeffrey; Drexel University; Philadelphia, Pennsylvania; Belkoff, Laurence H.; Urologic Consultants of Southeastern Pennsylvania; Bala Cynwyd, Pennsylvania; Mackintosh, F. Roy; VA Sierra Nevada Health Care System; Reno, Nevada; Mogul, Mark J.; Presbyterian Cancer Center; Charlotte, North Carolina; Bracken, R. Bruce; University of Cincinnati; Cincinnati, Ohio; Coffield, K. Scott; Texas A & M University, Temple, Texas; Vogelzang, Nicholas J.; Comprehensive Cancer Centers of Nevada; Las Vegas, Nevada; Lewis, Brian; Tulane University; New Orleans, Louisiana; Perry, David J. Washington Hospital Center; Washington, DC; Grunberger, Ivan; Brooklyn Urology Research Group; Brooklyn, New York; Bailen, James L.; First Urology, PSC; Jeffersonville, Indiana; Richardson, Stephen F. Salt Lake Research; Salt Lake City, Utah; Rainwater, Leslie Mark; St. Alexius Medical Center; Bismarck, North Dakota; Rosenberg, Steven J.; The Iowa Clinic; West Des Moines, Iowa; Celano, Paul; Greater Baltimore Medical Center; Baltimore, Maryland; Mohebtash, Mahsa; Medstar Union Memorial Hospital Cancer Center; Baltimore, Maryland; Velez-Cortes, Hector A.; Ponce School of Medicine; Ponce, Puerto Rico; Feliciano, Lourdes J.; Alliance for Research and Knowledge; San Juan, Puerto Rico; McLeod, David G.; Walter Reed Army Medical Center; Bethesda, Maryland; and Flaig, Thomas W.; University of Colorado, Denver; Aurora, Colorado; *A Randomized, Double-blind, Phase 3 Efficacy Trial of PROSTVAC ± GM-CSF in Men With Asymptomatic or Minimally Symptomatic Metastatic, Castrate-Resistant Prostate Cancer*. Sponsor: BN ImmunoTherapeutics.

NIH/OBA Receipt Date: 04-07-11. Publicly Reviewed at the June 2011 RAC meeting

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1104-1102 (Open) Gene Therapy/Phase I/Autosomal Recessive Congenital Disorder/Retinitis Pigmentosa /Immunotherapy/In Vivo/Lentivirus/Usher Syndrome Type 1B, MYO7A/Subretinal Injections

Francis, Peter J.; Oregon Health and Science University; Portland Oregon; *A Phase I/IIa Dose Escalation Safety Study of Subretinally Injected UshStat®, Administered to Patients with Retinitis Pigmentosa Associated with Usher Syndrome Type 1B*. Oxford BioMedica (UK) Ltd.

NIH/OBA Receipt Date: 04-12-11. Not Selected for RAC Public Review: 05-03-11

1104-1103 (Open) Gene Therapy/Phase I/Cancer/Solid Tumors/Immunotherapy/In Vivo/Adenovirus/ GM-CSF/Intratumoral Injection

Mims, Martha; Baylor College of Medicine; Houston, Texas; *GOAT; Phase I Single-Center Open Label Dose Escalation Study of CGTG-102, a GM-CSF Encoding Oncolytic Adenovirus, for Therapy of Advanced Cancers*. Sponsor: Oncos Therapeutics Ltd.

NIH/OBA Receipt Date: 04-12-11. Not Selected for RAC Public Review: 05-03-11

1104-1104 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/Obesity with *MC4R* Function-Altering Mutations or Prader-Willi Syndrome /In Vivo/Autologous Neurons/Adeno-associated Virus/Brain Derived Neurotrophic Factor (BDNF)/Stereotactic Hypothalamic Injection

Han, Joan C. and Yanovski, Jack A.; National Institutes of Health; Bethesda, Maryland; *AAV-BDNF Gene Therapy of Obesity*.

NIH/OBA Receipt Date: 04-12-11. Publicly Reviewed at the June 2011 RAC meeting

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1104-1105 (Open) Gene Therapy/Phase I/Monogenic Disease/Fanconi Anemia/In Vitro/CD34+ Autologous Cells/Lentivirus/Fanconi Anemia Complementation Group A cDNA/Intravenous

Goebel, W. Scott; Indiana University School of Medicine; Indianapolis, Indiana; *A Phase I/II Study of Lentiviral Gene Transfer for the Treatment of Fanconi Anemia Type A*.

NIH/OBA Receipt Date: 04-12-11. Not Selected for RAC Public Review: 05-03-11

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1104-1106 (Open; RAC reviewed with recommendations) Gene Therapy/Non-therapeutic (Healthy Volunteers)/Human Immunodeficiency Virus/In Vivo/Plasmid/Modified Vaccinia Virus Ankara (MVA)/HIV-1 Gag/Pol and Nef/Tat/Vif/Env cDNAs/GM-CSF cDNA/Intramuscular Injections

Buchbinder, Susan; San Francisco Department of Public Health; San Francisco, California; *A Phase 1 Placebo Controlled Clinical Trial to Evaluate the Safety and Immunogenicity of a Prime-boost Vaccine Regimen of GEO-D03 DNA and MVA/HIV62B Vaccines in Healthy, HIV-1-Uninfected Vaccinia Naïve Adult Participants*. Sponsor: HIV Vaccine Trials Network.

NIH/OBA Receipt Date: 04-12-11. Publicly Reviewed at the June 2011 RAC meeting

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1104-1107 (Open) Gene Therapy/Phase II/Cancer/Multiple Myeloma/Immunotherapy/In Vivo/Allogeneic Vaccine/Myeloma, GM-CSF/Intradermal Injections

Borrello, Ivan; Johns Hopkins University; Baltimore, Maryland; *Administration of an Allogeneic Myeloma GM-CSF Vaccine in Conjunction with a Lenalidomide containing Regimen in Myeloma Patients with Near Complete Remission*. Sponsor: Celgene Corporation.

NIH/OBA Receipt Date: 04-14-11. Not Selected for RAC Public Review: 05-05-11

1104-1108 (Open) Gene Therapy/Phase II/Cancer/ Hepatocellular Carcinoma /Immunotherapy/In Vivo/Vaccinia Virus/GM-CSF/Humanized Escherichia coli β-galactosidase/IT injection and intravenous (IV) infusion

Reid, Tony; University of California, San Diego; San Diego, California; Kothary, Nishita; Stanford University School of Medicine; Stanford, California; Baron, Ari; California Pacific Medical Center; San Francisco, California; Nieva, Jorge J.; Billings Clinical Research Center; Billings, Montana; Frenette, Catherine T.; The Methodist Hospital; Houston, Texas; Gabrail, Nashat Y.; Gabrail Cancer Center; Canton, Ohio; and Post, Anthony Benjamin; University Hospitals Case Medical Center; Cleveland, Ohio; *A Phase 2b Randomized Single-Blinded Trial of JX-594 (vaccinia GM-CSF/TK-deactivated virus) Plus Best Supportive Care Versus Placebo Plus Best Supportive Care In Patients With Advanced Hepatocellular Carcinoma Who Have Failed Sorafenib Treatment*. Sponsor: Jennerex Biotherapeutics

NIH/OBA Receipt Date: 04-14-11. Not Selected for RAC Public Review: 05-12-11

1105-1109 (Open) Gene Therapy/Phase II/Cancer/Pancreas/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Plasmid/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Herman, Joseph M.; Johns Hopkins University; Baltimore, Maryland; *A Phase II Multicenter Study Evaluating an Allogeneic GM-CSF-Transduced Pancreatic Tumor Cell Vaccine (GVAX) and Low Dose Cyclophosphamide Integrated with Fractionated Stereotactic Body Radiation Therapy (SBRT) and GTX chemotherapy in Patients with Resected Adenocarcinoma of the Pancreas*.

NIH/OBA Receipt Date: 05-04-11. Not Selected for RAC Public Review: 05-25-11

1105-1110 (Open) Gene Therapy/Phase II/Cancer/Stage IIIc Ovarian Cancer/Immunotherapy/In Vitro/Plasmid DNA/Electroporation/GM-CSF cDNA/ bi-shRNA^{furin}/Intradermal Injections

Barve, Minal; Tex Oncology, P.A.; Dallas, Texas; *Phase II Trial of Adjuvant bi-shRNA^{furin} and GMCSF Augmented Autologous Tumor Cell Vaccine (FANG™) Integrated with Chemotherapy for Patients with Recurrent Cisplatinum Sensitive Ovarian Cancer Participating in Study CL-PTL 105*. Sponsor: Gradalis, Inc.

NIH/OBA Receipt Date: 05-13-11. Not Selected for RAC Public Review: 06-07-11

1105-1111 (Open) Gene Therapy/Phase II/Cancer/Stage IIIc Ovarian Cancer/Immunotherapy/In Vitro/Plasmid DNA/Electroporation/GM-CSF cDNA/ bi-shRNA^{furin}/Intradermal Injections

Barve, Minal; Tex Oncology, P.A.; Dallas, Texas; *Phase II Trial of Adjuvant bi-shRNA^{furin} and GMCSF Augmented Autologous Tumor Cell Vaccine (FANG™) Integrated with Bevacizumab for Patients with Recurrent/Refractory Ovarian Cancer Participating in Study CL-PTL 105*. Sponsor: Gradalis, Inc.

NIH/OBA Receipt Date: 05-13-11. Not Selected for RAC Public Review: 06-07-11

1106-1112 (Open) Gene Therapy/Phase I/Monogenic Disease/Hemophilia A/In Vitro/CD34+ Autologous Cells/Lentivirus/Factor VIII cDNA/Intravenous

Doering, Christopher and Kempton, Christine; Emory University School of Medicine; Atlanta, Georgia; *Gene Therapy for Hemophilia A Incorporating a High Expressing Factor VIII Transgene and Hematopoietic Stem Cell Transplantation: a Pilot Study*. Sponsor: Lentigen Corporation.

NIH/OBA Receipt Date: 06-20-11. Not Selected for RAC Public Review: 07-12-11

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1107-1113 (Open) Gene Therapy/Phase I/II/Cancer/Colorectal/Immunotherapy/In Vivo/Vaccinia Virus/GM-CSF/Humanized Escherichia coli β-galactosidase/IT injection and intravenous (IV) infusion

Jonker, Derek; The Ottawa Hospital Cancer Center; Ottawa, Ontario, Canada; and Nieva, Jorge J.; Billings Clinical Research Center; Billings, Montana; *A Phase 1/2a Dose-escalation Study of JX-594 (Thymidine Kinase-Deactivated Vaccinia Virus plus GM-CSF) Administered by Multiple Intravenous (IV) Infusions Followed by Intratumoral (IT) Boosts Alone and in Combination with Irinotecan in Patients with Metastatic, Refractory Colorectal Carcinoma*. Sponsor: Jennerex, Inc.

NIH/OBA Receipt Date: 07-08-11. Not Selected for RAC Public Review: 07-29-11

1107-1114 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo/RNA knockdown/ TGFβ1 and TGFβ2 /Intradermal Injections

Barve, Minal; Tex Oncology, P.A.; Dallas, Texas; *Phase I Trial of FANG™ Autologous Tumor Cell Vaccine Combined in Advanced Melanoma*. Sponsor Gradalis, Inc.

NIH/OBA Receipt Date: 07-11-11. Not Selected for RAC Public Review: 08-01-11

1107-1115 (Open) Gene Therapy/Phase II/Cancer/Pancreatic/In Vivo/Plasmid/Diphtheria Toxin A Chain (DT-A)/H19 Promoter/Intratumoral Administration

Loren, David E.; Thomas Jefferson University; Philadelphia, Pennsylvania; Stavropoulos; Stavros N.; Winthrop University Hospital Clinical Trial Center; Mineola, New York; and Tafur, Isaac; Joe Arrington Cancer Research and Treatment Center; Lubbock, Texas; *A Multi-Center, Open-Label, Randomized, Phase 2b Study to Evaluate the Efficacy and Safety of BC-819 and Gemcitabine versus Gemcitabine Alone in Treatment-Naïve Patients with Locally Advanced Pancreatic Adenocarcinoma*. Sponsor: BioCancell Therapeutics Ltd.

NIH/OBA Receipt Date: 07-14-11. Not Selected for RAC Public Review: 08-04-11

1107-1116 (Open) Gene Therapy/Phase I/II/Cancer/Non-Small Cell Lung Cancer (NSCLC)/In Vivo/Tumor Suppressor Gene/Cationic Liposome Complex/DOTAP:Cholesterol/*Fus1* cDNA/Intravenous Injection

Lu, Charles; The University of Texas MD Anderson Cancer Center; Houston, Texas; *Phase I/II Clinical Trial combining FUS1-nanoparticles and Erlotinib in Stage IV Lung Cancer*.

NIH/OBA Receipt Date: 07-18-11. Not Selected for RAC Public Review: 08-08-11

1107-1117 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I-II/Cancer/Pro-drug/Cytosine Deaminase/*Bifidobacterium longum*/Intravenous Infusion

Nemunaitis, John J.; Mary Crowley Cancer Research Centers; Dallas, Texas; *A Phase I/II Safety, Pharmacokinetic, and Pharmacodynamic Study of APS001F with Flucytosine and Maltose for the Treatment of Advanced and/or Metastatic Solid Tumors*. Sponsor: Anaeropharma Science.

NIH/OBA Receipt Date: 07-19-11. Publicly Reviewed at the September 2011 RAC meeting

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1107-1118 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vivo/ Autologous T lymphocytes /T Cell Receptor Specific for Cancer Testes Antigens/Intravenous Injections

Odunsi, Kunle; Roswell Park Cancer Institute; Buffalo, New York; Coukos, George; University of Pennsylvania School of Medicine; Philadelphia, Pennsylvania; and Edwards, Robert P.; University of Pittsburgh; Pittsburgh, Pennsylvania; *Phase I, open label, dual cohort, triple center clinical trial evaluating the safety and efficacy of autologous T cell expressing enhanced TCRs specific for Mage-A3/6/B18 or NY-ESO-1/LAGE in patients with recurrent or treatment refractory ovarian cancer*. Sponsor: Adaptimmune Ltd.

NIH/OBA Receipt Date: 07-19-11. Not Selected for RAC Public Review: 08-09-11

1107-1119 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Solid Tumors/In Vivo/*Saccharomyces cerevisiae*/Brachyury Oncoprotein cDNA/Subcutaneous injections

Gulley, James L.; National Institutes of Health; Bethesda, Maryland; *An Open Label Phase I Study to Evaluate the Safety and Tolerability of GI-6301 a Vaccine Consisting of Whole, Heat-Killed Recombinant Saccharomyces cerevisiae (Yeast) Genetically Modified to Express Brachyury Protein in Adults with Metastatic Carcinoma.*

NIH/OBA Receipt Date: 07-20-11. Publicly Reviewed at the September 2011 RAC meeting

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1107-1120 (Open; RAC reviewed with recommendations) Gene Therapy Phase I/Cancer/Glioblastoma Multiforme/Pro-drug/Replication Competent Retrovirus/In Vivo/Yeast 1 Cytosine Deaminase cDNA/5-Flurocytosine /Injections into the Wall of the Resection Cavity

Elder, James B.; The Ohio State University Medical Center; Columbus, Ohio; Foltz, Greg; Swedish Medical Center – Cherry Hill; Seattle, Washington; Landolfi, Joseph; JFK Medical Center; Edison, New Jersey; Mikkelsen, Tom; Henry Ford Health System; Detroit, Michigan; and Cloughesy, Timothy F.; University of California, Los Angeles; Los Angeles; *A Phase 1 Ascending Dose Trial of the Safety and Tolerability of Toca 511, a Retroviral Replicating Vector, Administered to Subjects at the Time of Resection for Recurrent High Grade Glioma and Followed by Treatment with Toca FC, Extended-Release 5-FC*. Sponsor: Tocagen Inc.

NIH/OBA Receipt Date: 07-14-11. Publicly Reviewed at the September 2011 RAC meeting

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1108-1121 (Open) Gene Therapy/Phase III/Peripheral Artery Disease/Other/Plasmid DNA/Hepatocyte Growth Factor cDNA/intramuscular Injection

Powell, Richard; Dartmouth-Hitchcock Medical Center; Lebanon, New Hampshire; *A Phase III Double-Blind, Randomized, Placebo-controlled Study to Evaluate the Safety and Efficacy of AMG0001 (HGF Plasmid) in Subjects with Critical Limb Ischemia (AG-CLI-0206)*. Sponsor: AnGes, Inc.

NIH/OBA Receipt Date: 08-08-11. Not Selected for RAC Public Review: 09-02-11

1108-1122 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vivo/ Epitope DNA Based Vaccine/Insulin Like Growth Factor Binding Protein 2 (IGFBP-2)/Intradermal Injections

Dsis, Mary L.; University of Washington; Seattle, Washington; *A Phase I Trial of the Safety and Immunogenicity of a DNA Plasmid Based Vaccine Encoding the Amino Acids 1-163 of IGFBP-2 in Patients with Advanced Ovarian Cancer*.

NIH/OBA Receipt Date: 08-16-11. Publicly Reviewed at the December 2011 RAC meeting

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1109-1123 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/GD-2-Specific scFvFc-Zeta T Cell Receptor/Intravenous Injections

Fulbright, Joy M.; Children's Mercy Cancer Center; Kansas City, Missouri; MARVSmALo: *Phase I Study of Vaccine Enriched, Autologous, Activated T-Cells Redirected to the Tumor Marker GD2 in Patients with Relapsed/Refractory Melanoma*.

NIH/OBA Receipt Date: 09-25-11. Not Selected for RAC Public Review: 10-18-11

1109-1124 (Open) Phase I/Non-therapeutic/Human Immunodeficiency Virus/In Vivo/Plasmid/Adenovirus Serotype 35/HIV-1 Gag/Pol and Nef/Tat/Vif/Env cDNAs/Interleukine-12 cDNA/Intramuscular Injections

Karita, Etienne; Project San Francisco; Kigali, Rwanda; Anzala, Omu; College of Health Sciences; Nairobi, Kenya; and Mpendo, Juliet; UVRI-IAVI HIV Vaccine Program; Entebbe, Uganda; *A Phase 1 Double-Blind, Randomized, Placebo-Controlled Trial to Evaluate the Safety and Immunogenicity of a Multiantigen HIV (HIV-MAG) plasmid DNA (pDNA) Vaccine Co-administered with Recombinant Human IL-12 pDNA (GENEVAX® IL-12) Followed or Preceded by Recombinant Ad35-GRIN/ENV HIV Vaccine in HIV-Uninfected, Healthy Volunteers*. Sponsor: International AIDS Vaccine Initiative.

NIH/OBA Receipt Date: 09-30-11. Not Selected for RAC Public Review: 10-24-11

1110-1125 (Open) Gene Therapy/Phase I/Ovarian Cancer/Apoptosis Induction/In Vivo/Adenovirus/Serotype 5/Fas-TNF Receptor Chimera Transgene/Intravenous Injection

Penson, Richard T.; Massachusetts General Hospital; Boston, Massachusetts; *A Phase I Trial of Multiple Dose VB-111 and Weekly Paclitaxel for the Treatment of Recurrent Platinum-Resistant Müllerian Cancer*. Sponsor: Vascular Biogenics Ltd

NIH/OBA Receipt Date: 10-11-11. Not Selected for RAC Public Review: 11-01-11

1110-1126 (Open) Gene Therapy/Phase I/Cancer/Breast and Her2+/Immunotherapy/In Vivo/Venezuelan Equine Encephalitis (VEE) Virus Replicon/Her2 cDNA/Subcutaneous Injection

Lyerly, H. Kim; Duke University Medical Center; Durham, North Carolina; *A Phase I Study to Evaluate the Antitumor Activity and Safety of Duke-002-vrp (huher2-ecd+tm) (avx901), an Alphaviral Vector Encoding the Her2 Extracellular Domain and Transmembrane Region, in Patients with Locally Advanced or Metastatic Human Epidermal Growth Factor Receptor 2-Positive (Her2+) Cancers Including Breast Cancer*.

NIH/OBA Receipt Date: 10-11-11. Not Selected for RAC Public Review: 11-01-11

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1110-1127 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/Triple Negative Breast Cancer/Immunotherapy/In Vitro/Autologous T Lymphocytes/mRNA Electroporation/c-Met-scFv with Signaling Domains Comprised of TCRζ and 4-1BB cDNA/Intratumoral followed by Intravenous Injections

Tchou, Julia C.; University of Pennsylvania; Philadelphia, Pennsylvania; *Pilot Clinical Trial of Autologous Met Redirected T cells Administered Intratumorally and Intravenously in Patients with Operable Triple Negative Breast Cancer*.

NIH/OBA Receipt Date: 10-11-11. Publicly Reviewed at the December 2011 RAC meeting

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1110-1128 (Open) Gene Therapy/Phase I/Cancer/Head and Neck/Immunotherapy/In Vivo/Plasmid DNA/HPV 16 E7 cDNA/Intramuscular Injection

Pai, Sara I.; Johns Hopkins School of Medicine; Baltimore, Maryland; *A Phase I Clinical Trial Assessing the Safety and Feasibility of Administration of pNGVL4a-CRT/E7(detox) DNA Vaccine using the Intramuscular TriGridTM Delivery System in Combination with Cyclophosphamide in HPV-16 Associated Head and Neck Cancer Patients*.

NIH/OBA Receipt Date: 10-12-11. Not Selected for RAC Public Review: 11-02-11

1110-1129 (Open) Gene Therapy/Phase I/Monogenic Disease/Hemophilia B/In Vivo/Self Complementary Adeno-Associated Virus (2/8)/Factor IX Gene/Intravenous Administration

Monahan, Paul; University of North Carolina at Chapel Hill; Chapel Hill, North Carolina; *A Phase 1 Open-Label, Ascending-Dose trial of AskBio009 in Patients with Severe Hemophilia B*. Sponsor: Asklepios BioPharmaceutical Inc.

NIH/OBA Receipt Date: 10-12-11. Not Selected for RAC Public Review: 11-08-11

1110-1130 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I-II/Infectious Diseases/HIV/Cell-Delivered/Lentiviral Vector/LVsh5/C46/Hematopoietic Cells/Subcutaneous Injections

Mitsuyasu, Ronald, University of California, Los Angeles; Los Angeles, California; *An Adaptive Phase I-II Study of the Safety of CD4+ T cells and CD34+ Hematopoietic Stem/Progenitor Cells Transduced with CAL-1, a Dual Anti-HIV Gene Transfer Construct, in Busulfan Conditioned HIV-Infected Adults Previously Exposed to ART*. Sponsor: Calimmune Inc.

NIH/OBA Receipt Date: 10-18-11. Publicly Reviewed at the December 2011 RAC meeting

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1110-1131 (Open) Gene Therapy/Phase I/Other/Wound Healing/In Vivo/Plasmid DNA/Stromal Cell-Derived Factor-1/Dermal Injection

Patel, Amit; University of Utah; Salt Lake City, Utah; and Espinal, Eric Alexei; Summa Health System; Akron, Ohio; *A Phase I/II Randomized, Double-Blind, Placebo Controlled Dose Escalation Study to Evaluate the Safety and Efficacy of JVS-100 Administered by Needle-free Dermal Injection to Cohorts of Adults Receiving Surgical Incisions*. Sponsor: SironRX Therapeutics, Inc.

NIH/OBA Receipt Date: 10-17-11. Not Selected for RAC Public Review: 11-07-11

1110-1132 (Open) Gene Therapy/Phase II-III/Cancer/Non-Small Cell Lung Cancer (NSCLC)/Immunotherapy/In Vivo/Vaccinia Virus/MUC-1/Interleukin-2/Subcutaneous Injection

Nemunaitis, John J.; Mary Crowley Cancer Research Centers; Dallas, Texas; *A Phase IIB/III Randomized, Double-Blind, Placebo-Controlled Study Comparing First-Line Therapy with or without TG4010 Immunotherapy Product in Patients with Stage IV Non-Small Cell Lung Cancer (NSCLC)*. Sponsor: Transgene S.A.

NIH/OBA Receipt Date: 10-19-11. Not Selected for RAC Public Review: 11-08-11

1110-1133 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Cancer/NY-ESO-1 or LAGE-1 Expressing Tumors/Immunotherapy/In Vivo/Canarypox Virus/NY-ESO-1, TRICOM cDNAs/Subcutaneous Injections

Odunsi, Kunle; Roswell Park Cancer Institute; Buffalo, New York; *A Phase I Clinical Trial of mTOR Inhibition with Sirolimus for Enhancing ALVAC(2)-NY-ESO-1(M)/TRICOM Vaccine Induced Anti-Tumor Immunity In Ovarian, Fallopian Tube and Primary Peritoneal Cancer*.

NIH/OBA Receipt Date: 10-19-11. Publicly Reviewed at the December 2011 RAC meeting

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1110-1134 (Open) Gene Therapy/Phase II/Cancer/Colorectal Carcinoma/Immunotherapy/In Vivo/RNA knockdown/ TGFβ1 and TGFβ2/Intradermal Injection

Barve, Minal; Mary Crowley Medical Research Center; Dallas, Texas; *Randomized Phase II Trial of Post-operative Adjuvant Chemotherapy ± FANG™ Autologous Tumor Cell Vaccine in Colorectal Carcinoma with Liver Metastases (CL-PTL 107)*.

NIH/OBA Receipt Date: 10-20-11. Not Selected for RAC Public Review: 11-08-11

1110-1135 (Open) Gene Therapy/Phase II/Cancer/Melanoma/Immunotherapy/In Vivo/Plasmid DNA/IL-12/ Intratumoral Injections

Daud, Adil I.; University of California, San Francisco; San Francisco, California; *Phase II Trial of Intratumoral pIL-12 Electroporation in Advanced Stage Cutaneous and in Transit Malignant Melanoma*.

NIH/OBA Receipt Date: 10-19-11. Not Selected for RAC Public Review: 11-08-11

1110-1136 (Open) Gene Therapy/Phase II/Cancer/Chronic Lymphocytic Leukemia, Small Lymphocytic Lymphoma/Immunotherapy/In Vivo/Adenovirus/Serotype 5/CD154/Intranodal Injection

Castro, Januario E.; University of California San Diego; La Jolla, California; *A Phase Ib/IIa Study of Administration of Lenalidomide in Combination with Repeat Intranodal Direct Injections of Adenovirus-Isf35 (Ad-Isf35) in Subjects with Chronic Lymphocytic Leukemia/Small Lymphocytic Lymphoma*.

NIH/OBA Receipt Date: 10-28-11. Not Selected for RAC Public Review: 11-28-11

1111-1137 (Open) Gene Therapy/Single Subject/Cancer/Pancreatic/In Vivo/Plasmid/DOTAP and Synthetic Cholesterol/RNA Knockdown/PDX-1/Intravenous Infusion

Barve, Minal; Mary Crowley Medical Research Center; Dallas, Texas; *Single Patient Compassionate Study of Intravenous Delivery of BIV-Liposome Enveloped bi-shRNAPDX-1 for Recurrent Pancreatic Adenocarcinoma*. Sponsor: Gradalis, Inc.

NIH/OBA Receipt Date: 11-15-11. Not Selected for RAC Public Review: 11-28-11

1112-1138 (Open) Gene Therapy/Phase I/Cancer/Advanced or Metastatic/In Vivo/Plasmid/DOTAP and Synthetic Cholesterol/RNA Knockdown/Stathmin 1/Intratumoral Injection

Barve, Minal; Mary Crowley Medical Research Center; Dallas, Texas; *Phase I Trial of Intratumoral Bi-functional shRNA Stathmin 1-knockdown Lipoplex in Patients with Advanced and/or Metastatic Cancer*. Sponsor: Gradalis, Inc.

NIH/OBA Receipt Date: 12-12-11. Not Selected for RAC Public Review: 01-04-12

1112-1139 (Open) Gene Therapy/Phase I-II/Cancer/Mesothelin Expressing Metastatic Cancer/Immunotherapy/In Vitro/Autologous Peripheral Blood Lymphocytes/Retrovirus/α-mesothelin-scFv with signaling domains comprised of TCRζ and CD28 cDNA/Intravenous Infusion

Rosenberg, Steven; National Institutes of Health; Bethesda, Maryland; *Phase I/II Study of Metastatic Cancer Using Lymphodepleting Conditioning Followed by Infusion of Anti-mesothelin Gene Engineered Lymphocytes*.

NIH/OBA Receipt Date: 12-29-11. Not Selected for RAC Public Review: 01-23-12

1201-1140 (Open) Gene Therapy/Phase II/Other Diseases-Disorders/Heart Failure/In Vivo/Plasmid/Stromal Cell-Derived Factor (SDF-1) cDNA/Myocardial Administration Using Biocardia Helix Needle Injection Catheter

Miller, Leslie W.; University of South Florida; Tampa, Florida; Sherman, Warren; Columbia University Medical Center; New York, New York; Silver, Kevin H.; Northeast Ohio Cardiovascular Specialists, Inc.; Akron, Ohio; Shin, Jooyoung Julia; Montefiore Medical Center; Bronx, New York; Patel, Amit; The University of Utah; Salt Lake City, Utah; and Mendelsohn, Farrell O.; Cardiology, P.C.; Birmingham, Alabama; *A Phase II Randomized, Double-Blind, Placebo Controlled Study to Evaluate the Safety and Efficacy of a Single Dose of JVS-100 Administered by Endomyocardial Injection to Cohorts of Adults with Ischemic Heart Failure*. Sponsor: Juventis Therapeutics, Inc.

NIH/OBA Receipt Date: 01-09-12. Not Selected for RAC Public Review: 01-31-12

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1201-1141 (Open) Gene Therapy/Phase I-II/Monogenic Disease/Hemophilia B/In Vivo/ Adeno-Associated Virus (2/8)/Factor IX Gene/Intravenous Administration

Ragni, Margaret V.; Hemophilia Center of Western Pennsylvania; Pittsburgh, Pennsylvania; and High, Katherine A; The Children's Hospital of Philadelphia; Philadelphia, Pennsylvania; *A Phase 1 Safety Study in Subjects with Severe Hemophilia B (Factor IX Deficiency) Using a Single-Stranded, Adeno-Associated Pseudotype 8 Viral Vector to Deliver the Gene for Human Factor IX [AAV8-hFIX19-101]*.

NIH/OBA Receipt Date: 01-09-12. Not Selected for RAC Public Review: 01-31-12

1201-1142 (Open) Gene Therapy/Phase I/Cancer/B-cell chronic lymphocytic leukemia/Immunotherapy/In Vitro/Autologous CD4+ and CD8+ T lymphocytes /Sleeping Beauty (SB) Transposon/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Cooper, Laurence J. N.; MD Anderson Cancer Center; Houston, Texas; *Adoptive Immunotherapy for B-cell chronic lymphocytic leukemia using Sleeping Beauty Transposition to Express a CD19-specific Chimeric Antigen Receptor in Autologous Ex Vivo Expanded T cells*.

NIH/OBA Receipt Date: 01-10-12. Not Selected for RAC Public Review: 02-01-12

1201-1143 (Open; RAC reviewed with recommendations) Gene Therapy/Phase II/Other Diseases Disorders/Anemia of End Stage Renal Disease (ESRD)/In Vitro/Adenovirus/Erythropoietin (EPO) cDNA/Subcutaneous Implantation of Transduced Micro-organs Harvested from the Dermis

Block, Geoffrey; Denver Nephrology Research; Denver, Colorado; *A Phase 2, Randomized, Active Control, Open-Label, Multi-Center Study to Evaluate the Safety and Efficacy of EPODURE for Sustained Treatment of Anemia in Hemodialysis Patients*. Sponsor: Medgenics Medical Israel

NIH/OBA Receipt Date: 01-10-12. Publicly Reviewed at the March 2012 RAC meeting

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1201-1144 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/Familial Hypercholesterolemia/In Vivo/Adeno-Associated Virus (2/8)/Low Density Lipoprotein Receptor (LDLR) cDNA/Intravenous Administration

Moriarty, Patrick; The University of Kansas Medical Center; Kansas City, Kansas; *AAV8-mediated Low Density Lipoprotein Receptor Gene Replacement in Subjects with Homozygous Familial Hypercholesterolemia*.

NIH/OBA Receipt Date: 01-10-12. Publicly Reviewed at the March 2012 RAC meeting

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1201-1145 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Other/Coronary Artery Disease/In Vivo/Ischemic Myocardium/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Cardiac Administration

Crystal, Ronald G.; Weill Cornell Medical College; New York, New York; and Rosengart, Todd; Stony Brook Medical Center; Stony Brook, New York; *Phase I Study of Direct Administration of AdVEGF-All6A+, a Replication Deficient Adenovirus Vector Expressing a cDNA/Genomic Hybrid of Human Vascular Endothelial Growth Factor to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease via Minimally Invasive Surgery*.

NIH/OBA Receipt Date: 01-11-12. Publicly Reviewed at the March 2012 RAC meeting

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1201-1146 (Open) Gene Therapy/Phase I/Cancer/Melanoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/MART-1, Tyrosinase, MAGE-A6 cDNAs/Intradermal Injections

Kirkwood, John M.; University of Pittsburgh Medical Center; Pittsburgh, Pennsylvania; *A Phase II Trial Testing Multiple Antigen-Engineered DC Followed by IFNα2b Boost for Immunization of HLA-Unrestricted Melanoma Patients*.

NIH/OBA Receipt Date: 01-10-12. Not Selected for RAC Public Review: 02-01-12

1201-1147 (Open) Gene Therapy/Phase I/Cancer/B Cell Malignancies/Immunotherapy/In Vitro/Autologous CD3+ T Lymphocytes/Retrovirus/CD19 antigen specific-Zeta T Cell Receptor/Intravenous Infusion

Wayne, Alan S.; National Institutes of Health; Bethesda, Maryland; *Phase I Study of T Cells Expressing an Anti-CD19 Chimeric Receptor in Children and Young Adults with B Cell Malignancies*.

NIH/OBA Receipt Date: 01-11-12. Not Selected for RAC Public Review: 02-01-12

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1201-1148 (Open) Gene Therapy/Phase I/Peripheral Artery Disease/In Vivo/Plasmid DNA/Hepatocyte Growth Factor cDNA/Intramuscular Injection

Kessler, John A.; Northwestern University; Chicago, Illinois; and Vinik, Aaron; Eastern Virginia Medical School; Norfolk, Virginia; *A Phase II, Double-blind, Randomized, Placebo-controlled, Multicenter Study to Assess the Safety and Efficacy of VM202 in Subjects with Painful Diabetic Peripheral Neuropathy*. Sponsor: ViroMed Co. Ltd (dba VM BioPharma)

NIH/OBA Receipt Date: 01-18-12. Not Selected for RAC Public Review: 02-09-12

1201-1149 (Open) Gene Therapy/Phase I/Infectious Disease/Human Immunodeficiency Virus/In Vitro/Autologous Lymphocytes/Adenovirus/Serotype 5/cDNA of Engineered Zinc Finger Nucleases (ZFNs) Targeting the Human CCR5 Locus (SB-728)/Intravenous

Mitsuyasu, Ronald; University California Los Angeles; Los Angeles, California; *A Phase I, Open-Label Study to Assess the Effect of Escalating Doses of Cyclophosphamide on the Engraftment of SB-728-T in Aviremic HIV-Infected Subjects on HAART*. Sponsor: Sangamo Biosciences, Inc.

NIH/OBA Receipt Date: 01-19-12. Not Selected for RAC Public Review: 02-09-12

1202-1150 (Open) Gene Therapy/Phase I-II/Cancer/CD19+ Malignancies/Immunotherapy/In Vitro/Allogeneic CD8+ T Lymphocytes/Lentivirus/CD19 Antigen Specific Chimeric Antigen Receptor (CAR)/Intravenous Injections

Turtle, Cameron; Fred Hutchinson Cancer Research Center; Seattle, Washington; *A Phase 1/11 Study of Cellular Immunotherapy with Donor Central Memory -Derived Virus-Specific CD8+ T-Cells Engineered to Target CD 19 for CD 19+ Malignancies after Allogeneic Hematopoietic Stem Cell Transplant*.

NIH/OBA Receipt Date: 02-08-12. Not Selected for RAC Public Review: 03-01-12

1202-1151 (Open) Gene Therapy/Phase I-II/Cancer/B-cell Chronic Lymphocytic Leukemia (B-CLL)/Immunotherapy/In Vitro/Autologous Leukemia Cells/Adenovirus/Serotype 5/Interleukin-2 cDNA/CD40 Ligand cDNA/Subcutaneous Injection

Mims, Martha; Brenner, Malcolm; and Foster, Aaron; Baylor College of Medicine; Houston, Texas; *Treatment of B-CLL with Autologous IL2 and CD40 Ligand-Expressing Tumor Cells + Lenalidomide (TAIL)*.

NIH/OBA Receipt Date: 02-13-12. Not Selected for RAC Public Review: 03-06-12

1202-1152 (Open) Gene Therapy Phase I/Cancer/Prostate/Immunotherapy/In Vivo/Plasmid DNA/Prostatic Acid Phosphatase (PAP) cDNA/Intradermal Injection

McNeel, Douglas; University of Wisconsin; Madison, Wisconsin; *Pilot Trial of Sipuleucel-T, with or without pTVG-HP DNA Booster Vaccine, in Patients with Castrate-Resistant, Metastatic Prostate Cancer*.

NIH/OBA Receipt Date: 02-21-12. Not Selected for RAC Public Review: 03-13-12

1202-1153 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Infectious Disease/HIV/Replication inhibition/In Vitro/Lentiviral Vector/CCR5shRNA/RNA Decoy for TAR/TRIM5alpha/CD34+ Cells/Intravenous

Abedi, Mehrdad; University of California Davis; Sacramento, California; *Stem Cell Gene Therapy for HIV in AIDS Lymphoma Patients*.

NIH/OBA Receipt Date: 02-21-12. Publicly Reviewed at the June 2012 RAC meeting

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1203-1154 (Open) Gene Therapy/Phase II/Cancer/ Hepatocellular Carcinoma/Immunotherapy/In Vivo/Vaccinia Virus/GM-CSF/Humanized *Escherichia coli* β-galactosidase / IT Injection and Intravenous (IV) Infusion

Heo, Jeong; Pusan National University Hospital; South Korea; *A Single-Arm, Open-Label, Phase 2 Study of JX-594 (Thymidine Kinase-Deactivated Vaccinia Virus plus GM-CSF) Administered by Weekly Intravenous (IV) Infusions in Sorafenib-Naïve Patients with Advanced Hepatocellular Carcinoma (HCC)*.

NIH/OBA Receipt Date: 03-02-12. Not Selected for RAC Public Review: 03-23-12

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*The term “RAC Recommends Approval” for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1203-1155 (Open) Gene Therapy/Phase I/Cancer/HER2+ Cancers/Immunotherapy/In Vivo/ Adenovirus Vector/Human Epidermal Growth Factor Receptor-2/Subcutaneous Injection

Morse, Michael A.; Duke University; Durham, North Carolina; *A Phase I Study To Evaluate The Antitumor Activity And Safety Of Duke-001-Ad(HuHER2-ECD+TM), An Adenoviral Vector Encoding The HER2 Extracellular Domain And Transmembrane Region, In Patients With Locally Advanced Or Metastatic Human Epidermal Growth Factor Receptor 2-Positive (HER2+) Cancers Including Breast Cancer*.

NIH/OBA Receipt Date: 03-12-12. Not Selected for RAC Public Review: 04-02-12

1203-1156 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Vector-Directed Cell Lysis/In Vivo/Adenovirus/Type 5/Replication-Competent Virus/RGD Motif/Limb Infusion

Fields, Ryan C.; Washington University School of Medicine; St. Louis, Missouri; *A Phase I/II Study of Isolated Limb Infusion and Targeted Gene Therapy for Advanced, Unresectable Extremity Melanoma*.

NIH/OBA Receipt Date: 03-21-12. Not Selected for RAC Public Review: 04-11-12

1203-1157 (Open) Gene Therapy/ Phase II/Cancer/NY-ESO-1 Expressing Cancer/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/T Cell Receptor alpha and beta cDNAs Chains/Intravenous Infusion

Singh, Arun; University of California Los Angeles; Los Angeles, California; *Adoptive Transfer of NY-ESO-1 TCR Engineered Peripheral Blood Mononuclear Cells (PBMC) after a Nonmyeloablative Conditioning Regimen, with Administration of NY-ESO-$1_{157-165}$ Pulsed Dendritic Cells and Interleukin-2, in Patients with Advanced Malignancies*.

NIH/OBA Receipt Date: 03-26-12. Not Selected for RAC Public Review: 04-16-12

1204-1158 (Open) Gene Therapy/Phase I-II/Cancer/Hematological Malignancies/Elimination of Graft versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/Inducible Caspase 9 Suicide Gene/AP1903/Intravenous

Lazarus, Hillard; University Hospitals of Cleveland; Cleveland, Ohio; *A Phase1/2 Study Evaluating Escalating Doses of CaspaCIDe™ T Cells (T Cells Genetically Modified with the iCasp9 Suicide Gene) after Partially Mismatched, Related, T Cell-Depleted HSCT*. Sponsor: Bellicum Pharmaceuticals

NIH/OBA Receipt Date: 04-10-12. Not Selected for RAC Public Review: 05-01-12

1204-1159 (Open) Gene Therapy/ Phase I-II/Cancer/Hematological Malignancies/Elimination of Graft versus Host Disease/In Vitro/Allogeneic T Cells/Retrovirus/Inducible Caspase 9 Suicide Gene/AP1903/Intravenous

Champlin, Richard E.; The University of Texas MD Anderson Cancer Center; Houston, Texas; *A Phase 1/2 Trial Evaluating Treatment of Emergent Graft versus Host Disease (GvHD) with AP1903 after Planned Donor Infusions (DLIs) of T- cells Genetically Modified with the iCasp9 Suicide Gene in Subjects with Hematologic Malignancies*. Sponsor: Bellicum Pharmaceuticals

NIH/OBA Receipt Date: 04-11-12. Not Selected for RAC Public Review: 05-01-12

1204-1160 (Open) Gene Therapy/Phase I/Cancer/Mesothelioma/Immunotherapy/In Vivo/Listeria monocytogenes/Mesothelin cDNA/Intravenous Injection

Hassan, Raffit; National Institutes of Health; Bethesda, Maryland; and Antonia, Scott J.; University of South Florida; Tampa, Florida; *A Phase IB Study to Evaluate the Safety and Induction of Immune Response of CRS-207 in Combination with Pemetrexed and Cisplatin as Front-line Therapy in Adults with Malignant Pleural Mesothelioma*. Sponsor: Aduro BioTech, Inc.

NIH/OBA Receipt Date: 04-12-12. Not Selected for RAC Public Review: 05-03-12

1204-1161 (Open) Gene Therapy/Phase I/Cancer/CD19+ Acute Lymphoblastic Leukemia/Immunotherapy/In Vitro/Lentivirus/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Infusions

Gardner, Rebecca; Seattle Children's Hospital; Seattle, Washington; *A Phase 1 Feasibility and Safety Study of Cellular Immunotherapy for Relapsed Pediatric CD19+ Acute Lymphoblastic Leukemia Using Autologous T-cells Lentivirally Transduced to Express a CD19-Specific Chimeric Antigen Receptor*.

NIH/OBA Receipt Date: 04-16-12. Not Selected for RAC Public Review: 05-07-12

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*The term "RAC Recommends Approval" for any submission indicates that the RAC made a recommendation to the NIH Director for approval of that submission.

1204-1162 (Open) Gene Therapy/Phase II/Other Diseases-Disorder/Oral Mucositis/*Lactococcus lactis* /Human Trefoil Factor 1 (hTFF1) cDNA/Oral Topical Application

Murphy, Barbara; Vanderbilt University; Nashville, Tennessee; *A Phase 2b, Multi-center, Randomized, Double-blind, Placebo-controlled Study to Assess the Safety and Efficacy of Two Dosing Schedules of Topically-applied AG013 for the Attenuation of Oral Mucositis in Subjects with Cancers of the Head and Neck Receiving Concomitant Chemoradiation Therapy*. Sponsor: ActoGeniX

NIH/OBA Receipt Date: 04-20-12. Not Selected for RAC Public Review: 05-11-12

1204-1163 (Open) Gene Therapy/Phase II/Cancer/Bladder/Immunotherapy/In Vivo/Adenovirus Serotype 5/Interferon Alpha-2b cDNA/Intravesical Administration

Dinney, Colin P. N.; The University of Texas MD Anderson Cancer Center; Houston, Texas*; A Phase II, Randomized, Open Label, Parallel Arm Study to Evaluate the Safety and Efficacy of rAd-IFN/Syn3 Following Intravesical Administration in Subjects with High Grade, BCG Refractory, Relapsed or Resistant Non-Muscle Invasive Bladder Cancer (NMIBC)*. Sponsor: FKD Therapies

NIH/OBA Receipt Date: 04-23-12. Not Selected for RAC Public Review: 05-14-12

1204-1164 (Open; RAC reviewed with recommendations) Gene Therapy/Phase I/Monogenic Disease/ β-Thalassemia/In Vitro/Autologous CD34+ Cells/Lentivirus/Human β-Globin Gene/Intravenous

Kohn, Donald; University of California Los Angeles; Los Angeles, California; *A Phase 1/2, Open Label Study Evaluating the Safety and Efficacy of Gene Therapy in Subjects with β-Thalassemia Major by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with a Lentiviral $\beta^{A\text{-}T87Q}$-Globin Vector*. Sponsor: bluebird bio

NIH/OBA Receipt Date: 04-24-12. Publicly Reviewed at the June 2012 RAC meeting

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1204-1165 (Open) Gene Therapy/Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Lethally Irradiated/Adeno-Associated Virus/Cytokine/Granulocyte-Macrophage Colony Stimulating Factor/Intradermal Injection

Antonarakis, Emmanuel S.; Johns Hopkins University; *A Neoadjuvant Immunologic Study of Androgen Deprivation Therapy Combined with a GM-CSF–Secreting Allogeneic Prostate Cancer Vaccine and Low-dose Cyclophosphamide in Men with High-risk Localized Prostate Cancer Undergoing Radical Prostatectomy*.

NIH/OBA Receipt Date: 04-24-12. Not Selected for RAC Public Review: 05-15-12

1204-1166 (Open) Gene Therapy/Phase I-II/Monogenic Disease/Achromatopsia/In Vitro/Plasmid/Encapsulated Cell-based Drug Delivery Device/Human Ciliary Neurotrophic Growth Factor (CNTF) cDNA/Intraocular Implantation (via Sclerotomy)

Sieving, Paul A.; National Institutes of Health; Bethesda, Maryland; *A Phase I/II Study of the NT-501 Intraocular Implant Releasing Ciliary Neurotrophic Factor (CNTF) in Participants with CNGB3 Achromatopsia*. Sponsor: Neurotech USA, Inc.

NIH/OBA Receipt Date: 04-26-12. Not Selected for RAC Public Review: 05-15-12

1205-1167 (Open) Gene Therapy/Phase II-III/Other Diseases-disorders/Heart Failure /In Vivo/Adeno-associated Virus/Serotype 1/ Sarcoplasmic Reticulum Calcium ATPase 2a (SERCA2a) cDNA/Intracoronary Administration

Bernard, Denise B.; University of California, San Diego; San Diego, California; Klapholz, Marc; University of Medicine and Dentistry of New Jersey; Newark, New Jersey; Adamson, Philip B.; Oklahoma Cardiovascular Research Group; Oklahoma City, Oklahoma; Koren, Michael J.; Jacksonville Center for Clinical Research, Memorial Hospital; Jacksonville, Florida; and Heilman, K. John, III; Black Hills Cardiovascular Research; Rapid City, South Dakota; *A Phase 2/3, Double-Blind, Placebo-Controlled, Multinational, Multicenter, Randomized Study Evaluating the Safety and Efficacy of Intracoronary Administration of MYDICAR® (AAV1/SERCA2a) in Subjects with Heart Failure*. Sponsor: Celladon Corporation

NIH/OBA Receipt Date: 05-14-12. Not Selected for RAC Public Review: 06-06-12

1205-1168 (Open) Gene Therapy/Phase I/Cancer/Ovarian/Immunotherapy/In Vivo/Lipopolymer Delivery/Human IL-12/Intraperitoneal Injection

Thaker, Premal; Washington University School of Medicine; St. Louis, Missouri; Mannel, Robert; University of Oklahoma Health Sciences Center; Oklahoma City, Oklahoma; and Mutch, David G.; Barnes-Jewish Hospital; St. Louis, Missouri; *A Phase I Study of Intraperitoneal EGEN-001 (IL-12 Plasmid Formulated with PEG-PEI-Cholesterol Lipopolymer) Administered in Combination with Pegylated Liposomal Doxorubicin (PLD), Doxil and Lipodox (NSC# 712227) in Patients with Recurrent or Persistent Epithelial Ovarian, Fallopian Tube or Primary Peritoneal Cancer*. Sponsor: EGEN, Inc.

NIH/OBA Receipt Date: 05-14-12. Not Selected for RAC Public Review: 06-06-12

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1206-1169 (Under review) Gene Therapy/Phase I-II/Cancer/Acute Myeloid Leukemia (AML), Myelodysplastic Syndrome (MDS), and Chronic Myeloid Leukemia (CML)/Immunotherapy/In Vitro/Lentivirus/Alpha and Beta Chains of Wilms' Tumor Antigen 1 (WT1) T Cell Receptor/Intravenous Infusion

Bar, Merav; University of Washington/Fred Hutchinson Cancer Research Center; Seattle, Washington; *Phase I/II Study of Adoptive Immunotherapy after Allogeneic HCT with Virus Specific CD8+ T Cells that have been Transduced to Express a WT1-specific T Cell Receptor for Patients with High Risk or Relapsed AML, MDS, or CML.*

NIH/OBA Receipt Date: 06-18-12.

1207-1170 (Open) Gene Therapy/Phase I/Cancer/CEA-Expressing Liver Metastases/Immunotherapy/In Vitro/Retrovirus/CEA, TCRzeta and CD28/Hepatic Artery Infusion

Katz, Steven C.; Roger Williams Medical Center; Providence, Rhode Island; *Phase I Trial of Intrahepatic Infusion of 2ND Generation Designer T Cells for CEA-Expressing Liver Metastases*.

NIH/OBA Receipt Date: 07-03-12. Not Selected for RAC Public Review: 07-17-12

1207-1171 (Open) Gene Therapy/Phase II/Cancer/Adenocarcinomas/Immunotherapy/In Vitro/Retrovirus/CEA, TCRzeta and CD28/Intravenous Infusion

Junghans, Richard P.; Roger Williams Medical Center; Providence, Rhode Island; *Phase II/Pilot Studies of 2nd Generation Anti-CEA Designer T Cells in Adenocarcinomas*.

NIH/OBA Receipt Date: 07-03-12. Not Selected for RAC Public Review: 07-17-12

1207-1172 (Open) Gene Therapy/Phase I-II/Cancer/Anal Cancer/Immunotherapy/In Vivo/Listeria monocytogenes/Human Papilloma Virus E7 Gene/Intravenous Administration

Safran, Howard; Brown University; Providence, Rhode Island*; A Phase I/II Evaluation of ADXS11-001, Mitomycin, 5-fluorouracil (5-FU) and IMRT for Anal Cancer*.

NIH/OBA Receipt Date: 07-10-12. Not Selected for RAC Public Review: 07-31-12

1207-1173 (Open) Gene Therapy/Phase I/Cancer/Head and Neck/Vector-directed Cell Lysis/Vesicular Stomatitis Virus (VSV)/Tumor Lysis/Intratumoral Injection

Merchan, Jaime R.; University of Miami; Miami, Florida; *Phase I Trial of Intratumoral Administration of Recombinant Vesicular Stomatitis Virus Expressing Human Interferon Beta (VSV-h-IFNβ) in Patients with Recurrent/Metastatic Squamous Cell Carcinoma of the Head and Neck*.

NIH/OBA Receipt Date: 07-16-12. Not Selected for RAC Public Review: 08-06-12

1207-1174 (Open) Gene Therapy/Phase I/Cancer/Metastatic Melanoma/Immunotherapy/In Vitro/Retrovirus/Tumor Infiltrating T lymphocytes/NGFR cDNA/Dominant Negative TGFβ Receptor II (DNRII) cDNA/Intravenous Injections

Hwu, Patrick; University of Texas MD Anderson Cancer Center; Houston, Texas; *Lymphodepletion Plus Adoptive Cell Transfer with TGF-β Resistant (DNRII) and NGFR Transduced T-Cells Followed by High Dose Interleukin-2 in Patients with Metastatic Melanoma*.

NIH/OBA Receipt Date: 07-16-12. Not Selected for RAC Public Review: 08-07-12

1207-1175 (Open) Gene Therapy/ Phase I-II/Cancer/Prostate/Immunotherapy/In Vitro/Autologous Dendritic Cells/Adenovirus/Serotype 5/CD40 cDNA/PSMA cDNA/AP1903Intradermal Injection

Fong, Lawrence; University of California San Francisco; San Francisco, California; *A Phase I/II Study of BPX-201 Vaccine plus AP1903, in Patients with Metastatic Castrate Resistant Prostate Cancer (mCRPC)*. Sponsor: Bellicum Pharmaceuticals.

NIH/OBA Receipt Date: 07-17-12. Not Selected for RAC Public Review: 08-07-12

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1207-1176 (Open) Gene Therapy/Phase I-IIa/Cancer/ Hepatocellular Carcinoma/Immunotherapy/In Vivo/Lentiviral Vector/Autologous CD4+ and CD8+ T cells/NY-ESO-1/LAGE-1 /Intravenous Injections

Kaplan, David; University of Pennsylvania; Philadelphia, Pennsylvania; *A Phase I/IIa, Open Label, Clinical Trial Evaluating the Safety and Efficacy of Autologous T Cells Expressing Enhanced TCRs Specific for NY-ESO-1/LAGE-1 in Patients with Unresectable Hepatocellular Carcinoma*. Sponsor: Adaptimmune Ltd.

NIH/OBA Receipt Date: 07-17-12. Not Selected for RAC Public Review: 08-07-12

1207-1177 (Open) Gene Therapy/Phase I-IIa / Cancer/ Hepatocellular Carcinoma/Immunotherapy/In Vivo/ Lentiviral Vector / Autologous CD4+ and CD8+ T cells/NY-ESO-1/LAGE-1/Intravenous Injections

Stadtmauer, Edward; University of Pennsylvania; Philadelphia, Pennsylvania; Rapoport, Aaron; University of Maryland; Baltimore, Maryland; and Htut, Myo; City of Hope; Duarte, California; *A Phase I/IIa Open Label, Multiple Site Clinical Trial Evaluating the Safety and Activity of Engineered Autologous T cells Expressing an Affinity-enhanced TCR Specific for NY-ESO-1 and LAGE-1, in Patients with Relapsed or Progressive Disease following Prior Auto-HSCT*. Sponsor: Adaptimmune Ltd.

NIH/OBA Receipt Date: 07-17-12. Not Selected for RAC Public Review: 08-07-12

1207-1178 (Open) Gene Therapy/Phase I/Cancer/Pancreatic/Immunotherapy/In Vitro/RNA Transfer (Electroporation)/α-mesothelin-scFv with Signaling Domains Comprised of TCRζ, CD28, and 4-1BB cDNA/Intravenous Injection for First Cycle; Second Cycle may be Intraperitoneal Administration

Beatty, Gregory L.; University of Pennsylvania; Philadelphia, Pennsylvania; *Phase I Clinical Trial of Autologous Mesothelin Re-directed T Cells in Patients with Chemotherapy Refractory Metastatic Pancreatic Cancer*.

NIH/OBA Receipt Date: 07-17-12. Not Selected for RAC Public Review: 08-07-12

1207-1179 (Open) Gene Therapy/Phase I/Cancer/HER2 Expressing Solid Tumors (1+ to 3+)/Immunotherapy/In Vivo/Adenovirus/Human Epidermal Growth Factor Receptor-2/Intradermal Administration

Wood, Lauren V. National Institutes of Health; Bethesda, Maryland; *A Phase I Study of an Adenoviral Transduced Autologous Dendritic Cell Vaccine Expressing Human HER2/neu ECTM in Adults with Tumors with 1-3+ HER2/neu Expression*.

NIH/OBA Receipt Date: 07-17-12. Not Selected for RAC Public Review: 08-07-12

1208-1180 (Under review) Gene Therapy/Phase I/Cancer/Breast/Immunotherapy/In Vivo/Autologous Dendritic Cells/RNA Transfer/RNAs encoding: EGFR, MUC1, MAG-3, Glucocorticoid-induced Tumor Necrosis Factor Receptor Ligand (GITR-L), Humanized Heavy and Light Chains of an Antagonistic Anti-Cytotoxic T-Lymphocyte Antigen 4 (CTLA-4) mAb/Intranodal Injection

Pruitt, Scott K.; Duke University Medical Center; Durham, North Carolina; *Local Modulation of Immune Receptor Function to Enhance Immune Responses to Dendritic Cell Vaccination in Subjects with Triple Negative Breast Cancer*.

NIH/OBA Receipt Date: 08-01-12.

1208-1181 (Open) Gene Therapy/Phase I-II/Cancer/Melanoma/Herpes Simplex Virus Type-1/Vector-Directed Tumor Lysis/Granulocyte-macrophage Colony Stimulating Factor (GM-CSF)/Intratumoral Injection

Kaufman, Howard L.; Rush University Medical Center; Chicago, Illinois; *A Phase 1b/2, Multicenter, Open-label Trial to Evaluate the Safety and Efficacy of Talimogene Laherparepvec and Ipilimumab Compared to Ipilimumab Alone in Subjects with Previously Untreated, Unresectable, Stage IIIb-IV Melanoma*. Sponsor: Amgen, Inc. (BioVex, now a subsidiary of Amgen)

NIH/OBA Receipt Date: 08-30-12. Not Selected for RAC Public Review: 09-21-12

1209-1182 (Under review) Gene Therapy/Phase I/Cancer/Neuroblastoma/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/GD-2-Specific Zeta, CD28, OX40 T Cell Receptor/iCaspase/Intravenous Injections

Louis, Chrystal; Baylor College of Medicine; Houston, Texas; *Autologous Activated T-Cells Transduced with a 3rd Generation GD-2 Chimeric Antigen Receptor and iCaspase9 Safety Switch Administered to Patients with Relapsed or Refractory Neuroblastoma (GRAIN)*.

NIH/OBA Receipt Date: 09-05-12.

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1209-1183 (Open) Gene Therapy/Phase I/Cancer/Non-Hodgkin's Lymphoma/Immunotherapy/In Vitro/Autologous Central Memory T Lymphocytes (Tcm)/Lentivirus/CD19 antigen specific Chimeric Antigen Receptor (CAR)/Epidermal Growth Factor Receptor cDNA/Intravenous Injections

Popplewell, Leslie; City of Hope; Duarte, California; *Phase I Study of Cellular Immunotherapy Using Central Memory Enriched T Cells Lentivirally Transduced to Express a CD19-Specific, CD28-Costimulatory Chimeric Receptor and a Truncated EGFR Following Peripheral Blood Stem Cell Transplantation for Patients with High-Risk Intermediate Grade B-Lineage Non-Hodgkin Lymphoma*.

NIH/OBA Receipt Date: 09-14-12. Not Selected for RAC Public Review: 10-05-12

1209-1184 (Open) Gene Therapy/Phase II-III/Cancer/Non-small Cell Lung Cancer/Immunotherapy/In Vitro/Allogeneic Tumor Cells/Retrovirus/α(1,3)galactosyltransferase gene/intradermal injection

Morris, John C.; University of Cincinnati; Cincinnati, Ohio; *An Open-label, Randomized Phase IIb/III Active Control Study of Second-line HyperAcute®-Lung (Tergenpumatucel-L) Immunotherapy versus Docetaxel in Progressive or Relapsed Non-Small Cell Lung Cancer*. Sponsor: NewLink Genetics Corporation

NIH/OBA Receipt Date: 09-14-12. Not Selected for RAC Public Review: 10-05-12

1209-1185 (Open) Gene Therapy/Phase II/Cancer/MDS/AML/Immunotherapy/In Vitro/Autologous leukemia cells/Adenovirus/GM-CSF cDNA/Intradermal and Subcutaneous Injection

Dranoff, Glenn; and Ho, Vincent; Dana-Farber Cancer Institute; Boston, Massachusetts; *A Randomized Placebo-controlled Phase II Trial of Irradiated, Adenovirus Vector Transfected GM-CSF Secreting Autologous Leukemia Cell Vaccination (GV AX) Versus Placebo Vaccination in Patients with Advanced MDS/ AML after Allogeneic Hematopoietic Stem Cell Transplantation*.

NIH/OBA Receipt Date: 09-14-12. Not Selected for RAC Public Review: 10-05-12

1209-1186 (Open) Non-Therapeutic (Health Volunteers)/Phase I/ /Human Immunodeficiency Virus/Plasmid/HIV-1 Env, Gag, Pol cDNAs/Interleukin-12 cDNA/Intradermal or Intramuscular Injections via Electroporation

Edupuganti, Srilatha; Emory University School of Medicine; Atlanta, Georgia; *A Phase 1 Clinical Trial to Evaluate the Safety and Immunogenicity of PENNVAX®-GP (gag, pol, env) DNA Vaccine, with or without IL-12 Plasmid, Delivered via Intradermal or Intramuscular Electroporation in Healthy, HIV-1–uninfected Adult Participants*. Sponsor: HIV Vaccine Trials Network

NIH/OBA Receipt Date: 09-27-12. Not Selected for RAC Public Review: 10-19-12

1210-1187 (Open) Gene Therapy/Phase I/II/Monogenic Disease/ Sickle Cell Disease/In Vitro/Autologous CD34+ Cells/Lentivirus/Human β-Globin gene/Intravenous

Schiller, Gary; University of California, Los Angeles; Los Angeles, California; *Clinical Research Study of Autologous Bone Marrow Transplantation for Sickle Cell Disease (SCD) using Bone Marrow CD34+ Cells Modified with the Lenti/βAS3-FB Lentiviral Vector*.

NIH/OBA Receipt Date: 10-04-12. Not Selected for RAC Public Review: 10-26-12

1210-1188 (Under review) Gene Therapy/Phase I-II/Monogenic Disease/Spinal Muscular Atrophy Type 1/In Vivo/Adenovirus Associated Virus Serotypes 2 and 9/Survival Motor Neuron cDNA/Intravenous

Mendell, Jerry; The Research Institute at Nationwide Children's Hospital; Columbus, Ohio; *Phase I Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type 1 Delivering the Survival Motor Neuron Gene by Self-complementary AAV9*.

NIH/OBA Receipt Date: 10-09-12.

1210-1189 (Under review) Gene Therapy/Phase II/Cancer/Breast/Immunotherapy/In Vitro/tumor Cells/Adenovirus/Interleukin-12 cDNA/ RheoSwitch® Therapeutic System/Intratumoral Injection

Nemunaitis, John; Mary Crowley Research Center; Dallas, Texas; *A Phase II, Randomized, Open Label Study of Ad-RTS-hIL-12 Monotherapy or Combination with Palifosfamide-Tris in Subjects with Recurrent/Metastatic Breast Cancer and Accessible Lesions*. Sponsor: Ziopharm Oncology, Inc.

NIH/OBA Receipt Date: 10-09-12.

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1210-1190 (Under review) Gene Therapy/Phase I/Other/Secondary Lymphedema Associated with the Treatment of Breast Cancer /In Vitro/Fat Tissue Surrounding Lymph Nodes/Adenovirus/Serotype 5/Vascular Endothelial Growth Factor cDNA/Perinodal Injection Prior to Transplantation

Rockson, Stanley; Stanford University School of Medicine; Stanford, California; *A Phase I Open–Label Study to Assess the Safety, Tolerability and Preliminary Efficacy of LX-1101(Lymfactin™, VEGF-C Adenoviral Vector) in the Treatment of Patients with Secondary Lymphedema Associated with the Treatment of Breast Cancer*. Sponsor: Laurantis Pharma Ltd.

NIH/OBA Receipt Date: 10-09-12.

1210-1191 (Open) Gene Therapy/Phase I/Cancer/Non-Hodgkin's Lymphoma/Chronic Lymphocytic leukemia/Immunotherapy/In Vitro/Autologous T Lymphocytes/Retrovirus/CD19 antigen specific-Zeta T Cell Receptor/Intravenous Injections

Ramos, Carlos A.; Baylor College of Medicine; Houston, Texas; *SAGAN – Phase I Study of Activated T-Cells Expressing Second or Third Generation CD19-Specific Chimeric Antigen Receptors for Advanced B-Cell Non-Hodgkin's Lymphoma*.

NIH/OBA Receipt Date: 10-09-12. Not Selected for RAC Public Review: 10-31-12

1210-1192 (Under review) Gene Therapy/Phase I/Cancer/B-cell Chronic Lymphocytic Leukemia/Immunotherapy/In Vitro/Autologous CD4+ and CD8+ T lymphocytes /Sleeping Beauty (SB) Transposon/CD19 Antigen Specific-Zeta T Cell Receptor/Intravenous Injections

Wierda, William and Cooper, Laurence; The University of Texas MD Anderson Cancer Center; Houston, Texas; *A Study to Infuse ROR1-Specific Autologous T Cells for Patients with CLL* (MDACC Protocol 2012-0932).

NIH/OBA Receipt Date: 10-09-12.

1210-1193 (Open) Gene Transfer/Phase I/Cancer/Melanoma/Immunotherapy/In Vivo Electroporation/Plasmid DNA/Interleukin-12 cDNA with Tumor Targeting Peptide NVTANST/Intratumoral In Vivo Electroporation

Hwu, Wen-Jen; The University of Texas MD Anderson Cancer Center; Houston, Texas; *A Phase I Study of Intratumoral Tumor-targeted Interleukin-12 Plasmid DNA (ttIL-12 pDNA) Gene Therapy via Percutaneous Electroporation in Patients with Refractory Metastatic Melanoma and In Transit Metastases*.

NIH/OBA Receipt Date: 10-09-12. Not Selected for RAC Public Review: 10-31-12

1210-1194 (Open) Gene Therapy/Phase III/Cancer/Renal Cell Carcinoma/Immunotherapy/In Vitro/Autologous Dendritic Cells/RNA Transfer/Total Tumor RNA/Plasmids/CD40L cDNA/Intravenous Infusion

Figlin, Robert; Cedars-Sinai Medical Center; Los Angeles, California; *An International Phase 3 Randomized Trial of Autologous Dendritic Cell Immunotherapy (AGS-003) Plus Standard Treatment of Advanced Renal Cell Carcinoma (ADAPT)*. Sponsor: Argos Therapeutics, Inc.

NIH/OBA Receipt Date: 10-09-12. Not Selected for RAC Public Review: 10-31-12

(Scroll down for Summary Table)

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HUMAN GENE TRANSFER PROTOCOLS

(please see explanation of review levels below)

	Review Level 1	Review Level 2	Review Level 3	Review Level 4	Review Level 5	Review Level 6	Review Level 7	TOTAL
MARKING	23	2	5	0	0	10	1	41
THERAPY	83	5	92	5	13	718	203	1119
NON-THERAPEUTIC	1	0	0	0	0	11	6	18
INFECTIOUS DISEASES	8	1	11	1	0	35	10	66
1. Human Immunodeficiency Virus	8	1	11	1	0	29	7	57
2. Other viral diseases	0	0	0	0	0	6	3	9
MONOGENIC DISEASES	20	1	9	0	1	28	40	99
1. Alpha-1-Antitrypsin Deficiency	1	0	0	0	0	1	2	4
2. Chronic Granulomatous Disease	1	0	1	0	0	1	0	3
3. Cystic Fibrosis	10	1	5	0	0	6	2	24
4. Familial Hypercholesterolemia	1	0	0	0	0	0	1	2
5. Fanconi Anemia	1	0	0	0	0	4	1	6
6. Storage Diseases (Gaucher, Hunter, Galactosialidosis)	4	0	0	0	0	0	2	6
7. Ornithine Transcarbamylase Deficiency	0	0	1	0	0	0	0	1
8. Purine Nucleoside Phosphorylase Deficiency	1	0	0	0	0	0	0	1
9. SCID	1	0	1	0	0	3	5	10
10. Leukocyte Adherence Deficiency	0	0	1	0	0	0	0	1
11. Canavan Disease	0	0	0	0	0	1	2	3
12. Hemophilia	0	0	0	0	0	3	6	9
13. Muscular Dystrophy (Including Pompe)	0	0	0	0	0	1	4	5
14. Epidermolysis Bullosa	0	0	0	0	0	0	2	2
15. Retinal Disorders (e.g., LCA)	0	0	0	0	0	4	3	7
16. Neuronal Ceroid Lipofuscinosis	0	0	0	0	0	2	1	3
17. Blood disorders (non-Hemophilia)	0	0	0	0	0	2	3	5
18. Inclusion Body Myopathy	0	0	0	0	0	0	1	1
19. Familial Adenomatous Polyposis	0	0	0	0	0	0	1	1
20. Wiskott-Aldrich	0	0	0	0	0	0	1	1
21. Adrenoleukodystrophy	0	0	0	0	0	0	2	2
22. Obesity Prader-Willi	0	0	0	0	0	0	1	1
23. Spinal Muscular Atrophy	0	0	0	0	1	0	0	1
OTHER DISEASES / DISORDERS	2	0	2	2	3	80	57	146
1. Peripheral Artery Disease	1	0	0	1	0	38	6	46
2. Arthritis	1	0	0	0	0	1	4	6
3. Arterial Restenosis	0	0	1	0	0	1	1	3
4. Heart Failure	0	0	0	0	0	5	4	9
5. Cubital Tunnel Syndrome	0	0	1	0	0	0	0	1
6. Coronary Artery Disease	0	0	0	1	0	16	7	24
7. Alzheimer's Disease	0	0	0	0	0	0	3	3
8. Ulcer	0	0	0	0	0	3	3	6
9. Bone Fracture	0	0	0	0	1	0	0	1
10. Peripheral Neuropathy	0	0	0	0	0	4	1	5
11. Parkinson's Disease	0	0	0	0	0	1	6	7
12. Epilepsy	0	0	0	0	0	0	1	1
13. Eye Disorders	0	0	0	0	0	4	5	9
14. Erectile Dysfunction	0	0	0	0	0	0	1	1
15. Intractable Pain	0	0	0	0	0	0	2	2
16. Autoimmune Disease	0	0	0	0	0	3	4	7
17. Salivary gland hypofunction	0	0	0	0	0	0	1	1
18. Overactive Bladder Syndrome	0	0	0	0	0	0	1	1
19. Renal	0	0	0	0	0	1	1	2
20. Allergy	0	0	0	0	0	0	1	1
21. Stroke	0	0	0	0	0	0	1	1
22. Cachexia	0	0	0	0	0	0	2	2
23. Oral mucositis	0	0	0	0	0	1	1	2
24. Amyotrophic Lateral Sclerosis	0	0	0	0	1	1	0	2
25. Non-malignant disorders	0	0	0	0	0	0	1	1
26. Wound Healing	0	0	0	0	0	1	0	1
27. Lymphedema	0	0	0	0	1	0	0	1

CANCER *(by approach)*	53	3	70	2	9	575	96	808
1. Antisense	4	0	0	0	1	8	0	13
2. Chemoprotection	4	0	4	0	0	6	1	15
3. Immunotherapy/In Vitro Transduction	22	2	19	0	3	229	27	302
4. Immunotherapy/In Vivo Transduction	7	0	28	1	3	215	22	276
5. Pro-drug/HSV-TK and Ganciclovir	12	1	10	0	2	24	12	61
6. Tumor Suppressor Gene	3	0	6	0	0	29	3	41
7. Single Chain Antibody	0	0	2	0	0	0	0	2
8. Oncogene Down-Regulation	1	0	1	1	0	7	0	10
9. Vector-Directed Cell Lysis/Death	0	0	0	0	0	41	21	62
10. Dominant Negative Mutation	0	0	0	0	0	3	2	5
11. Radiotherapy	0	0	0	0	0	1	1	2
12. Apoptosis Induction/Tumor Necrosis	0	0	0	0	0	5	5	10
13. Suicide Gene	0	0	0	0	0	3	1	4
13. Immunotoxin	0	0	0	0	0	2	1	3
14. RNA Interference	0	0	0	0	0	2	0	2
TOTAL GENE TRANSFER PROTOCOLS *(THERAPY, MARKING and NON-THERAPEUTIC)*	107	7	97	5	13	739	210	*1178

Note:** ***The total number of protocols classified in the above table does not equal the total number on this list. Protocol 9903-295 has been withdrawn; Protocol 9907-331 was replaced by protocol 0004-393; Protocols 9910-347 and 9910-348 have been withdrawn; Protocol 9910-349 was replaced by protocol 0010-427; Protocol 0001-374 was replaced by protocol 0007-407; Protocol 0001-375 was replaced by protocol 0010-425; Protocol 0001-377 has been withdrawn; Protocols 0001-383 and 0001-384 have been withdrawn; Protocol 0107-492 was replaced by protocol 0110-499; Protocol 0207-547 has been withdrawn; Protocol 0010-417 has been replaced by protocol 0503-701; Protocol 0610-811 is a duplicate submission of protocol 0502-697; Protocol 1007-1046 has been withdrawn; Protocol 1010-1077 was replaced by 1102-1087.

Review Level 1 = Full RAC review + NIH Director Approval + FDA Investigational New Drug (IND) approval. This review process is no longer in effect.

Review Level 2 = Accelerated RAC Review + NIH Office of Recombinant DNA Activities (ORDA) Approval + FDA IND Approval. This review process is no longer in effect.

Review Level 3 = Sole FDA Review Recommended by NIH/ORDA. Simultaneous submission to NIH (ORDA) required for the purpose of data monitoring and adverse event reporting. This review process is no longer in effect.

Review Level 4 = Sole FDA Review [submission to NIH (OBA) not required]. This is only for non-NIH funded (either direct or collaborative) institutions who elect to submit to NIH (OBA) under voluntary compliance.

Review Level 5 = Received by NIH (OBA). Review level pending.

Review Level 6 = Not Selected for RAC Public Review. Submission to NIH (OBA) required for the purpose of data monitoring and adverse event reporting. This review process is currently in effect.

Review Level 7 = Full RAC discussion + FDA approval. This review process is currently in effect.