Rare Disease Patient Advocacy Day
Food and Drug Administration (FDA)
March 1, 2012

Photos courtesy of Maureen Stewart, FDA

Participants arrived at the Rare Disease Patient Advocacy Day meeting.

ICF International employees prepared to greet participants at the registration table.

Dr. Gayatri Rao, Acting Director, Office of Orphan Products Development, provided opening remarks.

Ms. Tiffany House, President, Acid Maltase Deficiency Association, spoke about her experiences as a patient advocate.

Dr. Anne Pariser, Associate Director for Rare Diseases, Office of New Drugs, spoke about the drug development process.

Dr. Margaret Hamburg, Commissioner of the Food and Drug Administration, addressed participants.

Participants listened as Dr. Markham Luke, Deputy Office Director for Clinical Policy, Office of Device Evaluation, discussed rare diseases from a medical device perspective.

Mr. Robert Knutzen, Pituitary Network Association, reviewed the FDA's patient representative program brochure.

Mr. Frank Sasinowski, National Organization for Rare Disorders (NORD), moderated the first panel discussion where participants presented questions to panelists.

Panelists answered and discussed questions from participants.

Ms. Tiffany House, President of the Acid Maltase Deficiency Association, answered and discussed questions from participants during the panel session.

Ms. Diane Dorman, Vice President of Public Policy, NORD, and Mr. Richard Klein, Director, Patient Liaison Program, Office of Special Health Issues, engaged in conversation during a break.

CDR Emily Thakur, U.S. Public Health Service, discussed FDA's Drug Shortage Program.

Dr. Stephen Groft, Director, National Institutes of Health (NIH), Office of Rare Diseases Research, discussed the various programs at NIH.

Dr. Sharon Terry, President & CEO, Genetic Alliance and Mr. John Burke, Policy Advisor, FDA, listened as Dr. Stephen Groft discussed the programs at NIH.

Dr. Sharon Terry, President & CEO, Genetic Alliance, moderated the second panel discussion where participants presented questions to panelists.

Dr. Stephen Spielberg, Deputy Commissioner for Medical Products and Tobacco, delivered the keynote address.

Dr. Ameeta Parekh, Senior Advisor, Office of Translational Sciences; Dr. Anne Pariser, Associate Director for Rare Diseases, Office of New Drugs; and Dr. Christopher Leptak, Biomarker Lead, Office of New Drugs, engaged participants during the Regulatory Science breakout session.

Ms. Shawne Suggs-Anderson, Center for Food Safety and Applied Nutrition, engaged participants during the Regulation of Medical Foods breakout session.

Dr. Gayatri Rao, Acting Director, Office of Orphan Products Development, moderated the Senior Town Hall panel and provided closing remarks.