THAT ALWAYS HAPPENS. GOOD AFTERNOON, EVERYONE. THANK YOU FOR COMING TODAY.

I WANT TO WELCOME ALL OF YOU TO THE NIH. I'M JOHN BURKLOW, ASSOCIATE DIRECTOR FOR COMMUNICATIONS AND PUBLIC LIAISON AT NIH.

THE RESPONSE TO THE INVITATION TO JOIN US HERE TODAY WAS OVERWHELMING AND YOUR PRESENCE IS CERTAINLY PROOF OF THAT.

IT'S ALSO PROOF OF YOUR CRITICAL IMPORTANCE TO THE MISSION OF THIS INSTITUTION, TO THE MEMBERS YOU SERVE, AND TO OUR NEWEST DIRECTOR OF THE NIH, DR. FRANCIS COLLINS.

I BELIEVE YOU'RE FAMILIAR WITH DR. COLLINS, SO I WON'T SPEND TIME ON HIS BIOGRAPHY. I'LL MENTION A FEW THINGS.

DR. COLLINS IN THE HUMAN GENOME RESEARCH INSTITUTE COMPLETED THE HUMAN GENOME PROJECT AHEAD OF SCHEDULE AND UNDER BUDGET AND ALSO RECEIVED THE PRESIDENTIAL MEDAL OF FREEDOM IN 2007, THE HIGHEST CIVIL HONOR THAT A PRESIDENT CAN BESTOW. HE'S AN ELECTED MEMBER OF THE INSTITUTE OF MEDICINE AND THE NATIONAL ACADEMY OF SCIENCES. HE'S ALSO REMAINED A WORLD-RENOWNED GENETICIST, A TIRELESS ETHICIST, AND A FIERCE ADVOCATE FOR ALL AMERICANS TO ENSURE PRIVACY AND PREVENT DISCRIMINATION. HE LIKES TO BREAK NEW GROUND, LIKE TODAY.

I HAVE BEEN AT NIH A LONG TIME AND I BELIEVE THIS IS THE FIRST MEETING OF ITS KIND AND SIZE. IT REFLECTS DR. COLLINS' COMMITMENT TO OPENNESS AND COMMUNITY.

A FEW WORDS BEFORE WE BEGIN.

FIRST, THIS IS BEING TAPED FOR ARCHIVING ON OUR NIH WEBSITE FOR FUTURE REFERENCE. AFTER DR. COLLINS SPEAKS WE'LL OPEN IT UP FOR QUESTIONS AND ANSWERS. WE INVITED EACH OF YOU TO SUBMIT COMMENTS AND QUESTIONS BEFORE THE MEETING, AND DR. COLLINS HAS SOME OF THEM HERE TODAY WITH HIM. IF WE DON'T GET TO YOUR QUESTION, DON'T WORRY, WE'LL SEE TO IT THAT HE GETS YOUR QUESTION, AND THE STAFF AS WELL.

WE HAVE MICROPHONES IN THE AISLE AND WE'LL TAKE QUESTIONS FROM THE FLOOR, PERHAPS IN AN ALTERNATING FASHION. SINCE WE HAVE SO MANY GUESTS HERE WHO MAY WANT TO ASK QUESTIONS, PLEASE KEEP YOUR QUESTION BRIEF AND TO THE POINT. IF I SENSE THAT

YOU'RE MOVING AWAY FROM THE QUESTION MODE TO THE SPEECH MODE, I'LL START CLEARING MY THROAT. IF YOU CONTINUE, I'LL BREAK INTO A FULL-FLEDGED COUGH. IF THAT DOESN'T WORK, DR. COLLINS WILL START COUGHING, TOO. BUT INTO HIS ELBOW, OF COURSE. REGARDLESS, YOU'LL GET THE HINT.

SO THERE'S A LOT OF GROUND TO COVER TODAY. SO AGAIN I WANT TO WELCOME YOU AND INTRODUCE THE DIRECTOR OF THE NATIONAL INSTITUTES OF HEALTH, DR. FRANCIS COLLINS. [APPLAUSE]

THANKS, JOHN, FOR THAT KIND INTRODUCTION, AND GOOD AFTERNOON TO ALL OF YOU. IT'S GREAT TO SEE AN AMAZING TURN-OUT FOR THIS FIRST-OF-ITS- KIND CONSTITUENTS' MEETING. THIS IS NOT QUITE THE END OF MY FIRST MONTH, SO I'M STILL THE NEW KID ON THIS PARTICULAR BLOCK, ALTHOUGH OTHER PARTS OF THE BLOCK ARE FAMILIAR TO ME FROM HAVING HAD THE PRIVILEGE OF LEADING THE GENOME INSTITUTE SINCE 1993. BUT HERE IN THE BEGINNING OR THE MIDDLE OF THE FOURTH WEEK OF BEING NIH DIRECTOR, I WANTED VERY MUCH TO HAVE A CHANCE TO MEET COLLECTIVELY WITH ALL OF YOU TO TELL YOU A BIT ABOUT SOME OF THE IDEAS THAT HAVE BEEN PERCOLATING ABOUT AREAS OF EXCEPTIONAL OPPORTUNITY THAT NIH NOW SEES IN FRONT OF THEM, AND PERHAPS PARTICULARLY TO LISTEN TO YOUR CONCERNS AND WE WILL BE SURE TO KEEP A BIG CHUNK OF THIS HOUR FOR THOSE QUESTIONS THAT YOU MAY WANT TO POSE AND MY ATTEMPT TO PROVIDE ANSWERS, ALTHOUGH I SUSPECT THERE WILL BE AREAS THAT YOU HAVE QUESTIONS ABOUT THAT I WILL HAVE TO GET BACK TO YOU ABOUT SINCE SOME OF THESE ISSUES ARE STILL RELATIVELY NEW.

I WANT TO, BEFORE I GO ANY FURTHER, SAY A WORD OF THANKS, SPECIAL THANKS TO DR. RAYNARD KINGTON, WHO HAS SERVED ABLY AS THE ACTING DIRECTOR OF NIH SINCE LAST NOVEMBER, AND RAYNARD IS SITTING RIGHT HERE IN THE FRONT ROW AND I THINK WE SHOULD GIVE HIM A ROUND OF APPLAUSE. [APPLAUSE]

I DON'T KNOW IF LARRY TABAK IS HERE, I'M NOT SPYING HIM LOOKING ACROSS THE AUDIENCE, BUT I WANT TO EXPRESS THANKS TO LARRY TABAK, WHO SERVED AS THE ACTING DEPUTY DIRECTOR DURING THAT SAME TIME FRAME AND ESPECIALLY FOCUSED ON THE RECOVERY ACT AND OPPORTUNITIES IT PROVIDED FOR NIH RESEARCH AND WHO HAS AGREED TO CONTINUE TO HELP WITH RECOVERY ACT ISSUES EVEN AS HE'S ABLE NOW TO STEP BACK A LITTLE BIT FROM SOME OF THOSE INTENSE INVOLVEMENTS AND CONTINUE TO OVERSEE HIS OWN INSTITUTE, THE NIDCR. I ALSO WANT TO RECOGNIZE SOMEONE WHO IS

HERE WITH US, A FORMER CONGRESSMAN WHO IS RESPONSIBLE FOR THE FACT THAT NIH SAW SUCH AN IMPRESSIVE INCREASE IN THEIR SUPPORT DURING HIS TIME AS CHAIRMAN OF THE APPROPRIATIONS SUBCOMMITTEE OF THE HOUSE LABOR-H COMMITTEE, AND THAT'S JOHN PORTER. JOHN, THANK YOU FOR BEING HERE. [APPLAUSE]

SO IN THINKING ABOUT TODAY AND ESPECIALLY ABOUT THE RESPONSIBILITY AND THE MISSION THAT WE HAVE AT NIH TO TRY TO DO SOMETHING FOR THOSE WHO ARE AFFLICTED WITH CONDITIONS, MANY DIFFERENT TYPES AND THEIR FAMILIES WHO ALSO WRESTLE WITH THE CONSEQUENCES OF THOSE ILLNESSES, COULDN'T HELP BUT THINK BACK TO THE MOMENT AT WHICH I FIRST DECIDED MYSELF THAT THIS WAS A CALLING TO WORK IN MEDICAL RESEARCH.

I HAD INITIALLY STARTED OUT MY LIFE AS A CHEMIST. THAT WAS THE FIRST SCIENCE I ENCOUNTERED THAT I THOUGHT WAS EXCITING, INTELLECTUALLY STIMULATING. WHEN I HAD A COURSE IN THAT SUBJECT AS A 10TH GRADER IN A PUBLIC HIGH SCHOOL ABOUT THREE HOURS FROM HERE I FELT THAT WAS IT, THAT WAS GOING TO BE MY LIFE'S WORK, FIGURING OUT ALL OF THOSE ISSUES ABOUT HOW ATOMS AND MOLECULES BIND TOGETHER AND GO THROUGH CHEMICAL REACTIONS. I THOUGHT BIOLOGY BY CONTRAST AT THAT POINT WAS PROBABLY THE MOST BORING OF ALL THE SCIENCES. THAT'S PARTLY, I'M AFRAID, A CONSEQUENCE OF THE WAY IT WAS TAUGHT AND PERHAPS IS STILL TAUGHT IN MANY INSTANCES AS A DESCRIPTIVE SCIENCE THAT AT LEAST, IN MY 10TH GRADE VIEW, DIDN'T HAVE A LOT GOING FOR IT AS FAR AS PRINCIPLES OR INTELLECTUAL STIMULATION. IT WAS ALL ABOUT MEMORIZING THE PARTS OF THE CRAYFISH, AND I KNEW I DIDN'T CARE ABOUT THAT.

SO IT CAME AS A SHOCK TO ME AFTER I HAD GONE THROUGH COLLEGE AS A CHEMISTRY MAJOR AND WENT ON TO GRADUATE SCHOOL TO GET A PH.D. IN CHEMISTRY THAT I RECOGNIZED I KIND OF MISSED THE BOAT AND REALIZED THAT ACTUALLY BIOLOGY IS RICH WITH EXCITING DETAILED DIGITAL INFORMATION. THAT WOULD BE DNA, WOULDN'T IT?

SO I CHANGED DIRECTIONS, SURPRISED AT MYSELF FOR DOING SO AND WENT OFF TO MEDICAL SCHOOL TO TRY TO LEARN ABOUT THE SCIENCE OF THE HUMAN BODY. BUT AT THAT POINT I WAS NOT CONVINCED THAT RESEARCH WAS WHAT I SHOULD BE DOING WITH THE REST OF MY LIFE.

IT WAS IN THAT FIRST YEAR AS A MEDICAL STUDENT IN A MEMORABLE SERIES OF LECTURES GIVEN BY A PEDIATRICIAN WHO ALSO HAPPENED TO BE A GENETICIST, WHO BROUGHT PATIENTS TO OUR CLASS FOR US TO

INTERVIEW, TO INTERACT WITH, AND HELPED US SEE HOW THIS BASIC SCIENCE INVESTIGATION OF THE MOST BASIC ASPECTS OF LIFE, DNA AND RNA AND PROTEIN, COULD HAVE FUNDAMENTAL CONSEQUENCES FOR HUMAN BEINGS.

A YOUNG MAN WITH SICKLE CELL DISEASE CAME AND SPOKE TO US, AN OLDER WOMAN WITH NEUROFIBROMATOSIS, A CHILD WITH DOWN SYNDROME, A NEWBORN WITH GALACTOSEMIA, ALL OF THESE AS EXAMPLES OF HOW ONE SMALL GLITCH IN THE GENOME COULD CAUSE A GREAT DEAL OF MISERY FOR THE AFFECTED INDIVIDUAL AND GREAT CONSEQUENCES FOR THEIR FAMILIES AS WELL.

I HAD THE SENSE THAT IF I WAS GOING TO BE INVOLVED IN MEDICINE IT WOULD NOT BE SATISFYING TO SIMPLY LEAVE THOSE PROBLEMS TO SOMEONE ELSE TO TRY TO FIGURE OUT, THAT THIS WAS PROBABLY SOMETHING I WANTED TO TURN MY ATTENTION TO.

THAT WAS 35 YEARS AGO. AND NEVER DURING THOSE EARLY MONTHS OF TRYING TO IMAGINE A PATHWAY COULD I HAVE CONTEMPLATED BEING HERE IN FRONT OF YOU HERE THIS AFTERNOON HAVING THIS ENORMOUS PRIVILEGE OF BEING ASKED TO SERVE AS THE DIRECTOR OF THE NATIONAL INSTITUTES OF HEALTH, THE MOST SIGNIFICANT ORGANIZATION SUPPORTING BIOMEDICAL RESEARCH IN THE WORLD.

AND I CONSIDER IT A GREAT HONOR INDEED TO HAVE BEEN CHOSEN BY PRESIDENT OBAMA TO TAKE ON THIS TASK, AND I'M HONORED BY IT AND DAUNTED BY IT, AND I'M GOING TO NEED YOUR HELP.

THAT'S PART OF WHAT THIS AFTERNOON IS ABOUT, IS FOR ME TO LISTEN TO SOME OF YOUR CONCERNS AND SEE HOW NIH CAN SERVE THEM AS WELL AS WE POSSIBLY CAN.

I'M STEPPING INTO A ROLE THAT HAS BEEN BRILLIANTLY PLAYED BY PREDECESSORS. I MET JIM SHANNON, THE PERSON WHO PRESIDED OVER NIH WAY BACK 40, 50 YEARS AGO, AND FOR WHOM THE BUILDING MY OFFICE IS NOW IN IS CALLED, BUILDING 1 BUT IT'S ALSO THE SHANNON BUILDING, IS NAMED, AND OF COURSE MORE RECENTLY LEGENDARY LEADERS SUCH AS HAROLD VARMUS AND ELIAS ZERHOUNI, HAVE PLACED THIS ORGANIZATION IN WONDERFUL SHAPE, AND I'M IN THEIR DEBT TO BE ABLE TO STEP INTO A CIRCUMSTANCE THAT'S ALREADY IN SUCH GOOD SHAPE.

BUT I'M PARTICULARLY THRILLED TO BE, AT THIS TIME IN HISTORY, ABLE TO COME BEFORE YOU NOMINATED BY A PRESIDENT WHO HAS CLEARLY STOOD UP FOR THE VALUE OF SCIENCE, THE VALUE OF SCIENCE AS A POTENTIAL ANSWER TO A LOT OF THE QUESTIONS THAT FACE US IN OUR

COUNTRY AND IN OUR WORLD, AND WHO SEES SCIENCE AS AN IMPORTANT VOICE THAT NEEDS TO BE AT THE TABLE WHEN DECISIONS ARE BEING MADE, WHO SEES SCIENCE AS IN NEED OF SUPPORT AS FAR AS ITS INTEGRITY, WHO SEES PARTICULAR AREAS OF SCIENCE THAT NEED GREAT EMPHASIS BECAUSE OF THEIR POTENTIAL AS AREAS THAT OUGHT TO BE SUPPORTED AND NOT HAVE POLITICAL INFLUENCES GET IN THE WAY, AND A PRESIDENT WHO SEES OPENNESS AS AN AREA OF GREAT IMPORTANCE ACROSS GOVERNMENT.

IN A RECENT STATEMENT FROM HIS, PRESIDENT OBAMA SAID OPENNESS WILL STRENGTHEN OUR DEMOCRACY AND PROMOTE EFFICIENCY AND EFFECTIVENESS IN GOVERNMENT. I PLEDGE TO SUPPORT THAT AND I BELIEVE NIH SHOULD BE AT THE FOREFRONT OF THAT ATTITUDE OF OPENNESS, SHARING INFORMATION. AND TODAY IS INTENDED TO BE ONE EXAMPLE OF THAT.

SO I WANTED TO SHOW YOU A COUPLE OF SLIDES AND THEN PARTICULARLY TO TELL YOU A LITTLE BIT ABOUT AREAS THAT I THINK ARE EXCITING SCIENTIFICALLY, BUT THEN WE WILL LEAVE PLENTY OF TIME FOR YOUR QUESTIONS.

THE MISSION OF NIH, FAMILIAR TO MOST OF YOU, RIGHT THERE IN THE MISSION STATEMENT MAKES IT CLEAR, THAT WE'RE IN PURSUIT OF FUNDAMENTAL KNOWLEDGE ABOUT THE NATURE AND BEHAVIOR OF LIVING SYSTEMS, SO THIS IS THE MORE BASIC SCIENCE PART OF OUR MISSION, BUT ALSO, THE APPLICATION OF THAT KNOWLEDGE, TO EXTEND HEALTHY LIFE AND REDUCE THE BURDENS OF ILLNESS AND DISABILITY.

IT IS THIS WONDERFUL TENSION BETWEEN BASIC AND CLINICAL SCIENCE THAT CHARACTERIZES MANY OF OUR DISCUSSIONS, AND IT'S A PRODUCTIVE TENSION AND NOT ONE THAT CAUSES DIFFICULTIES, BUT IT IS ALWAYS ON OUR MINDS.

HOW DO WE MAKE THE MOST BOTH OF THE RESEARCH OPPORTUNITIES TO UNDERSTAND THE FUNDAMENTALS BUT SPARE NO EFFORT TO APPLY THEM QUICKLY IN THE TRANSLATIONAL REALM? I'LL COME BACK TO THAT IN A MOMENT.

MANY PEOPLE, ASSUMING THAT BECAUSE I HAD THE PLEASURE AND PRIVILEGE OF LEADING THE HUMAN GENOME PROJECT, ASSUMED THAT I WOULD COME TO NIH FOCUSED ON BIG SCIENCE AND NOT SO INTERESTED PERHAPS IN THE EFFORTS OF INDIVIDUAL INVESTIGATORS WHO SEND IN THEIR GRANT PROPOSALS BASED ON THEIR OWN CAREFULLY CRAFTED HYPOTHESES. THAT WOULD NOT BE CORRECT.

I THINK THE BEDROCK OF NIH'S EFFORTS IS INVESTIGATOR-INITIATED RESEARCH. THAT IS WHERE SO MUCH OF THE BREAKTHROUGHS HAVE OCCURRED DOWN THROUGH THE DECADES AND WILL CONTINUE TO AS THIS QUOTE FROM JIM SHANNON, WHO I MENTIONED A MINUTE AGO, EMPHASIZES.

AND SO WE WILL CONTINUE TO THINK PARTICULARLY OF YOUNG INVESTIGATORS BUT IN FACT OF INVESTIGATORS ACROSS THEIR CAREER SPANS WHO COME TO US WITH THESE BRIGHT, CREATIVE IDEAS. WE WILL CONTINUE TO TRY TO MODERATE OUR TENDENCY PERHAPS TO EXPECT PEOPLE TO HAVE ALREADY DONE THE EXPERIMENT BEFORE THEY SEND IN THE GRANT PROPOSAL, WHICH HAS BEEN AN ISSUE, AND WE WILL CONTINUE TO PUSH THE INNOVATION AGENDA AS PART OF THE WAY OUR PEER REVIEW LOOKS AT WHAT COMES TO US.

WE'RE IN THE PROCESS RIGHT NOW OF FAIRLY SIGNIFICANT OVERHAUL OF OUR PEER REVIEW PROCESS WITH SPECIFIC FOCUS ON TRYING TO ENCOURAGE INNOVATION. THIS INVESTIGATOR-INITIATED RESEARCH HAS BEEN A WONDERFUL ENGINE OF PROGRESS.

JUST TWO NICE IMAGING EXAMPLES OF THAT HERE, MITOCHONDRIA HERE AND THIS 3-D IMAGE OF AN ENZYME COMPLEX THAT CONVERTS PYROVATE TO ACETYL-COA HERE, SHOWING HOW THE MOLECULES COME INTO THIS AMAZING COMPLEX, GET CHEMICALLY CONVERTED AND GO OUT AGAIN.

THE NUMBER OF THESE ACTIVITIES THAT'S GOING ON RIGHT NOW AND THE DEPTH OF MOLECULAR UNDERSTANDING THEY'RE PROVIDING US HOW LIFE WORKS IS TRULY BREATHTAKING. AND IF YOU'RE INTERESTED IN UNDERSTANDING HOW WHEN DISEASE STRIKES SOMETHING GOES WRONG, HAVING THIS KIND OF FUNDAMENTAL BASIC INFORMATION ALWAYS TURNS OUT TO BE CRITICAL.

I WILL SAY, OF COURSE, THAT THERE ARE ALSO AREAS WHERE BIG SCIENCE CAN EMPOWER EVERYONE. WHEN I SEE PROJECTS LIKE THE GENOME PROJECT, OR SOME OF THOSE THAT HAVE FOLLOWED AFTER, THAT WILL IN FACT PUT TOOLS INTO THE HANDS OF INVESTIGATORS THAT ENABLE THEM TO GO FASTER TO GET ANSWERS MORE QUICKLY, TO USE THEIR RESOURCES IN THE MOST EFFICIENT WAY, THEN WE'RE GOING TO TRY TO FIGURE OUT HOW TO SUPPORT THOSE AS WELL, WITH AN EMPHASIS THAT THE BIG SCIENCE PROJECTS HAVE SPECIAL RESPONSIBILITY TO RELEASE THEIR DATA ESSENTIALLY IMMEDIATELY SO THAT EVERYONE ELSE CAN BEGIN TO TAKE ADVANTAGE OF THOSE DISCOVERIES.

IF YOU'RE INTERESTED IN HOW NIH IS CURRENTLY INVESTING THE RESOURCES WE ARE GIVEN BY THE TAXPAYERS, A NEW TOOL YOU MAY NOT HAVE ENCOUNTERED IS SOMETHING YOU MIGHT WANT TO BOOKMARK AND LOOK AT, CALLED RePORT WHICH STANDS FOR RESEARCH PORTFOLIO ONLINE REPORTING TOOL. THIS, WHICH HAS JUST BEEN ENABLED IN THE COURSE OF THE LAST COUPLE OF MONTHS, ALLOWS YOU TO GO AND SEE WITH A VARIETY OF DIFFERENT KINDS OF SEARCHES WHAT IS IT THAT ACTUALLY NIH IS CURRENTLY SUPPORTING.

YOU CAN SEARCH BY FISCAL YEAR. IF YOU HAPPEN TO BE IN THE POLITICAL REALM, WHICH SOME PEOPLE SEEM TO BE, CONGRESSIONAL DISTRICT OR STATE, OR YOU CAN SEARCH BY THE INSTITUTE OR THE CENTER, BY FUNDING MECHANISM, BY AWARD TYPE, BY ACTIVITY CODE, BY STUDY SECTION, A VARIETY OF OTHER WAYS IN WHICH YOU CAN QUERY THE DATA AND GO RIGHT DOWN TO THE INDIVIDUAL GRANT AND SEE WHAT WE'RE DOING.

THIS IS ANOTHER STEP IN THE DIRECTION OF TRYING TO BE AS TRANSPARENT AS WE POSSIBLY CAN. PARTICULARLY TO ALL OF YOU, OUR PARTNERS, OUR COLLABORATORS, OUR CONSTITUENTS, WHO WE BELIEVE HAVE THE RIGHT TO KNOW WHAT WE'RE DOING AND WHY WE'RE DOING IT.

NOW I WANT TO TURN TO THESE OPPORTUNITIES THAT I SEE AS PARTICULARLY EXCITING RIGHT NOW HERE IN 2009 AS WAYS IN WHICH NIH COULD PARTICULARLY PUSH THE BALL FORWARD IN TERMS OF SPECIFIC INVESTMENTS.

AFTER A LOT OF THOUGHT AND DISCUSSION WITH A LOT OF PEOPLE I SEE THESE BREAKING DOWN INTO FIVE THEMES, FIVE OPPORTUNITIES. AND I WANT TO QUICKLY WALK THROUGH WHAT THOSE ARE. AGAIN, PLEASE PAY NO ATTENTION TO THE DETAILS HERE BECAUSE YOU SHOULD NOT BE KEEPING SCORE ON WHAT GETS MENTIONED AND WHAT DOES NOT, THAT'S NOT THE POINT HERE.

THESE OPPORTUNITIES ARE IN FACT FAIRLY BROAD, AND I WOULD SUBMIT WHATEVER DISORDER YOU'RE PARTICULARLY INTERESTED IN SEEING NIH WORK ON ONE OR MORE OF THESE OPPORTUNITIES HOLDS GREAT PROMISE.

ONE OF THEMES I WANTED TO EMPHASIZE TODAY IS THAT THE WAY SCIENCE IS MOVING OVER THE COURSE OF THE LAST FEW YEARS IS BREAKING DOWN BOUNDARIES BETWEEN OUR UNDERSTANDING OF DISEASE, THAT WHAT WE THOUGHT WE WERE DOING TO STUDY CANCER TURNS OUT TO TELL US SOMETHING CRUCIAL ABOUT HEART DISEASE, THAT WHAT WE THOUGHT WE WERE DOING STUDYING SOME RARE

DISORDER THAT MAYBE ONLY EFFECTS A FEW HUNDRED PEOPLE TURNS OUT TO HAVE PROFOUND IMPLICATIONS FOR PEOPLE WITH HIGH BLOOD PRESSURE OR ASTHMA THAT AFFECTS MILLIONS OF PEOPLE.

SO THE NOTION THAT WE COULD APPROACH MEDICAL RESEARCH IN A COMPARTMENTALIZED WAY IS TURNING OUT TO BE WRONG IN TERMS OF THE SCIENCE INVOLVED, AND THEREFORE WE HAVE ALL THE MORE REASON IN A PLACE LIKE THIS TODAY TO THINK ABOUT HOW WE CAN WORK TOGETHER TO TRY TO RAISE ALL THOSE BOATS, ALL THOSE BOATS YOU CARE ABOUT, AND I DO AS WELL, BUT WHICH PROBABLY INDIVIDUALLY CAN'T MOVE AS WELL AS IF THEY CAN MOVE TOGETHER BY UNDERSTANDING SCIENTIFICALLY HOW WE'RE CONNECTED AND OF COURSE HOW WE'RE CONNECTED IN OTHER WAYS AS WELL, INCLUDING POLITICALLY.

OPPORTUNITY NUMBER 1 IS THE APPROACH THAT WE CAN NOW TAKE USING NEW TECHNOLOGIES THAT ARE REALLY BREATHTAKING TO UNDERSTAND HOW LIFE WORKS, AND HOW IN SOME INSTANCES IT DOESN'T QUITE DO WHAT IT'S SUPPOSED TO WHEN A DISEASE ARISES. GENOMICS IS ONE OF THOSE TECHNOLOGIES AND IT HAS BEEN AN ABSOLUTE CHANGE IN PARADIGM FOR OUR ABILITY TO UNDERSTAND A LONG LIST OF DISEASES, BUT IT'S NOT THE ONLY ONE.

NANOTECHNOLOGY, THE MARRIAGE OF BIOLOGY AND CHEMISTRY, IMAGING AND COMPUTATIONAL BIOLOGY, ARE VERY MUCH ALSO EMPOWERING IN THEIR ABILITY TO SHED LIGHT ON DISEASES THAT WE HAVE NOT UNDERSTOOD WELL ENOUGH IN THE PAST TO BE ABLE TO MAKE GOOD INFERENCES ABOUT HOW TO INTERVENE.

JUST TO NAME A COUPLE OF INSTANCES WHERE THIS KIND OF EFFORT IS LIKELY TO PAY BIG DIVIDENDS IN THE NEXT FEW YEARS. CANCER WOULD BE AT THE TOP I THINK OF MOST PEOPLE'S LIST BECAUSE WE KNOW CANCER IS A DISEASE OF DNA. THEREFORE THE ABILITY TO CATALOG ALL OF THE THINGS THAT HAVE GONE WRONG IN A CANCER CELL NOT JUST LOOKING UNDER THE LAMP POST AS WE HAVE BEEN FORCED TO DO IN THE PAST BUT LOOKING AT THE ENTIRE GENETIC SCRIPT TO IDENTIFY IN HUNDREDS OF TUMORS OF A PARTICULAR TYPE. WHAT IS THE CATALOG OF GLITCHES THAT HAVE OCCURRED THAT HAVE MADE A GOOD CELL GO BAD AND START THE PROCESS WE RECOGNIZE AS CANCER?

THERE'S A REAL REVOLUTION IN THE OFFERING HERE DEMONSTRATED IN A PILOT EFFORT BY THE CANCER GENOME ATLAS FOR BRAIN TUMORS AND SOON TO BE EXPANDED DRAMATICALLY TO A LONG LIST OF OTHER CANCERS AS WELL. THIS WILL CHANGE FOREVER OUR VIEW OF WHAT CANCER IS ALL ABOUT AND WHAT TO DO TO PREVENT AND TO TREAT IT.

THERE ARE OTHER DISEASES WHERE THIS SAME APPROACH CAN BE APPLIED—SUCH AS HEART DISEASE—FOR INSTANCE, IN THE FRAMINGHAM STUDY. WHAT REALLY ARE THE THINGS THAT ARE CAUSING PARTICULAR ILLNESSES TO APPEAR, AUTISM, OBESITY, ALZHEIMER'S DISEASE? YOU SAW A COUPLE OF ANNOUNCEMENTS ABOUT THAT THIS WEEK AND ON DOWN THE LIST.

THE MICROBIOME PROJECT SHOWN IN THIS PICTURE HERE PRESENTS ANOTHER EXCITING OPPORTUNITY TO APPLY THESE NEW TECHNOLOGIES TO UNDERSTAND THE WAY IN WHICH HUMANS AND THE MICROBES THAT LIVE ON US AND IN US INTERACT IN WAYS THAT MAY PROMOTE OUR HEALTH, OR IF THINGS GO AWRY, MAY CAUSE DISEASE. THERE'S MORE MICROBECAL CELLS ON YOU AND IN YOU THAN THERE ARE OF YOUR OWN CELLS, THAT MAY NOT BE A HAPPY THOUGHT BUT IT'S THE FACT, AND THE ABILITY TO UNDERSTAND THAT BETTER WHEN MANY OF THESE MICROBES HAVE NOT BEEN POSSIBLE TO ISOLATE IN THE LABORATORY IS EXCITING, BECAUSE THEY HAVE DNA, SO NOW WE CAN FIND OUT ABOUT THEM. YOU'RE GOING TO SEE A LOT HAPPENING IN THAT AREA. SO THAT WOULD BE ONE AREA OF OPPORTUNITY.

A SECOND ONE, ONE I CONFESS I HAVE A PARTICULAR PASSION FOR, IS TO TAKE THESE BASIC DISCOVERIES ABOUT DISEASES, RARE DISEASES AND COMMON DISEASES, AND TRY TO UNDERSTAND HOW TO APPLY THEM THERAPEUTICALLY. THIS IS THE TRANSLATIONAL AGENDA. THIS IS HARD.

THIS PICTURE HERE, YOU MIGHT NOT RECOGNIZE THIS PART OF THE COUNTRY BUT IT'S OFTEN A YAWN AND GAP BETWEEN BASIC RESEARCH AND DEVELOPMENT OF EFFECTIVE THERAPEUTICS. WHAT ONE NEEDS, AS YOU CAN SEE IN THIS MORE MODERN PICTURE OF SAN FRANCISCO, IS TO BUILD THIS BRIDGE.

MANY PEOPLE HAVE IN THE PAST BASICALLY ASCRIBED THAT RESPONSIBILITY TO THE PRIVATE SECTOR, AND IN FACT BIOTECHNOLOGY AND PHARMACEUTICAL COMPANIES DO THIS AND DO IT VERY WELL, AND DO IT BRILLIANTLY, BUT THE NUMBER OF NEW TARGETS THAT ARE TURNING UP IS VASTLY GREATER THAN ANY COMPANIES COULD ATTEND TO ALL BY THEMSELVES.

AND CERTAINLY FOR RARE DISEASES WHERE THERE'S A LIMITED MARKET POTENTIAL, IT'S UNLIKELY THAT THE PRIVATE SECTOR IS LIKELY TO INVEST THE KIND OF RESOURCES NECESSARY TO TAKE THAT BASIC DISCOVERY AND MOVE IT IN THE DIRECTION OF THERAPEUTICS.

SO PUTTING TOGETHER PUBLIC/PRIVATE PARTNERSHIPS, AND ONE WHICH IS ALREADY UNDERWAY IS CALLED THE "TRND" PROGRAM—THERAPEUTICS FOR RARE AND NEGLECTED DISEASES—WILL BRING

TOGETHER ACADEMIC INVESTIGATORS WHO MAY KNOW MORE THAN ANYBODY ON THE PLANET ABOUT THE BASIC MOLECULAR PROBLEM IN A DISEASE, EMPOWER THEM TO BEGIN THE? PROCESS OF BUILDING THIS BRIDGE BY GETTING TO THE POINT OF SOME OF THE FIRST COMPOUNDS THAT LOOK AS IF THEY MIGHT HAVE PROMISE.

AND AT THE POINT WHERE SOME OF THE RISK HAS ALREADY BEEN TAKEN ON AND REMOVED BY NIH SUPPORT, COMPANIES MAY VERY WELL AT THAT POINT SEE THIS AS AN OPPORTUNITY TO MAKE AN INVESTMENT, AND THEN A PARTNERSHIP IS BORN THAT COULD BE WONDERFULLY BENEFICIAL FOR THE PUBLIC.

MANY MORE OF THESE ARE COMING ALONG. IT'S A VERY EXCITING TIME TO SEE THAT KIND OF TRANSLATIONAL OPPORTUNITY EMERGING. AND IT'S NOT JUST ABOUT SMALL-MOLECULE THERAPY, IT'S ABOUT BIOLOGICS AND IT'S ABOUT OTHER APPROACHES, GENE THERAPY OR STEM CELL THERAPY.

WE'RE ALL EXCITED BY THE PRESIDENT'S EXECUTIVE ORDER FREEING UP THE OPPORTUNITY FOR ACADEMIC INVESTIGATORS TO WORK WITH HUMAN EMBRYONIC STEM CELLS IN WAYS THAT HAVE NOT BEEN POSSIBLE WITH NIH SUPPORT IN THE RECENT PAST AND WHAT THE EXPECTATION IS WITH THAT RESEARCH AND DEVELOPMENT OF IPS CELLS, THESE REMARKABLE INDUCED PLURIPOTENT STEM CELLS, THE THERAPEUTIC DEVELOPMENTS ARE LIKELY TO OCCUR BUT WILL NEED A LOT OF SUPPORT. IT'S RATHER UNCLEAR AT THE MOMENT EXACTLY JUST WHAT THAT PROMISE IS GOING TO BE, BUT WE NEED TO MOVE THAT BALL FORWARD AS QUICKLY AS POSSIBLE.

SO ANOTHER REALLY IMPORTANT OPPORTUNITY, TRANSLATION. A THIRD OPPORTUNITY, AND MUCH I MUST SAY ON THE MINDS OF MANY PEOPLE RIGHT NOW, IS PUTTING SCIENCE TO WORK FOR THE BENEFIT OF HEALTH CARE REFORM. THIS EVENING THE PRESIDENT WILL SPEAK ABOUT THIS. IF YOU LOOK AT ALL THE ISSUES THAT SWIRL ABOUT OUR HEALTH CARE ISSUES IN THIS COUNTRY, THE COST OF HEALTH CARE EVER RISING HAS TO BE A BIG ONE. THIS PARTICULAR DIAGRAM HERE NOT ONLY SHOWS YOU, YOU CAN'T READ THE DETAILS, BUT THIS IS THE UNITED STATES AS AN OUTLIER WHERE WE BOTH SPEND A LOT OF MONEY ON HEALTH CARE AND WE DON'T SEEM TO ENJOY THE BENEFITS OF IT IN TERMS OF LONGEVITY. SO WE HAVE INEFFICIENCIES, TO SAY THE LEAST, IN OUR SYSTEM.

ONE OF THE AREAS THAT NIH HAS BEEN WORKING ON FOR SOME TIME AND WHICH IS NOW ENCOURAGED FOR US TO DO EVEN MORE, IS THIS AREA OF COMPARATIVE EFFECTIVENESS RESEARCH WHERE WE STUDY ALTERNATIVE APPROACHES TO A GIVEN CLINICAL PROBLEM AND TRY TO

DETERMINE WHAT WORKS. WE HAVE TO BE THOUGHTFUL ABOUT THAT, BECAUSE YOU WOULD NOT WANT TO LOSE THE INDIVIDUAL IN THE PROCESS OF THOSE STUDIES. AND FOR ME, SOMEBODY WHO HAS BEEN IN FAVOR OF THE IDEA OF PERSONALIZED MEDICINE FOR SOME TIME, THIS IS A BIT OF A CONCERN. BUT I BELIEVE THERE ARE WAYS TO DESIGN THOSE STUDIES IN ORDER TO ACHIEVE BOTH GOALS. NOT ONLY DO WE NEED TO DO COMPARATIVE EFFECTIVENESS RESEARCH, WE NEED TO INVEST MORE IN THE AREA OF UNDERSTANDING PHARMACOGENOMICS, HOW IT IS THAT DIFFERENT DRUGS WORK FOR DIFFERENT PEOPLE IN DIFFERENT WAYS.

THE PERSONALIZED MEDICINE AGENDA NEEDS TO BE PUT FORWARD. WE NEED TO UNDERSTAND THE CAUSES AND INTERVENTIONS FOR HEALTH DISPARITIES IS A MAJOR IMPORTANT TASK. NONE OF THIS IS GOING TO WORK WELL IF WE DON'T INVEST IN SOCIAL AND BEHAVIORAL RESEARCH, BECAUSE A LOT OF THE HOPE FOR INTERVENTIONS ARE GOING TO REQUIRE OUR UNDERSTANDING HOW PEOPLE ABSORB INDIVIDUAL INFORMATION AND ACTUALLY ALTER THEIR OWN HEALTH BEHAVIORS.

I THINK AN AREA OF GREAT INTEREST IS ACTUALLY HEALTH CARE RESEARCH ECONOMICS. WHAT IS IT THAT NIH COULD DO TO BETTER UNDERSTAND HOW WE COULD IMPLEMENT A PLAN FOR INTERVENING IN OUR EVER-GROWING HEALTH CARE COSTS THAT WOULD REIGN IN COSTS BUT IMPROVE OUTCOMES? ARE THERE, FOR INSTANCE, PAYMENT INCENTIVE MODELS THAT COULD BE COMPARED WITH EACH OTHER IN A RESEARCH ENVIRONMENT TO ANSWER SOME OF THE QUESTIONS THAT ARE CURRENTLY FLOATING OUT THERE?

THE FOURTH OPPORTUNITY, ONE WHICH I THINK IS PARTICULARLY TIMELY AT THE MOMENT, IS TO FOCUS OUR ATTENTION AS MUCH AS WE CAN ON GLOBAL HEALTH. OBVIOUSLY WITH MANY OTHER PARTNERS INVOLVED IN THIS, WHO AND OF COURSE THE GATES FOUNDATION AND OTHER PHILANTHROPIES, BUT AGAIN WE'VE ARRIVED AT A POINT WHERE OUR MOLECULAR UNDERSTANDING OF MANY OF THESE DISEASES THAT ARE PARTICULARLY COMMON IN THE DEVELOPING WORLD HAS COME QUITE A DISTANCE. AND THE OPPORTUNITY TO TAKE THAT INFORMATION AND APPLY IT THERAPEUTICALLY HAS NEVER BEEN BETTER.

THE SAME ARGUMENTS I MADE ABOUT RARE DISEASES CAN BE MADE FOR NEGLECTED DISEASES, THAT THERE'S NOT A STRONG ECONOMIC INCENTIVE AT THE MOMENT FOR THE PRIVATE SECTOR TO SHOULDER THIS EFFORT ENTIRELY BY THEMSELVES, BUT WITH PARTNERSHIP WITH NIH WE BELIEVE THIS COULD BE MOVED FORWARD, AND I COULD ALREADY CITE EXAMPLES WHERE THAT'S STARTING TO HAPPEN, SUCH AS THE DISEASE FOR THIS WORM WHICH THERE'S ALREADY BEEN THE FIRST

NEW EXCITING DRUG DEVELOPMENT THERAPY IN 50 YEARS ON THE BASIS OF NIH RESEARCH.

THE FIFTH ONE REALLY COVERS OVER ALL OF THE OTHER AREAS OF SCIENCE BUT IS CRITICAL FOR OUR FUTURE. WE COULD DO EVERYTHING POSSIBLE TO TRY TO COME UP WITH NEW IDEAS AND TECHNOLOGIES BUT IF WE DON'T HAVE THE PEOPLE TO DO THE RESEARCH, IF THOSE PEOPLE DON'T SEE FOR THEMSELVES THE POSSIBILITY OF A CAREER THAT'S GOING TO SUPPORT INNOVATION AND CREATIVITY, THEN WE'RE GOING TO CONTINUE TO LOSE OUT IN TERMS OF OUR STANDING IN THE WORLD. WE HAVE LOST QUITE A BIT IN THE LAST FEW YEARS IN TERMS OF OUR ABILITY TO BE COMPETITIVE.

BECAUSE OF COURSE THE NIH BUDGET WAS HELD FLAT FROM 2003 TO 2008, AND DURING THAT TIME CLEARLY INVESTIGATORS HAD A GREATER AND GREATER DIFFICULTY GETTING THEIR RESEARCH FUNDED. UP UNTIL ABOUT 2008, THE LIKELIHOOD, IF YOU SENT IN A GRANT TO NIH, OF ACTUALLY GETTING FUNDED FELL TO ABOUT ONE CHANCE IN FIVE. AND NOBODY WOULD ARGUE THAT THAT'S HEALTHY FOR OUR PARTICULAR ENVIRONMENT, ESPECIALLY FOR YOUNG INVESTIGATORS WHOSE ARE TRYING TO FIGURE OUT IS THERE A STABLE CAREER FOR THEM HERE.

SO WE DO NEED TO FOCUS ON TRYING TO SUPPORT THE COMMUNITY IN THE BEST WAY WE CAN. I THINK THAT ALSO MEANS IN TRYING TO MAKE THE CASE FOR THE VALUE OF MEDICAL RESEARCH FOR ANYONE WHO IS INTERESTED IN LISTENING.

AND WE NEED TO TAKE THE RESOURCES THAT WE ARE GRANTED BY THE ADMINISTRATION AND THE CONGRESS AND APPLY THEM CREATIVELY. ONE AREA OF PARTICULAR INTEREST IN THE LAST FEW YEARS HAS BEEN THE SO-CALLED ROADMAP FOR MEDICAL RESEARCH. AS THE NIH DIRECTOR IT'S MY JOB TO OVERSEE THE WAY IT'S ADMINISTERED. THIS IS A GRAND OPPORTUNITY ACTUALLY TO FUND RESEARCH THAT DOESN'T FIT INTO A SPECIFIC INSTITUTE BUT MIGHT BENEFIT LOTS OF INSTITUTES AND LOTS OF DISEASES. WE WILL BE LOOKING AT WAYS TO CREATIVELY UTILIZE THOSE RESOURCES.

WE HAVE ALSO USED SOME OF THOSE RESOURCES TO TRY TO ENCOURAGE PARTICULARLY INNOVATIVE SCIENTIFIC PROPOSALS AS REPRESENTED HERE BY THE PIONEER AWARDS. THERE'S ALSO OTHER AWARDS CALLED NEW INNOVATORS. AND TRANSFORMATIVE RO1s, WHICH ARE ALSO ENCOURAGING INVESTIGATORS TO COME FORWARD WITH THINGS THAT ARE REALLY SOMEWHAT RISKY, AND MAYBE THEY DON'T HAVE ALL THE PRELIMINARY DATA, BUT IF IT HAPPENED TO WORK THIS WOULD BE TRANSFORMATIONAL.

WE'RE ENCOURAGING AS MUCH OF THAT AS POSSIBLE, NEW IDEAS. WE NEED TO WORK ON OUR TRAINING PROGRAMS. WE NEED TO PARTICULARLY REACH OUT AND TRY TO BRING INDIVIDUALS INTO OUR RESEARCH COMMUNITY WHO ARE TRADITIONALLY UNDER-REPRESENTED.

I THINK WE NEED TO ENCOURAGE THE WHOLE COMMUNITY TO FEEL THE SENSE OF COHESION AND SUPPORT, WHICH HAS BEEN A LITTLE TOUGH IN THE LAST FEW YEARS FOR A LOT OF OUR PARTICIPANTS.

I CAN'T FINISH THIS PRESENTATION, THOUGH, ABOUT THOSE OPPORTUNITIES WITHOUT POINTING OUT THAT WE HAVE HAD AN EXCEPTIONAL OPPORTUNITY COME IN FRONT OF US IN JUST THE LAST FEW MONTHS. THAT IS BY THE AVAILABILITY OF FUNDS, $10 BILLION IN FACT, FOR NIH COMING FROM THE ARRA—AMERICAN RECOVERY AND REINVESTMENT ACT OF 2009. IN ADDITION TO THE $10 BILLION FOR NIH RESEARCH IN GENERAL, ANOTHER 400 MILLION WAS GIVEN TO US FOR COMPARATIVE EFFECTIVENESS RESEARCH.

ONE OF THE MAIN TASKS THAT I HAVE BEEN INVOLVED IN OVER THE LAST COUPLE OF WEEKS, SINCE JUST GETTING HERE, WAS TO OVERSEE HOW BEST WE COULD ALLOCATE THOSE FUNDS HERE AT THE END OF THE FISCAL YEAR IN ORDER TO GET THE GRANTS FUNDED AND OUT THE DOOR, INTO THE HANDS OF INVESTIGATORS WHO COULD USE IT, AND THE GOALS OF ARRA ARE TO STIMULATE THE ECONOMY. THE INTENTION WAS TO TRY TO PULL US OUT OF WHAT WAS CLEARLY THE GREATEST THREAT TO OUR ECONOMY SINCE THE GREAT DEPRESSION, TO CREATE AND PRESERVE JOBS, WHICH NIH DOES QUITE WELL.

EVERY GRANT WE GIVE ON THE AVERAGE CREATES ABOUT 7 JOBS. AND EVERY DOLLAR THAT NIH GIVES OUT TO A GRANTEE PAYS BACK MORE THAN $2 OF ECONOMIC GOODS AND SERVICES CREATED IN LESS THAN A YEAR. SO IF YOU WANT TO LOOK AT AREAS WHERE INVESTMENTS CAN STIMULATE THE ECONOMY IN A HURRY, CERTAINLY NIH IS NEAR THE TOP OF THAT LIST IN TERMS OF THE EFFICIENCY OF THE OUTCOME.

BUT OF COURSE OUR GOAL IS ALSO TO ADVANCE BIOMEDICAL RESEARCH, TO TAKE ADVANTAGE OF THE PENT-UP DEMAND AND INTEREST BY INVESTIGATORS TO TACKLE SOME REALLY INNOVATIVE AND CHALLENGING PROBLEMS AND ALLOW US ALSO TO TAKE ON SOME REALLY BIG PROJECTS THAT MIGHT OTHERWISE HAVE WAITED FOR SOME TIME.

ONE OF THOSE I HAVE MENTIONED ALREADY IS CANCER. WITH THIS PILOT EFFORT HAVING SHOWN US IT IS POSSIBLE TO BEGIN TO GET A COMPREHENSIVE LOOK AT WHAT GOES WRONG IN THE CANCER CELL

BASED ON BRAIN TUMORS AND OVARIAN CANCER, IT IS NOW INTENDED TO SCALE THAT UP TO TACKLE BETWEEN 20 AND 25 DIFFERENT TUMOR TYPES SO THAT IN THE COURSE OF THE NEXT TWO TO FOUR YEARS WE WILL HAVE THE COMPLETE VIEW OF WHAT GOES ON IN THOSE TUMORS AND WHAT TO DO ABOUT THEM WILL BE MUCH MORE APPARENT BECAUSE WE'LL KNOW WHAT TARGETS WE NEED TO GO AFTER. THAT IS JUST ONE OF A NUMBER OF INITIATIVES.

OTHERS, FOR INSTANCE, ON HIV/AIDS, ON H1N1, A TOPIC ON MANY PEOPLE'S MINDS, AUTISM—I COULD GO DOWN A LONG LIST—HEART DISEASE, HEALTH DISPARITIES, ALL IN HERE AS INITIATIVES SUPPORTED BY THE RECOVERY ACT.

HAVING READ THROUGH HUNDREDS OF SUMMARY STATEMENTS IN THE LAST COUPLE OF WEEKS TRYING TO DO FINE TUNING HOW TO BEST SPEND RECOVERY DOLLARS, I CAN TELL YOU THE SCIENCE REPRESENTED THERE IS TRULY EXCITING. THE OPPORTUNITY TO SEE THE IDEAS THAT OUR COMMUNITY IS COMING UP WITH WHEN GIVEN THE ENCOURAGEMENT TO DO SO IS THE SOURCE OF GREAT EXCITEMENT FOR ME AS JUST THE NEWLY ARRIVED NIH DIRECTOR. SO AMONG THE PROGRAMS THAT ARRA SUPPORTS INCLUDES THINGS THAT HAVE NAMES LIKE CHALLENGE GRANTS AND GRAND OPPORTUNITIES AND SIGNATURE INITIATIVES, ALSO PROVIDING OPPORTUNITY FOR NEW FACULTY AND EVEN SUMMER JOBS, A LOT OF WHICH GOT FUNDED FOR THIS PAST SUMMER TO TRY TO BRING MORE STUDENTS INTO LABORATORIES AND ENCOURAGE THEM TO THINK ABOUT CAREERS IN SCIENCE FOR THEMSELVES.

SO FINALLY THOUGH—AFTER GOING THROUGH THIS ENUMERATION OF WHAT I SEE AS EXCEPTIONAL OPPORTUNITIES—I NEED YOUR HELP.

SCIENCE IS NOT A HUNDRED-YARD DASH, IT'S A MARATHON. AND WHILE WE HAVE SEEN THIS WONDERFUL, TWO-YEAR DELUGE OF FUNDS COMING FROM THE RECOVERY ACT, PROVIDING A REMARKABLE BOOST IN THE MOMENTUM FOR SCIENTIFIC RESEARCH, CLEARLY SCIENCE DOESN'T OPERATE ON TWO-YEAR CYCLES AND THINGS WE GET STARTED IN THIS INTERVAL WILL NEED THAT ONGOING SUPPORT.

I THINK IF WE ARE TO SEE THAT HAPPEN WE ALL NEED TO SPEAK EFFECTIVELY ABOUT THE VALUE OF THIS. SO ONE THING I NEED YOUR HELP WITH IS TO FIGURE OUT HOW TO PROPAGATE THAT COMMON AND CONSISTENT VOICE IN SUPPORT OF THE IMPORTANCE OF MEDICAL RESEARCH.

I THINK A LOT OF PEOPLE IN THE COMMUNITY ARE GENERALLY AWARE OF THAT, BUT TO THE EXTENT THAT WE CAN TELL STORIES, AND STORIES

ARE OFTEN WHAT PEOPLE ARE LOOKING FOR, OF HOW THIS KIND OF RESEARCH HAS REALLY CHANGED LIVES FOR THE BETTER AND WE'RE READY TO DO SO AT THE DROP OF A HAT.

I THINK WE'RE GOING TO GET A BETTER HEARING AMONGST THE DECISION MAKERS. WHATEVER ORGANIZATION YOU REPRESENT, I IMAGINE YOU THINK ABOUT THAT, BUT TO HAVE AT YOUR FINGERTIPS TWO OR THREE COMPELLING STORIES HOW MEDICAL RESEARCH HAS CHANGED LIVES OF PEOPLE WHO ARE INVESTED IN THAT PARTICULAR CONDITION WOULD BE AN APPROPRIATE THING TO ALWAYS BE READY TO HAVE IN CASE SOMEONE ASKS.

AND I THINK WE NEED, IN ADDITION TO THOSE STORIES, OTHER NEW AND COMPELLING WAYS TO DESCRIBE NIH RESEARCH, THAT WE DO IN FACT BENEFIT THE ECONOMY, THAT WE DO IN FACT PROVIDE THE OPPORTUNITY OF BENDING THAT CURVE FOR HEALTH CARE COSTS THAT EVERYONE IS TERRIFIED ABOUT SEEING CONTINUE TO RISE. TO GET THOSE MESSAGES FORMULATED IN A VERY COHERENT AND CRISP WAY.

THEN I NEED YOUR HELP BECAUSE TODAY IS JUST AN HOUR IN THE AFTERNOON ON THE 9TH DAY OF THE 9TH MONTH OF THE 9TH YEAR IN THE MILLENNIUM CALLED 2000, BUT I WOULD LIKE TO SEE THOSE CHANNELS ACTUALLY MORE OPEN THAN PERHAPS YOU HAVE SEEN THEM IN THE PAST. MY STYLE IS VERY MUCH TO TRY TO HEAR FROM AS MANY PEOPLE AS I CAN. I CAN'T ALWAYS ANSWER IN THE WAY I WOULD LIKE BECAUSE THE NEEDS ARE ALWAYS GREATER THAN THE RESOURCES AVAILABLE. BUT I WOULD LIKE FOR TODAY TO BE A BIT OF A BEGINNING IN TERMS OF THAT KIND OF OPENNESS.

IN THAT REGARD I WOULD ASK YOU AS YOU THINK ABOUT THE CONVERSATION THIS AFTERNOON OVER THE NEXT FEW DAYS, PUT TOGETHER FOR ME A BRIEF SUMMARY OF THE ISSUES THAT YOUR ORGANIZATION THINKS THAT I AS THE NIH DIRECTOR AND THOSE 27 INSTITUTE AND CENTER DIRECTORS SHOULD KNOW ABOUT. DON'T MAKE IT TOO LONG BECAUSE WE DO HAVE LIMITATIONS IN THE NUMBER OF HOURS IN THE DAY. A PAGE AND A HALF OR SOMETHING LIKE THAT. BUT JUST HIT—DOESN'T HAVE TO BE ELOQUENT TEXT—EVEN BULLETS OF WHAT YOU SEE AND YOU WOULD WANT THE NIH TO KNOW AS THE MAJOR ISSUES THAT WE SHOULD BE PAYING ATTENTION TO.

AND SEND IT TO THIS EMAIL ADDRESS, NIH-LISTENS@NIH.GOV.

YOU'LL GET AN ECHO BACK THAT IT WAS RECEIVED. THEN IT WILL BE READ. WE WILL TRY IN INSTANCES WHERE THERE'S A NEED TO MAKE A RESPONSE MORE THAN THANK YOU, WE'LL TRY TO DO THAT AS QUICKLY AS POSSIBLE THOUGH IT MAY TAKE A LITTLE WHILE TO DISTILL

THROUGH THIS. THIS WEBSITE WILL CONTINUE TO BE THERE. THIS IS AT LEAST ONE WAY OF HEARING FROM YOU.

I WISH I COULD MEET WITH EACH ONE OF YOU INDIVIDUALLY AND SPEND AN HOUR IN MY OFFICE LISTENING TO WHAT THE CONCERNS ARE OF YOUR ORGANIZATION. THAT WOULD BE A GREAT EDUCATIONAL EXPERIENCE, I'M SURE, FOR ME, BUT I KNOW, GIVEN THE NUMBER OF PRESSURES THAT CURRENTLY FALL UPON NIH, THAT THAT'S NOT PRACTICAL OPPORTUNITY. MAYBE IN SOME SMALL WAY THIS IS ANOTHER WAY FOR ME TO BE ABLE TO TRY TO HEAR FROM YOU ABOUT WHAT'S ON YOUR MIND.

I DO HOPE YOU'LL USE THIS. I PROMISE YOU I WILL PAY CLOSE ATTENTION TO IT. SO WE ARE A COMMUNITY. AND NIH IS A CITY OF HOPE AND HEALING—IN MANY PEOPLE'S VIEW—FOR THE WORLD.

BUT TO SUCCEED AT THAT WE'RE GOING TO HAVE TO DO THIS TOGETHER. WHEN I TALK TO THE STAFF ON THE FIRST DAY I CAME HERE ON AUGUST 17TH I IDENTIFIED MYSELF NOT AS THE PERSON WHO'S ACTUALLY MAKING THE MUSIC, NO ONE CAN WHISTLE A SYMPHONY BUT MAYBE I'M A LITTLE BIT OF A CONDUCTOR HERE AND IT TAKES A TEAM TO MAKE THE MUSIC WE NEED TO MAKE. YOU'RE ALL PART OF THE TEAM. YOU'RE TALENTED MUSICIANS. I'LL TRY IF I CAN NOT TO DROP THE BATON AT THE WRONG MOMENT AND TO TRY TO ENCOURAGE THE MUSIC TO RISE FROM THE GROUP IN A WAY THAT THE WHOLE WORLD WILL LISTEN. WE WILL ONLY SUCCEED IF WE DO THIS TOGETHER.

THANK YOU VERY MUCH. [APPLAUSE]

* * * * * * * * * * *

THANK YOU, DR. COLLINS. WE'LL NOW BEGIN OUR Q&A. THERE ARE MICROPHONES, AND IF YOU WANT TO LINE UP AND DR. COLLINS HAS SOME QUESTIONS AS WELL.

I'M REPRESENTING THE COLITIS FOUNDATION, ALSO PROFESSOR AT UNC CHAPEL HILL, SO WE INTERACTED BACK IN TRAINING DAYS, FRANCIS. [I HAVE] A GASTROENTEROLOGY QUESTION FOR YOU. IN TODAY'S ENVIRONMENT OF LIMITED RESOURCES—BOTH PUBLIC AND PRIVATE—HOW CAN THE NIH AND NOT-FOR-PROFIT ORGANIZATIONS EFFECTIVELY PARTNER TO FUND, TO JOINTLY FUND, INNOVATIVE INITIATIVES OF CONSENSUS HIGH PRIORITY?

[DR. COLLINS] SO A STRATEGY THAT I COULD SEE MIGHT BE AT THE RFA, THE REQUEST FOR APPLICATION MECHANISMS IN WHICH THOSE AREAS OF HIGHEST PRIORITY THAT WERE DEVELOPED BY A SCIENTIFIC PANEL

OF EXPERTS FROM NIH AND THE DISEASE-ORIENTED SOCIETY, COULD SHARE RESPONSIBILITY FOR EITHER PICKING AND CHOOSING DIFFERENT HIGH PRIORITY OR JOINTLY FUNDED PRIORITIES. WE INFORMALLY DO THIS WITH NIDDK. STEVE JAMES ATTENDS OUR STRATEGY SESSIONS BUT THERE'S NO FORMAL MECHANISM. CERTAINLY A JOINT MECHANISM OF FUNDING WILL EXTEND OUR FUNDS THAT ARE LIMITED.

SO THERE HAVE BEEN MODELS OF THAT SORT THAT I'M AWARE OF THAT HAVE BEEN IMPLEMENTED SUCCESSFULLY. OBVIOUSLY LET ME SAY FIRST OF ALL THAT CERTAINLY WHEN NIH IS TRYING TO IDENTIFY AREAS THAT ARE RIPE FOR SCIENTIFIC EXPLORATION, GETTING INPUT FROM NONPROFIT ORGANIZATIONS THROUGH VARIOUS MEANS, INCLUDING THE FACT THAT PEOPLE SIT ON OUR ADVISORY COUNCILS WHO REPRESENT THOSE ORGANIZATIONS, IS OFTEN VERY HELPFUL IN SAYING THE TIME IS RIGHT TO TRY AND RFA ON THIS CONDITION TAKING THIS APPROACH. THEN ONCE YOU HAVE A STUDY SECTION REVIEW, I KNOW OF SOME INSTANCES—AND I THINK THE CF FOUNDATION WAS PARTICULARLY SUCCESSFUL AT THIS—WHERE YOU BASICALLY PARTNER WITH THAT FOUNDATION. NIH CAN ONLY FUND A CERTAIN NUMBER OF THESE BUT AS THE FOUNDATION COMES IN WITH SOME RESOURCES WE CAN GO DEEPER INTO THIS LIST AND NOT HAVE TO REJECT SOME OF THE THINGS WE THOUGHT WERE EXCITING BUT DIDN'T QUITE MAKE OUR PAY LINE. I WOULD THINK THAT KIND OF MODEL IS VERY WORTH PURSUING. I THINK NIH IN GENERAL WOULD BE VERY OPEN TO THOSE KINDS OF CONVERSATIONS ABOUT SUCH MODELS. BUT OBVIOUSLY RESOURCES COMING FROM BOTH PARTNERS IS A CRITICAL WAY TO MAKE THAT A REALITY.

THANK YOU.

[QUESTIONER] THANK YOU VERY MUCH. MY NAME IS MARK HARRINGTON FROM TREATMENT ACTION GROUP IN NEW YORK CITY. WE WORK ON A COLLECTION OF DISEASES THAT AFFECT THE SAME PEOPLE: HIV, TB, AND VIRAL HEPATITIS. I WOULDN'T BE HERE IF IT WEREN'T FOR RESEARCH DONE BY THE NIH IN THE '80s AND THE DRUG INDUSTRY IN THE '90s BECAUSE I CONTRACTED HIV IN 1985. NOW, AS YOU KNOW, BECAUSE OF NIH AND INDUSTRY MORE THAN 30 DRUGS ARE AVAILABLE AROUND THE WORLD AND MORE THAN 44 MILLION PEOPLE ARE RECEIVING THEM. I WANT TO ASK YOU IF YOU WOULD COMMIT TO BEING A STRONG PUBLIC ADVOCATE FOR MULTI-YEAR SUSTAINED INCREASES IN GROWTH FOR THE NIH BUDGET FOR THE NEXT 5 TO 10 YEARS STARTING AT A BASELINE OF $40 BILLION IN FISCAL YEAR 2011.

[DR. COLLINS] YOU KNOW, THIS IS ALWAYS A TRICKY MOMENT FOR A FEDERAL EMPLOYEE WHO IS PART OF THE EXECUTIVE BRANCH AND WHO

CAN BE ACCUSED OF BUDGET BUSTING IF HE MAKES COMMENTS THAT SOUNDED IF OUT OF SYNC WITH HIS BOSSES. CLEARLY THE OPPORTUNITIES IN MEDICAL RESEARCH RIGHT NOW ARE NEVER BETTER. IF YOU WERE TO ASK MY PROFESSIONAL JUDGMENT FOR THE OPPORTUNITIES AND WHAT KIND OF SUPPORT THEY NEED, THE KINDS OF NUMBERS YOU ARE MENTIONING WOULD NOT BE AT ALL OUT OF THE REALM OF WHAT I THINK WE COULD USEFULLY APPLY TO MAKE GREAT ADVANCES. THAT'S PROBABLY AS FAR AS I SHOULD GO.

THANK YOU VERY MUCH.

[APPLAUSE]

[QUESTIONER] I'M MEIGHAN HAUPT WITH THE NATIONAL ASSOCIATION OF STATE MENTAL HEALTH PROGRAM DIRECTORS. THIS IS DR. CANDICE TATE WITH THE NATIONAL COALITION OF DEAF INDIVIDUALS, WHICH IS AN AFFILIATE. DR. TATE HAS A QUESTION.

[QUESTIONER] WELCOME BACK, DR. COLLINS. I APPRECIATE THE OPPORTUNITY. YOU MENTIONED YOU WANT TO BE THE CONDUCTOR FOR THIS MUSICAL GROUP OF US. AND I WANTED TO LET YOU KNOW THAT WE CAN'T HEAR YOUR MUSIC. THERE IS NO RESEARCH OUT THERE ON THE DEAF POPULATION. THERE IS NOT ACCURATE RESEARCH ON MENTAL HEALTH NEEDS OF THE DEAF POPULATIONS. HOW MANY DEAF INDIVIDUALS HAVE MENTAL HEALTH ISSUES? THERE'S NO ACCURATE INFORMATION OR RESEARCH ON THE MENTAL HEALTH TREATMENT FOR DEAF INDIVIDUALS. HOW DO YOU RESPOND TO THAT? WE'RE ONE OF THE GREATEST HEALTH DISPARITIES IN THE COUNTRY FOR OUR POPULATION. WHAT DO I TAKE BACK TO MY PEOPLE?

[DR. COLLINS] YOU'RE TEACHING ME, AS I'M SURE PEOPLE IN THE ROOM COULD, ABOUT NEEDS THAT I MIGHT NOT PREVIOUSLY HAVE BEEN ALL THAT AWARE OF, AND I APPRECIATE YOUR BRINGING THIS TO US ALL. IF I WERE TO INVESTIGATE THAT, I WOULD GO TO JIM BATTEY, DIRECTOR OF THE NATIONAL INSTITUTE ON DEAFNESS AND COMMUNICATION DISORDERS, AND ASK WHAT THE PLANS ARE IN TERMS OF RESEARCH IN THIS PARTICULAR AREA AND WHETHER THERE ARE IDEAS THAT COULD BE PUT FORWARD. AS THE NIH DIRECTOR, WHEN IT COMES TO DISORDERS, WHERE PARTICULAR INSTITUTES HAVE A FOCUS, I WOULD ALWAYS INTEND TO GO DOWN THAT PATH TO TRY TO FIND OUT WHAT THE PLANS ARE. SO I CAN'T GIVE YOU AN ANSWER AT THE MOMENT ABOUT WHAT THOSE PLANS MIGHT BE. BUT I PROMISE YOU, I WILL INVESTIGATE THIS WITH DR. BATTEY AND SEE WHAT THEIR ANSWERS MIGHT BE.

THANK YOU.

[QUESTIONER] THANK YOU. DR. COLLINS, MY NAME IS JIM TURNER. I'M PRESIDENT OF THE AMERICAN COLLEGE HEALTH ASSOCIATION. BUT MY DAY JOB IS I DIRECT THE STUDENT HEALTH CENTER AT THE UNIVERSITY OF VIRGINIA, AND I BRING GREETINGS FROM MR. JEFFERSON'S INSTITUTION.

[DR. COLLINS] THANK YOU. WAHOO. Y'ALL DON'T KNOW WHAT THAT MEANS, BUT WE DO.

[QUESTIONER] ONE OF MY NURSE PRACTITIONERS WENT TO HIGH SCHOOL WITH YOU AND SHE CALLED YOU A BRAINIAC.

[DR. COLLINS] I'M NOT SURE THAT WAS A COMPLIMENT IN HIGH SCHOOL, BUT ANYWAY.

[LAUGHTER]

[QUESTIONER] WE REPRESENT AMERICA'S 18 MILLION COLLEGE STUDENTS AND ARE RESPONSIBLE FOR THEIR CARE. THOUGH CONSIDERED A HEALTHY POPULATION IN GENERAL, THERE ARE OBVIOUSLY CERTAIN PUBLIC HEALTH ISSUES THAT ARE PROMINENT AMONG COLLEGE STUDENTS. SUBSTANCE USE AND ABUSE, SUCH AS BINGE DRINKING, DEPRESSION, SUICIDE, EATING DISORDERS, AND FINALLY CONTAGIOUS DISEASES, STIS, H1N1, 4,000 NEW CASES REPORTED LAST WEEK ON OUR CAMPUSES. DESPITE THESE IMPORTANT HEALTH ISSUES, THERE EXISTS NO NATIONALLY REPRESENTATIVE DATABASE THAT DESCRIBES OR SUMMARIZES THE CLINICAL PROBLEMS THAT WE'RE SEEING ON OUR CAMPUSES. AND UVA AND THE AMERICAN COLLEGE HEALTH ASSOCIATION DID SUBMIT A PROPOSAL FOR YOUR CHALLENGE GRANTS THIS PAST SUMMER, AND BASED ON THE REVIEWERS' COMMENTS WE THOUGHT THERE WAS MINIMAL APPRECIATION FOR THE IMPORTANCE OF THIS PARTICULAR AREA. SO I'M HERE JUST ADVOCATING ON BEHALF OF COLLEGE HEALTH, ASKING THAT NIH WOULD FOCUS SOME ATTENTION ON THAT VERY, VERY IMPORTANT AREA TO HELP US BETTER UNDERSTAND OUR COLLEGE STUDENTS.

THANK YOU VERY MUCH, AND CONGRATULATIONS ON BEING NAMED NIH DIRECTOR.

[DR. COLLINS] THANK YOU VERY MUCH. I WILL TAKE THOSE COMMENTS UNDER ADVISEMENT. I DO RECALL THAT GRANT COMING THROUGH, AND I'M SORRY, THE REVIEWERS WEREN'T QUITE AS ENTHUSIASTIC AS YOU WOULD HAVE LIKED, BUT OBVIOUSLY I WILL TELL YOU FOR ANYBODY ELSE WHO IS IN THE AUDIENCE WHO KNOWS OF A GRANT THAT CAME IN THAT THEY THOUGHT WAS SUPER BUT DIDN'T GET FUNDED, THE

NUMBERS ARE JUST ASTOUNDING HERE. WHEN WE ISSUED THAT CALL FOR CHALLENGE GRANTS WE THOUGHT WE MIGHT GET TWO OR 3,000. WE GOT 21,000. WE COULD ONLY AFFORD TO FUND ABOUT 3% OF THOSE. MAYBE A LITTLE LESS THAN 3%, WHICH MEANT THERE WAS SOME WONDERFUL PROPOSALS THAT WE WERE NOT ABLE TO SUPPORT. ONE OF THE CONCERNS IS, OKAY, WHAT'S GOING TO HAPPEN, ALL OF THESE IDEAS ARE OUT THERE, A LOT OF PEOPLE ARE GOING TO PROBABLY COME BACK WITH A REVISED VERSION, THEY'LL LOOK AT THE REVIEW AND PERHAPS MAKE AN EVEN STRONGER CASE. WHERE ARE WE GOING TO BE AS FAR AS THE ABILITY TO SUPPORT THOSE? MANY OF US WORRY QUITE A BIT ABOUT FY 11 AND WHAT HAPPENS AFTER THE RECOVERY ACT TWO YEAR OF SUPPORT COMES TO AN END. I'M SURE YOU ARE THINKING ABOUT THAT, TOO. I HOPE YOU ARE.

[QUESTIONER] MY NAME IS DANIEL PREVEDELLO. I'M A NEUROSURGEON AT THE UNIVERSITY OF PITTSBURGH. I SEE SOME PROFESSORS OVER THERE. I'M REPRESENTING THE PITUITARY NETWORK ASSOCIATION. AS WE KNOW, PITUITARY PROBLEMS OR NODULES CAN BE FOUND IN ONE OUT OF FIVE PEOPLE, SO IT'S VERY COMMON FINDING IN AFFECTING SYSTEMICALLY MANY PEOPLE AROUND THE WORLD. AS WE KNOW, THE NIH IS STRUCTURED IN A WAY THAT IS TOTALLY FOCUSED ON ORGANS AND DISEASE. I'M VERY GLAD TO HEAR THERE ARE A LOT OF WILLING TO CHANGE. MY QUESTION IS, IS THERE ANY PLAN TO CREATE SOMETHING IN A SYSTEMIC APPROACH FOR ORGANISMS AND PEOPLE WITH SOMETHING, FOR INSTANCE, WITH THE CREATION OF THE NATIONAL INSTITUTES OF HORMONAL HEALTH OR IN METABOLIC DISORDERS?

[DR. COLLINS] I APPRECIATE THE QUESTION. I THINK CONGRESS HAS BEEN INTERESTED IN THE STRUCTURE OF NIH AND PARTICULARLY IN THE QUESTION ABOUT HOW MANY INSTITUTES SHOULD THERE REALLY BE. I KNOW OVER THE COURSE OF HISTORY OFTENTIMES THE CREATION OF A NEW INSTITUTE CAME ABOUT BECAUSE OF PRESSURE THAT A PARTICULAR AREA OF DISEASE WAS NOT GETTING ENOUGH ATTENTION. BUT I CAN TELL YOU NOW AS THE NIH DIRECTOR WITH 27 DIRECT REPORTS FOR THESE INSTITUTES AND CENTERS, IT IS A PRETTY CHALLENGING STRUCTURE TO TRY TO MANAGE. AND THE CONGRESS HAS MADE THAT CONCLUSION. IN FACT, THE NIH RE-AUTHORIZATION ACT WHICH WAS PASSED A COUPLE OF YEARS AGO NOW PUTS A CEILING AND SAYS THERE WILL NOT BE MORE THAN 27. SO THE IDEA OF CREATING NEW INSTITUTES OR CENTERS AT THE PRESENT TIME IS OFF THE TABLE UNLESS THE CONGRESS SHOULD DECIDE TO CHANGE THEIR MIND. SO I'M NOT SURE THAT WOULD BE THE RIGHT SOLUTION. IN FACT, FROM WHERE I STAND, HAVING WATCHED NIH EVOLVE OVER THE LAST 16 YEARS SINCE I CAME HERE INITIALLY IN '93, THE CREATION OF NEW INSTITUTES CAN SOMETIMES HAVE NOT THE IDEAL OUTCOME BECAUSE IT MAY TAKE AN AREA OF SCIENCE THAT'S BEING INVESTED IN ACROSS SEVERAL

INSTITUTES AND PUT IT OFF ON THE SIDE AND THEN EVERYBODY ELSE SAYS THAT ONE IS TAKEN CARE OF, WE DON'T HAVE TO WORK ON IT ANY MORE. WHEN IT COMES TO HORMONAL HEALTH CERTAINLY NIDDK BECAUSE OF THEIR INTEREST IN ENDOCRINOLOGY IS AN OBVIOUS PLACE FOR SOME OF THIS TO GO ON, THE CHILD HEALTH INSTITUTE ALSO VERY INTERESTED IN HORMONAL HEALTH. PERHAPS THERE IS AN OPPORTUNITY TO THINK ABOUT WHETHER WE HAVE A PORTFOLIO THAT'S TOUCHING ALL THE PLACES THAT IT SHOULD. I GUESS ONE OF MY JOBS AS THE NIH DIRECTOR IS, IF AREAS LIKE THAT TURN UP, WE SHOULD PAY ATTENTION. I SHOULD TELL YOU, ONE OF THE OTHER THINGS WE HAVE AT NIH THAT I'M EXCITED ABOUT SEEING HOW IT PLAYS OUT IS AN ABILITY TO NOW FINALLY LOOK ACROSS OUR ENTIRE PORTFOLIO AND SAY WHERE ARE THE GAPS? WITH SUCH A HUGE COMPLICATED ORGANIZATION, IT'S NOT ALWAYS EASY TO KNOW THE ANSWER TO THAT QUESTION, BUT AS ONE DIVISION THAT'S NEWLY CREATED BY THE SAME RE-AUTHORIZATION ACT, WE NOW HAVE SOME ABILITIES TO LOOK AT THAT. I APPRECIATE YOUR QUESTION AND THAT MIGHT BE A PLACE TO HAVE A LOOK AT THE PORTFOLIO AND SAY WHAT ARE WE DOING IN HORMONAL HEALTH? IS THIS A PLACE WE COULD DO MORE? AND IF SO, WHO SHOULD BE CHARGED WITH DOING IT? THANK YOU.

[QUESTIONER] MY NAME IS ZACHARY R. LANGWAY, HERE FOR FAMILY HEALTH INTERNATIONAL. YOU HAVE TOUCHED ON OPENNESS AND NEW AND COMPELLING WAYS TO REACH OUT TO THE PUBLIC. YOU'LL BE HAPPY TO KNOW IF IN ADDITION TO BEING HERE WE'RE ALSO BROADCASTING SOME OF YOUR ANNOUNCEMENTS TO ABOUT 2500 PEOPLE ON TWITTER TODAY, BROADCASTING TO OUR FOLLOWERS ON TWITTER. I HAVE A QUESTION FROM ONE OF THEM I WOULD LIKE TO ASK YOU. AMONGST THIS MYRIAD OF GREAT OPPORTUNITIES, WHERE DO YOU SEE HIV/AIDS AND THE U.S. RESPONSE TO HIV/AIDS DOMESTICALLY AND GLOBALLY FOLLOWING AMONGST THOSE PRIORITIES?

[DR. COLLINS] IT'S OBVIOUSLY A CRITICAL PUBLIC HEALTH CONCERN HERE AND ACROSS THE WORLD, ONE THAT I DON'T THINK ANYBODY IN MEDICAL RESEARCH COULD LOOK AT AND NOT FEEL A GREAT URGENCY ABOUT COMING UP WITH SOLUTIONS, WHICH HAVE OBVIOUSLY BEEN EXTREMELY DIFFICULT TO ACHIEVE IN TERMS OF AN ULTIMATE ERADICATION OF THE DISEASE. AS YOU KNOW, VACCINE EFFORTS HAVE ENCOUNTERED REPEATED OBSTACLES BY THE DIABOLICAL NATURE OF THE VIRUS THAT SEEMS CAPABLE OF EVADING THE IMMUNE RESPONSES ONE HOPES TO BE PROTECTED. I WILL TELL YOU THAT, AS PART OF THE RECOVERY ACT, THE NATIONAL INSTITUTE OF ALLERGY AND INFECTIOUS DISEASE, TONY FAUCI, DIRECTOR, HAS PUT FORWARD SOME DRAMATIC NEW PROGRAMS TO TRY—EVEN IN THE ABSENCE OF A VACCINE—TO SEE IF THERE'S A WAY TO GREATLY REDUCE THE INCIDENCE OF THE DISEASE. THAT INCLUDES PROGRAMS TO CONTEMPLATE TREATING PEOPLE PRE-

EXPOSURE IN HIGH-RISK GROUPS. IT INCLUDES THE IDEA OF TREATING PEOPLE IMMEDIATELY AFTER THEY ARE DISCOVERED TO BE HIV POSITIVE, WITHOUT WAITING FOR THE CD-4 COUNTS TO FALL. AND SOME NEW MICROBICIDAL APPROACHES AS WELL TO SEE IF WE COULD COME UP WITH A THERAPY THAT ACTUALLY WOULD ELIMINATE THE VIRUS AND NOT REQUIRE LIFE-LONG THERAPY. THOSE ARE ALL IN A VERY AMBITIOUS ROUGHLY $400 MILLION NEW AGENDA THAT'S COMING FROM THE RECOVERY ACT. AND THE SCIENCE IS QUITE EXCITING. BUT ALL OF US, REALISTICALLY WE'RE AWARE BY NOW WE'RE UP AGAINST A DIFFICULT FOE IN THIS VIRUS AND NOTHING SEEMS TO COME EASY. I THINK YOU WILL NOT FIND A PERSON AT NIH WHO DOES NOT SEE THIS AS ONE OF OUR MOST IMPORTANT PRIORITIES AND WHO IS NOT EXCITED ABOUT SOME OF THE NEW SCIENTIFIC OPPORTUNITIES THAT I HAVE JUST MENTIONED.

[QUESTIONER] HELLO, DR. COLLINS. MY NAME IS DAVE FLOYD. I'M A THREE-TIME MELANOMA METASTATIC CANCER SURVIVOR. I'M REPRESENTING THE MELANOMA INTERNATIONAL FOUNDATION. AND I GUESS, AS YOU KNOW, MELANOMA IS—THERE'S 50,000 CASES PER YEAR. OF THOSE, 8,000 PEOPLE DIE FROM MELANOMA. IT'S A CANCER THAT CAN BE EASILY DETECTED AND PREVENTED, BUT IF IT'S NOT DETECTED EARLY, IT CAN BE VERY DEADLY. AND IT GROWS VERY QUICKLY. SO I GUESS MY QUESTION—MY POINT IS THERE ARE OTHER CANCERS THAT ARE ACTUALLY REDUCING IN NUMBERS AND IMPROVEMENT, BUT MELANOMA IS THE ONE CANCER THAT CONTINUES TO GROW, CONTINUES TO GET WORSE, AND CONTINUES—WITH THE OZONE LAYER SHRINKING, USE OF TANNING SALONS, THIS KIND OF THING—INCREASING THE INCIDENCE OF MELANOMA. AND I CAN SPEAK FROM PERSONAL EXPERIENCE, YOU DON'T WANT TO GET MELANOMA. SO I WANT—I GUESS MY POINT IS, WHAT IS BEING DONE ABOUT MELANOMA AND FUNDING FOR MELANOMA AT NIH?

[DR. COLLINS] CERTAINLY THE NATIONAL CANCER INSTITUTE, WHICH, AS YOU KNOW, IS THE MAJOR SUPPORTER OF MELANOMA RESEARCH SEES THIS AS A VERY HIGH PRIORITY. YOU'RE QUITE RIGHT. WHEN YOU LOOK AT THE CURVES OF INCIDENCE, WHERE SOME OF CANCERS ARE NOW STARTING TO GO DOWNWARD IN THEIR INCIDENCE, MELANOMA CONTINUES TO CLIMB FOR ALL THE REASONS YOU MENTIONED, MOST OF WHICH WE THINK RELATE TO UV EXPOSURE, PARTICULARLY IN EARLY LIFE. OBVIOUSLY FOR MANY PEOPLE WHO ARE ALIVE TODAY, THEY'VE ALREADY HAD THAT EXPOSURE SOMEWHERE BACK IN CHILDHOOD AND WE CAN'T GO BACK AND CHANGE THAT BUT WE OUGHT TO FOCUS AS HARD AS WE CAN HOW TO DETECT AND HOW TO TREAT THE DISEASE SHOULD IT COME ABOUT. I MENTIONED THE CANCER GENOME ATLAS AS A PROGRAM THAT IS TRYING TO COMPREHENSIVELY IDENTIFY ALL OF THE THINGS THAT MIGHT CAUSE A MELANOCYTE TO GO BAD AND START

TO GROW TO A TUMOR. THERE'S A DR. DANIELS IN BUILDING 50 WORKING WITH STEVE ROSENBERG, AND THEY HAVE WORKED THROUGH A SERIES OF MORE THAN A HUNDRED SUCH TUMORS AND MADE A COUPLE OF PRETTY INTERESTING DISCOVERIES ABOUT PATHWAYS THAT SEEM TO BE COMMON IN MELANOMA THAT WEREN'T KNOWN BEFORE, IN WHICH POINT YOU IN THE DIRECTION OF NEW POSSIBLE DRUG THERAPIES. SO THERE'S A LOT OF WORK GOING ON. COULD THERE BE MORE? YEAH, THERE PROBABLY COULD IF RESOURCES WERE MORE PLENTIFUL. BUT I THINK I CAN ASSURE YOU THAT MELANOMA IS ON EVERYBODY'S RADAR AS A CANCER THAT DESERVES A GREAT DEAL OF EFFORT RIGHT NOW.

THANK YOU.

[QUESTIONER] THANK YOU FOR PUTTING TOGETHER THIS FORUM. I'M LISA SHULMAN, PROFESSOR OF VIROLOGY AT UNIVERSITY OF MARYLAND, REPRESENTING THE AMERICAN ACADEMY OF NEUROLOGY. AND AS YOU KNOW FOR SOME TIME THERE'S BEEN A TREMENDOUS FOCUS ON EVIDENCE-BASED MEDICINE. MANY PROFESSIONAL ORGANIZATIONS, INCLUDING THE ONE I'M REPRESENTING, ARE DOING MASSIVE AMOUNTS OF WORK ON DEVELOPING EVIDENCE-BASED GUIDELINES. BUT VIRTUALLY ALL PAPERS THAT DESCRIBE EVIDENCE-BASED GUIDELINES FOR CLINICAL PRACTICE END WITH THE DESCRIPTION OF THE EVIDENCE THAT WAS MISSING IN ORDER TO PUT TOGETHER THE PAPER. THIS GROWING BODY OF WORK ON EVIDENCE-BASED GUIDELINES IS REALLY A ROADMAP TO MANY CLINICAL TRIALS THAT NEED TO BE PERFORMED. IN CLINICAL PRACTICE A LARGE AMOUNT OF DECISION MAKES, PERHAPS THE MAJORITY REQUIRES EVIDENCE THAT IS CURRENTLY NOT AVAILABLE. AND THAT'S NOT APPRECIATED GENERALLY. YET PROGRAMS LIKE PAY FOR PERFORMANCE ARE BASED ON THIS INADEQUATE EVIDENCE BASE. SO MY QUESTION IS, ARE YOU CONSIDERING PROGRAMS THAT RESPOND TO THIS NEED?

[DR. COLLINS] GREAT QUESTION. ABSOLUTELY, YES. I THINK YOU'RE POINTING OUT, WE REALLY HAVE TWO PROBLEMS. ONE IS WHAT WE CALL THE T-1 WHERE YOU HAVE TO DO THE CLINICAL RESEARCH TO FIGURE OUT WHAT WORKS AND TO KNOW WHETHER IT WORKS. THEN THERE'S THE T-2, YOU KNOW IT WORKS, DOES IT ACTUALLY GET IMPLEMENTED? HOW DO YOU GET THAT BODY OF CLINICAL RESEARCH WHERE YOU DO HAVE THE EVIDENCE ACTUALLY BROUGHT INTO PRACTICE IN A WAY THAT BENEFITS PATIENTS AND REDUCES BAD OUTCOMES. AND THAT IS

ALSO A BIG CHALLENGE. AS WE KNOW, SOMETIMES IT TAKES YEARS, SOMETIMES DECADES TO TAKE RESEARCH THAT YOU DO HAVE EVIDENCE FOR AND GET IT IMPLEMENTED. I CAN TELL YOU THAT EVERY NIH INSTITUTE THAT RUNS CLINICAL TRIALS IS CONSTANTLY SAMPLING THEIR TERRITORY, THEIR PARTICULAR DISORDER, THEIR PARTICULAR

COLLECTION OF DISORDERS AND SAYING WHAT IS THE MOST CRITICAL THING THAT WE DON'T KNOW THAT WE NEED TO KNOW? AND LET US MODIFY OUR PLANS TO BE SURE WE HAVE A CLINICAL TRIAL TO TRY TO ANSWER THAT QUESTION. CLINICAL TRIALS ARE OF COURSE TERRIBLY EXPENSIVE. MANY OF THE QUESTIONS YOU WANT TO ANSWER CAN'T BE DONE WITH A FEW DOZEN PATIENTS. THEY MAY TAKE HUNDREDS OR THOUSANDS IN MULTI-CENTER TRIALS. THAT'S ALWAYS A LIMITATION IN TERMS OF HOW MANY OF THESE CAN BE CONDUCTED AT ONE TIME WITH THE RESOURCES AVAILABLE.

BUT I THINK IF YOU TALKED TO ANY OF THE PEOPLE WHO ARE PARTICULARLY FOCUSED ON THIS AREA OF EVIDENCE BASE, THEY ARE VERY MUCH SCANNING THOSE SAME PAPERS TRYING TO FIGURE OUT WHAT COULD NIH DO TO FILL A PARTICULARLY CRITICAL GAP THAT WE NEED INFORMATION ON AND WE JUST DON'T HAVE IT. THE COMPARATIVE EFFECTIVENESS RESEARCH IS A COMPONENT OF THIS, BUT OF COURSE, TO DO COMPARATIVE EFFECTIVENESS YOU HAVE TO HAVE THINGS TO COMPARE. THAT MEANS YOU HAVE TO HAVE MORE THAN ONE ALTERNATIVE FOR AN INTERVENTION AND YOU'RE NOT SURE WHICH ONE WORKS BEST BY THE DEFINITION OF COMPARATIVE EFFECTIVENESS RESEARCH. SOMETIMES YOU DON'T HAVE ANYTHING. YOU'RE TRYING TO FIGURE OUT WHAT WOULD WORK IN THIS SPACE. I THINK THE WHOLE COMPARATIVE EFFECTIVENESS RESEARCH DIALOGUE THOUGH IS TURNING OUT TO RAISE PEOPLE'S ATTENTION TO THIS, AND THE INSTITUTE OF MEDICINE WITH THEIR SPECIFIC IDENTIFICATION OF NO LESS THAN 100 AREAS THAT THEY FELT NEEDED THAT KIND OF COMPARATIVE EFFECTIVENESS CLINICAL EFFORT HAS FOCUSED ATTENTION IN A SPECIFIC WAY ON GAPS THAT MIGHT OTHERWISE NOT HAVE BEEN IDENTIFIED. AND NIH IS PAYING A LOT OF ATTENTION TO THAT IN OUR DESIGN OF HOW WE'RE SPENDING THE MONEY WE WERE GIVEN TO DO COMPARATIVE EFFECTIVENESS RESEARCH. SO YOUR POINT IS WELL TAKEN.

I THINK WE'RE NOT PERFECT AT THIS, I'M SURE. IF YOU FIND THERE ARE AREAS THAT YOU THINK ARE PARTICULARLY IN NEED OF THAT KIND OF CLINICAL TRIAL INFORMATION, AGAIN, SEND US A NOTE TO NIH-LISTENS OR GO TO THE SPECIFIC INSTITUTE, NINDS FOR YOU, SEND THEM AN E-MAIL AND ASK WHY AREN'T YOU DOING THAT? WE'RE HERE TO SERVE.

[QUESTIONER] MY NAME IS TIM MCNIGHT FROM THE OAK RIDGE NATIONAL LABORATORY. UNDER YOUR LEADERSHIP THE HUMAN GENOME RESEARCH PROJECT SHOWED THE SYNERGY REALIZED WHEN THE NATIONAL INSTITUTES OF HEALTH AND DEPARTMENT OF ENERGY COMBINED RESOURCES TOWARDS A COMMON GOAL. DO YOU HAVE ANY PLANS COMING UP FOR HOW TO TAP INTO THOSE RESOURCES IN THE FUTURE SO THAT YOU CAN ACCESS THE LABORATORIES, THE NATIONAL

LABORATORY COMPLEX, AND SOME UNIQUE CAPABILITIES THEY HAVE, LIKE THE NEUTRON SOURCE FOR STRUCTURAL BIOLOGY, NANOPHASE MATERIALS FOR DEVELOPING NEW BIOMATERIALS, AND THE INCREDIBLE COMPUTATIONAL ARCHITECTURE THAT THEY HAVE, FOR INSTANCE FOR THE CTA ASSAY NETWORKS?

[DR. COLLINS] GREAT QUESTION. I THINK IN THIS ERA WHERE RESOURCES ARE NOT PERHAPS AS PLENTIFUL AS WE ALL HOPED THEY WERE AND WHERE OPPORTUNITIES OF INTERACTIONS BETWEEN SCIENTISTS WHO DON'T HAPPEN TO LIVE AT THE SAME ADDRESS ARE REALLY EXCITING, WE SHOULD LEAVE NO STONE UNTURNED IN BUILDING THOSE RELATIONSHIPS. YES. WORKING WITH THE DOE ON THE HUMAN GENOME PROJECT WAS AN EXAMPLE OF HOW YOU COULD BRING THOSE TALENTS TOGETHER TO GREAT ADVANTAGE AND EVEN AFTER THAT I SERVED FOR A WHILE AS NIH LIAISON TO THE DEPARTMENT OF ENERGY AND GOT INVOLVED IN SOME OF THE THINGS THAT YOU HAVE MENTIONED. I THINK, AND ESPECIALLY NOW THAT YOU HAVE AS YOUR SECRETARY OF ENERGY FOR HEAVEN SAKE A NOBEL LAUREATE PHYSICIST, ALL THE MORE REASON TO EXPLORE SCIENTIFIC OPPORTUNITIES. STEVEN SHU WILL BE A GREAT ASSET IN THAT SPACE. SO BOTH IN TERMS OF LOOKING OUTSIDE OF THE DEPARTMENT OF HHS AND WITHIN HHS AT FDA AND CDC AND CMS, I CAN TELL YOU AS NIH DIRECTOR, THIS IS GOING TO BE ONE OF MY PRIORITIES, TRY TO BE SURE WE'RE NOT MISSING OUT ON COLLABORATIVE OPPORTUNITIES THAT COULD MOVE THINGS ALONG FASTER.

I KNOW WE MAY NOT BE ABLE TO GET TO ALL THE QUESTIONS. ESPECIALLY IF I TALK SO LONG.

BUT I'M HAPPY TO KEEP GOING.

[QUESTIONER] THANK YOU, DR. COLLINS. CAROL GREEN REPRESENTING THE SOCIETY FOR INHERITED METABOLIC DISORDERS WITH A QUESTION NOT ABOUT AN INDIVIDUAL DISORDER BUT A QUESTION RELATED TO THE EARLIER QUESTION ABOUT THE GAPS BUT MORE BASIC BEFORE WE CAN EVEN GET TO IMPROVED THEORIES OF DISEASE AND HYPOTHESES AND BEFORE WE CAN GET TO CLINICAL TRIALS WITH AN ANALOGY TO DARWIN'S ORIGINAL VOYAGE OF DISCOVERY AND THE USE OF THINGS THAT ARE DESCRIPTIVE USING THE CURRENT MORE POWERFUL TOOLS AND PERHAPS MORE MODERN ANALOGY TO THE WHOLE HUMAN GENOME APPROACH THAT YOU WERE THINKING ABOUT FOR CANCER, IN THIS TIME OF LIMITED RESOURCES, WHAT PLACE DO YOU SEE FOR NATURAL HISTORY AND LONG-TERM OUTCOME STUDIES ESPECIALLY FOR RARE DISORDERS THAT CAN ILLUMINATE THE MORE COMMON COMPLEX DISORDERS, ESPECIALLY WHERE COLLABORATIVE STUDIES ARE NEEDED THAT MODEL THE PKU STUDY THAT WAS ANOTHER EXAMPLE OF A

TREATMENT AND LIFE-CHANGING OUTCOMES STUDY, WHAT PLACE WOULD YOU SEE AND HOW WOULD YOU POSITION THAT?

[DR. COLLINS] RARE DISEASES, I THINK, ARE A PARTICULARLY IMPORTANT OPPORTUNITY, AND WHILE THEY MAY BE RARE INDIVIDUALLY, AS YOU KNOW, CAROL, GOODNESS, 21 MILLION PEOPLE IN THIS COUNTRY ARE AFFLICTED BY A RARE DISEASE. IF YOU ADD UP THEIR FAMILY MEMBERS, THAT BECOMES A VERY LARGE NUMBER INDEED. FOR THOSE CONDITIONS FOR WHICH WE DON'T EVEN YET HAVE AN ANSWER, THERE ARE I THINK BECAUSE OF THE ADVANCES IN TECHNOLOGY, CHANCES TO PUSH THAT AGENDA.

ONE PARTICULARLY EXCITING ONE HERE IN THE CLINICAL RESEARCH CENTER IS THIS UNDIAGNOSED DISEASES PROGRAM THAT BILL GAHL IS RUNNING TO BRING PEOPLE HERE TO OUR CLINICAL CENTER WHO HAVE PUZZLING CONDITIONS THAT HAVE FAILED TO BE FIGURED OUT BY A LONG LIST OF OTHER EXPERTS. AND THAT WHICH HAS BEEN RECENTLY WRITTEN UP IN THE NEW YORK TIMES SUNDAY MAGAZINE IS REALLY QUITE AN INTERESTING AND INSPIRING WAY TO UTILIZE A BIT OF THE NIH'S CLINICAL RESOURCES. FOR CONDITIONS WHERE YOU DO HAVE PERHAPS A PRETTY GOOD IDEA OF THE CAUSE BUT YOU'RE NOT YET SURE HOW TO INTERVENE, THE IDEA THAT I TRIED TO PUT FORWARD OF PARTNERSHIPS BETWEEN ACADEMICS AND THE PRIVATE SECTOR TO DEVELOP NEW THERAPEUTIC IDEAS IS, I THINK, RIPE FOR INVESTIGATION AND THAT'S WHAT THIS PROGRAM CALLED "TREND" AIMS TO MAKE POSSIBLE. WE HOPE TO SEE THAT EXPANDED. BUT OF COURSE IF YOU'RE LUCKY ENOUGH, OR YOU WORK HARD ENOUGH IS A BETTER WAY TO SAY IT, TO FIND SOME KIND OF THERAPEUTIC OPPORTUNITY FOR A RARE DISEASE, THE NEXT QUESTION IS, OKAY, WHO IS AVAILABLE TO ENROLL IN A TRIAL?

THAT'S WHERE I WANT TO COME TO YOUR MAIN QUESTION BECAUSE THIS IS THE TIME, SEEMS TO ME FOR RARE DISEASES TO TRY TO IDENTIFY COHORTS OF INDIVIDUALS TO BE SURE THAT THEY HAVE BEEN WELL CHARACTERIZED, THAT THE NATURAL HISTORY IS KNOWN, THAT SOME CLINICAL END POINT IS IDENTIFIED SO THAT IF YOU START A CLINICAL TRIAL YOU'LL KNOW WHETHER IT'S WORKING AS QUICKLY AS POSSIBLE WITH A RELIABLE BIOMARKER OF SOME SORT. THOSE THINGS OFTEN DON'T GET THOUGHT ABOUT UNTIL LATER IN THE PROCESS. SO FOR ANY RARE DISEASE WHO'S REPRESENTED HERE, THIS WOULD BE A GREAT MOMENT TO THINK ABOUT WHAT WOULD YOU DO RIGHT NOW IF SOMEBODY CALLED YOU UP AND SAID "I HAVE GOT A THERAPEUTIC STRATEGY THAT THE FDA HAS JUST APPROVED FOR A PHASE 1 OR PHASE 2 TRIAL. WHERE ARE YOUR PATIENTS? ARE THEY READY?" WE CAN GET THEM READY. I THINK, AND AGAIN I THINK NIH WOULD LIKE VERY MUCH TO BE PARTNERS. BUT THE PLACES WHERE THE PATIENTS ARE, ARE

OFTEN IN THE PATIENT-SUPPORT ORGANIZATIONS. AND SOME OF THEM, I THINK, HAVE GOTTEN REVVED UP AND ARE VERY MUCH INTO THIS; OTHERS, I THINK, ARE PERHAPS WAITING TO HEAR THE WORD THAT THE TIME IS NOW. WELL, THE TIME IS NOW. THANKS, CAROL.

[QUESTIONER] I THINK YOUR APPOINTMENT WAS MET WITH GREAT ACCLAIM THROUGHOUT THE SCIENTIFIC COMMUNITY. MY NAME IS SARAH YING, JUNIOR FACULTY AT JOHNS HOPKINS UNIVERSITY DEPARTMENT OF NEUROLOGY, REPRESENTING THE STUDENT AMBASSADOR ASSOCIATION. AS YOU KNOW, ATAXIA IS A RARE DISEASE, SO I PARTICULARLY APPRECIATED THE PRECEDING QUESTION, WHICH, ALTHOUGH WE'RE CONCERNED BECAUSE IT HAS A PROFOUND EFFECT ON AFFECTED INDIVIDUALS, IT'S ALSO A VERY IMPORTANT MODEL OF MOTOR LEARNING AND THUS I THINK COULD BE AN IMPORTANT MODEL FOR OTHER DISEASES AS WELL. MY QUESTION TO YOU FOLLOWS UP ON THINGS THAT YOU HAVE TOUCHED UPON. BACK HERE IN ATAXIA ROW, WE'RE READY. WE HAVE THE PATIENTS READY, WE'RE RUNNING THE NATURAL HISTORY STUDIES. WE'RE DEVELOPING THE BIOMARKERS AND WE HAVE RESEARCH GROUPS TAPPING INTO THIS DRUG PIPELINE THAT HAS BEEN ACCELERATED AND SET UP BY NIH. MY QUESTION TO YOU IS HOW CAN NIH TOGETHER WITH THE PATIENT GROUPS AND INDIVIDUAL RESEARCHERS PARTNER TOGETHER IN THIS COUNTRY AND AROUND THE WORLD TO DEVELOP A COMMON ROADMAP FOR EACH INDIVIDUAL DISEASE-ORIENTED AND/OR PROCESS-ORIENTED APPROACH IN ORDER TO COORDINATE OUR EFFORTS?

[DR. COLLINS] YOU'RE RIGHT THAT WE WOULDN'T WANT TO HAVE TO DO THIS AS A SERIES OF ONE-OFFS. THE IDEA OF BEING ABLE TO TACKLE MANY CONDITIONS WITH A SHARED APPROACH HAS GOT TO BE THE RIGHT ANSWER. SO THE VERY IDEA OF THIS THERAPEUTICS FOR RARE AND NEGLECTED DISEASE PROGRAM IS TO TRY TO PROVIDE THAT AS A UNIFIED COORDINATED PIPELINE FOR THERAPEUTIC DEVELOPMENT FOR DISEASES LIKE CEREBELLAR ATAXIA. THE CHALLENGE IS TO GET THE RESEARCHERS INTERESTED IN THE PROBLEM. THAT'S ALWAYS BEEN, I THINK, FOR MANY GROUPS A FRUSTRATION, THAT YOU'VE KIND OF GOT TO THE POINT WHERE IT LOOKED LIKE THERE MIGHT BE A POSSIBILITY, BUT WHO ARE YOUR CHAMPIONS WHO ARE GOING TO BE OUT THERE PUSHING THE AGENDA SCIENTIFICALLY? MAYBE THAT'S YOU, SINCE YOU'RE THE ASSISTANT PROFESSOR AT HOPKINS, YOU'RE GOING LEAD THE CHAMPION CHARGE, BUT SOME OTHER ORGANIZATIONS ARE STILL LOOKING. ONE THING THAT'S VERY SUCCESSFUL, I THINK, IS TO TRY TO CONVENE A WORKSHOP AND TALK TO THE PEOPLE AT NIH ABOUT WHO MIGHT BE INTERESTED AND MAYBE NEVER HEARD OF THE DISEASE YOU'RE WORKING ON, BUT IF THEY COULD SEE THE RESEARCH OPPORTUNITIES THEY MIGHT CONSIDER TURNING THEIR OWN RESEARCH DIRECTION THAT WAY. THAT HAS CERTAINLY WORKED. I MEAN, MY OWN

LAB, WHICH IS STILL ACTIVE OVER HERE ON THE INTRAMURAL CAMPUS, HAS WORKED PRIMARILY ON RARE DISEASES. MOST OF THE TIME THEY WERE DISEASES THAT I HAD SOME FAMILIARITY WITH, BUT A SCIENTIFIC OPPORTUNITY EMERGED. I THOUGHT, I COULD PUT A POST-DOC ON THAT. THAT'S HOW THINGS GET OFTEN DISCOVERED IN WAYS THAT WOULD HAVE TAKEN MUCH LONGER. SO TAKING THE ADVANTAGE TO DO THAT KIND OF CONVENING OF EXPERTISE, CLINICAL AND BASIC SCIENCE, TO START THE BALL ROLLING. THEN BE AWARE ON THE RESOURCES BEING DEVELOPED THROUGH THE ROADMAP AND OTHER MEANS THAT YOU CAN TAP INTO THAT ARE ALREADY PAID FOR, THAT WILL ALLOW YOU TO DO THE SAME HIGH-THROUGHPUT SCREENING OF A LIBRARY OF CHEMICAL COMPOUNDS THAT A PHARMACEUTICAL COMPANY CAN DO UP HERE IN GAITHERSBURG HAS THAT SCREENINGS CAPACITY. IT'S A BIT UNDERUTILIZED, IN THE VIEW OF MANY OF US IN TERMS OF THE APPLICATION TO RARE DISEASES. FIND OUT ABOUT THOSE RESOURCES. GO TO THE WEBSITE FOR THE ROADMAP; IT WILL TELL YOU MORE ABOUT THAT. GET PEOPLE FIRED UP AND I THINK YOU'RE IN A GOOD POSITION TO REALLY CHANGE THE WHOLE LANDSCAPE OF THIS DISEASE. THIS IS THE TIME TO DO IT.

[QUESTIONER] I'M SALLY SMITH, THE IMMEDIATE PAST CHAIR OF THE INDIAN HEALTH BOARD. I REMAIN AS REPRESENTATIVE FROM ALASKA. I'M ACCOMPANIED BY STACY BOHLEN, WHO IS HEADQUARTERED HERE IN WASHINGTON, D.C., BUT I WANT TO BEGIN BY SAYING, DR. COLLINS, SOME THREE AND A HALF YEARS AGO I CHAIRED THE HEALTH RESEARCH REVIEW COMMITTEE IN ANCHORAGE, ALASKA. WE INVITED YOU TO BE THE KEYNOTE SPEAKER AND YOU SPOKE ON YOUR HUMAN GENOME.

[DR. COLLINS] I LOVED BEING THERE. THAT WAS A WONDERFUL VISIT.

[QUESTIONER] OH, PLEASE COME BACK. I REPRESENT THE NATIVE VILLAGE OF IGIUGIG, ALASKA. DR. COLLINS, WHEN YOU CAME TO ALASKA YOU CAME AT A CRITICAL TIME BECAUSE RESEARCH, AS YOU KNOW, IN NATIVE COMMUNITIES IS LOOKED ON IN VARIOUS WAYS.

[DR. COLLINS] YES.

[QUESTIONER] OUR FIRST CONFERENCE TALKED ABOUT YESTERDAY, TODAY, AND TOMORROW. WE WERE STRATEGIC IN BRINGING YOU TO THE SECOND CONFERENCE BECAUSE YOU VAULTED US INTO GENETICS. GENOMES. AND NOW WE ARE MORE PREPARED THAN EVER TO TACKLE AND BECOME PARTNERS WITH THE NATIONAL INSTITUTES OF HEALTH, NOT ONLY FROM OUR NATIVE COMMUNITIES IN ALASKA BUT NATIONALLY. SO AGAIN, PLEASE COME BACK AND VISIT US. OUR EXECUTIVE DIRECTOR HAS A QUESTION TO POSE TO YOU.

[DR. COLLINS] ALL RIGHT, THANK YOU, SALLY. YES.

[QUESTIONER] I'M STACY BOHLEN, A MEMBER OF THE CHIPPEWA TRIBE IN THE UPPER PENINSULA OF MICHIGAN. STANDING HERE TODAY WE ARE THE 562 FEDERALLY RECOGNIZED TRIBES OF UNITED STATES AMERICAN INDIANS AND ALASKA NATIVES. I'M INTERESTED ON BEHALF OF ALL THE TRIBES IN THE COMMENTS YOU MADE. ONE THAT I THINK WAS THE MOST INTERESTING WAS THE NEED TO LOOK BEYOND AND HAVE A MORE SYNTHESIZED APPROACH TO SCIENCE THAT ALSO CAPTURES SOCIAL AND BEHAVIORAL, SOCIOLOGICAL AND BEHAVIORAL COMPONENTS OF WELLNESS. I WOULD ALSO CHALLENGE YOU TO INCLUDE CULTURAL. BECAUSE WHEN IT COMES TO OUR PEOPLE, THE NUMBER ONE DISEASE WE SUFFER FROM IS ANONYMITY. WE ARE MORE DIFFICULT TO FIND IN THE SCIENTIFIC COMMUNITY THAN WAS REPRESENTED ON THE TOP OF YOUR GRAPHIC. BUT WE ARE HERE. WE ARE 4.5 MILLION AMERICANS WHO HAVE THE WORST HEALTH DISPARITIES IN THE UNITED STATES, THE WORST HEALTH, IN SOME CASES, IN THE ENTIRE WORLD. THE SPEED WITH WHICH CERTAIN OF OUR SEGMENTS ARE ACQUIRING HIV AND AIDS, THE ASTOUNDING CANCER RATES IN ALASKA NATIVES, ALASKA NATIVE VILLAGES, TUBERCULOSIS RATES AND DIABETES RATES AMONG OTHERS. TWO THINGS I WANTED TO POINT OUT ON BEHALF OF THE TRIBES TODAY IS THERE ARE TWO AREAS WITH AMERICAN INDIAN ALASKA NATIVE COMMUNITIES AND THE NATIONAL INSTITUTES OF HEALTH THAT ARE WORKING VERY WELL. ONE IS THE SPECIAL DIABETES PROGRAM FOR INDIANS, WHICH—I'M GOING TO TAKE A CUE FROM THE GENTLEMAN EARLIER—IS GOING TO LOSE ITS FUNDING IN 2010 AT THE END OF SEPTEMBER. THE HARD SCIENCE COMPONENT OF THAT PROGRAM WORKS BECAUSE OF THE NATIONAL INSTITUTES OF HEALTH PARTNERSHIP. AND THE COMMUNITY-BASED DISEASE PREVENTION HEALTH PROMOTION DATA COLLECTION ON THE GROUND WITH US WORKS BECAUSE OF THE TRIBES. IT'S CRITICAL THAT THIS PROGRAM CONTINUES. IT WORKS AND IT WORKS FOR BOTH OF OUR COMMUNITIES. AND IT IS SAVING LIVES OF OUR PEOPLE. THE OTHER PROJECT IS THE DIABETES EDUCATION AND TRIBAL SCHOOLS PROJECT, WHICH IS DRS. AGADOA AND SANDY GARFIELD HERE AT NIH, WHO PARTNERED WITH THE TRIBAL COLLEGES AND UNIVERSITIES. I HAD THE PRIVILEGE OF WORKING ON THAT PROJECT AS WELL. THE DIABETES PREVENTION CURRICULUM DEVELOPED WITH THE TRIBES AND THE NATIONAL INSTITUTES OF HEALTH IS WORKING. AND IT'S—WE WANT TO THANK YOU FOR THE 20-YEAR COMMITMENT THAT NIH MADE TO THAT PROGRAM. AND FINALLY, IN TERMS OF WHAT I MENTIONED, BUILDING ON YOUR BEHAVIORAL SOCIOLOGICAL ASPECT, WE NEED TO SEE AMERICAN INDIAN AND ALASKA NATIVE SCIENTISTS AT NIH.

[DR. COLLINS] I AGREE WITH YOU.

[QUESTIONER] WE STAND READY TO WORK WITH YOU. WE HAVE BRILLIANT PEOPLE IN OUR COMMUNITIES. THEY'RE JUST—THEY NEED TO HAVE ENCOURAGEMENT, THEY NEED OPPORTUNITY. AND WE'RE HERE TO STAND WITH YOU TO SEE THAT THAT HAPPENS AND ASK FOR YOUR COMMITMENT TO THAT.

[DR. COLLINS] YOU HAVE MY COMMITMENT TO ABSOLUTELY SEE THAT OPPORTUNITIES ARE PROVIDED FOR THE TALENT IN YOUR COMMUNITY, AND WE WORKED A LITTLE BIT TOGETHER IN THAT IN THE PAST WHEN I WAS AT THE GENOME INSTITUTE, AND I CERTAINLY WANT TO SEE THAT EXPANDED. AGAIN, THE MEDICAL RESEARCH COMMUNITY, IF IT DOESN'T REPRESENT THE POPULATION IN SOME REASONABLE WAY IS GOING TO MISS OUT ON OPPORTUNITIES AND INSIGHTS. SO OUR PAUCITY OF PARTICIPATION FROM GROUPS LIKE AMERICAN INDIANS DOES HARM TO THE WHOLE COMMUNITY. ANY IDEAS YOU HAVE ABOUT HOW WE COULD BE MORE EFFECTIVE IN RECRUITING YOUNG PEOPLE TO SEE THEMSELVES AS REAL CAREER SCIENTISTS, I WOULD LOVE TO HEAR THOSE THOUGHTS.

[QUESTIONER] I'LL BE BACK TOMORROW.

[DR. COLLINS] OKAY.

[LAUGHTER]

[QUESTIONER] MY NAME IS KEITH CRANK WITH THE AMERICAN STATISTICAL ASSOCIATION. I WANT TO RECOGNIZE THAT NIH IS A LEADER IN RECOGNIZING THE NEED FOR STATISTICS AND BIOSTATISTICS IN RESEARCH. BUT I ALSO WANT TO COMPLAIN ABOUT THE LACK OF SUPPORT FOR PROGRAMS THAT DEVELOP NEW BIOSTATISTICIANS, GRADUATE PROGRAMS THAT NEED THE SUPPORT OF NIH BECAUSE THERE IS NO OTHER PLACE TO GET IT. AND WOULD ENCOURAGE MORE OPPORTUNITIES FOR BIOSTATISTICIANS FOR DEVELOPMENT OF PROGRAMS FOR GRADUATE STUDENTS, BUT ALSO FOR BIOSTATISTICIANS TO HAVE TIME TO MENTOR NEW FACULTY AND NEW RESEARCHERS IN BIOSTATISTICS AND THE SUPPORT.

[DR. COLLINS] I APPRECIATE YOUR COMMENTS. CERTAINLY FROM MY OWN FIELD OF GENETICS, THE PEOPLE THAT WE VALUE THE MOST ARE THE STATISTICAL GENETICISTS, THEY'RE IN SHORT SUPPLY. IT'S ONE OF THOSE FIELDS WHERE NOBODY HAS TO DO A POST-DOC BECAUSE YOU CAN GET A JOB STRAIGHT OUT OF YOUR PH.D. AND BE FOUGHT OVER BY FOUR OR FIVE INSTITUTIONS, SO CLEARLY WE NEED TO BEEF UP THIS PIPELINE.

[QUESTIONER] THAT'S RIGHT. WE HAVE SEEN INCREASES EVERY YEAR AT THE MASTER'S LEVEL, AT THE PH.D. LEVEL. THIS YEAR I POLLED

BIOSTATISTICS DEPARTMENTS TO FIND OUT HOW MANY APPLICATIONS THEY HAD FOR THEIR GRADUATE PROGRAMS. THEY HAD AN INCREASE OVER LAST YEAR BUT DID NOT ACCEPT MORE PRESUMABLY BECAUSE THEY COULDN'T SUPPORT THEM.

[DR. COLLINS] OUR TRAINING PROGRAMS ARE OF COURSE ONE OF OUR MOST CRITICAL ENTERPRISES. YET BECAUSE OF FINANCIAL CONSTRAINTS WE HAVE NOT BEEN ABLE TO SUPPORT AS MANY TRAINING PROGRAMS EITHER PROGRAMS THAT TAKE MANY STUDENTS OR INDIVIDUALS, I AGREE WITH YOU, THERE ARE MANY AREAS OF NEEDS, STATISTICS IS RIGHT NEAR THE TOP OF THAT LIST. MANY OF THE QUESTIONS THAT ARE BEING POSED OF COURSE RELATE TO HOW DOES NIH BALANCE RESOURCES WHEN RESOURCES ARE LIMITING? TO THE EXTENT THAT YOU CAN HELP WAS THAT, I'M NOT BUSTING IN ANY CASE ANYBODY THOUGHT I WAS. THAT WOULD BE APPRECIATED. WE SHOULD PROBABLY TAKE ONE MORE QUESTION, THEN WE SHOULD DRAW THIS TO A CLOSE. YOU GET THE LAST ONE; I'M SORRY TO THE REST OF YOU. YES.

[QUESTIONER] HI, MY NAME IS PADMA NATARAJAN, HERE TO REPRESENT THE INFECTIOUS DISEASE SOCIETY OF AMERICA. AND WE'RE A PROFESSIONAL MEDICAL SOCIETY THAT REPRESENTS INFECTIOUS DISEASE PHYSICIANS AND PHYSICIAN-SCIENTISTS. ONE OF THE PRIMARY ISSUES THAT OUR PHYSICIAN-SCIENTISTS HAVE COME ACROSS IS THE REGULATORY BURDENS THAT THEY FACE IN CLINICAL RESEARCH, PRIMARILY DUE TO THE INCLUSION OF RESEARCH INTO HIPAA PRIVACY RULE. AND WHICH—BUT IT WAS NEVER INTENDED TO BE THAT WAY. WHAT I WANT TO KNOW IS, AT NIH YOU'RE ALL SCIENTISTS. I WANT TO KNOW IF THIS HAS BECOME A PROBLEM WITH NIH RESEARCHERS AND IF NIH AS A WHOLE HAS SEEN IT AS AN ISSUE? THE INSTITUTE OF MEDICINE PUT OUT A REPORT IN MAY SAYING THAT RESEARCH SHOULD BE REMOVED FROM HIPAA, I WANT TO KNOW -- AND AS WE BEGIN OUR ADVOCACY EFFORTS IN TRYING TO REMOVE IT, HAS NIH FOUND THIS AN ISSUE AS WELL?

[DR. COLLINS] WE CERTAINLY AGREE THAT THERE ARE ALL KINDS OF CHALLENGES AND BARRIERS FOR CLINICAL INVESTIGATORS. HIPAA IS ONE. THERE ARE OTHERS OF COURSE THAT RELATE TO THE AMOUNT OF EFFORT ONE OFTEN HAS TO GO THROUGH TO GET IRB APPROVAL FOR A CLINICAL PROTOCOL WITH MANY ITERATIONS REQUIRED. I THINK ALL OF US WHO WATCH THAT SCENARIO ARE TROUBLED THAT WE HAVE A PROCESS IN PLACE THAT HAD GOOD INTENTIONS IN TERMS OF PROTECTING PRIVACY AND MAKING SURE THAT INFORMED CONSENT WAS ALWAYS PRESENT WHEN SOMEBODY WAS ENGAGED IN RESEARCH. BUT THE PROCESS SEEMS TO HAVE BUILT INTO THIS MOUNTAIN OF PAPERWORK AND STEPS AND TIME-CONSUMING MEETINGS OF BUSY PEOPLE, AND IT'S NOT ALWAYS CLEAR THAT THE PATIENTS ARE

NECESSARILY HAVING THE PROTECTION THAT WAS INTENDED. SO I AGREE. THIS IS A WRENCHINGLY DIFFICULT PROBLEM IN TERMS OF HOW TO HIT THAT BALANCE. BUT I CAN ASSURE YOU NIH IS PAYING A LOT OF ATTENTION TO THE FACT THAT THIS IS ONE OF THOSE BARRIERS THAT'S SLOWING US DOWN. IF THERE ARE WAYS WE CAN TRY TO STRIP AWAY SOME OF THOSE OBSTACLES WHILE STILL PROTECTING CONFIDENTIALITY, WHILE PRESERVING THE CARDINAL PRINCIPLES OF THE BELMONT REPORT AND INFORMED CONSENT WE SHOULD DO EVERYTHING WE CAN TO ACHIEVE THAT. I GET A SENSE THAT IN THE NEW DEPARTMENT THAT IS ASSEMBLING DOWNTOWN, THERE'S A LOT OF INTEREST, NOT JUST AT NIH BUT A HIGHER LEVEL, IN THE ASSISTANT SECRETARY FOR HEALTH, IN LOOKING INTO THIS ISSUE AND SEEING IF THERE ARE THINGS WE COULD DO TO TRY TO IMPROVE THE ABILITY OF CLINICAL RESEARCHERS TO BE ABLE TO DO CLINICAL RESEARCH. AND TO FOCUS ON WHAT THEY WANT TO DO, WHICH IS TO COME UP WITH NEW INSIGHTS INSTEAD OF DOING THIS MIND-NUMBING DEGREE OF PAPERWORK THAT SEEMS TO OCCUPY MANY OF THEIR HOURS.

THANK YOU.

[MODERATOR] DR. COLLINS, IF YOU WOULD LIKE TO MAKE A COMMENT OR TWO IN CLOSING.

[DR. COLLINS] JUST TO THANK YOU, BECAUSE I KEPT YOU LONGER THAN PROMISED BUT THE QUESTIONS WERE REALLY INTERESTING AND I APPRECIATE THE DIVERSITY OF PERSPECTIVES THAT YOU ALL BROUGHT TO THIS. I WANT TO CONTINUE THIS CONVERSATION. I WANT OUR COMMUNICATIONS WITH ALL OF YOU TO BE AS OPEN AS POSSIBLE. I HOPE YOU WILL SEND FURTHER IDEAS TO NIH-LISTENS@NIH.GOV. WE WILL LOOK AT THEM AND SEE IF THERE ARE THINGS THERE THAT NEED ATTENTION. AND I HOPE TO CONTINUE THE CHANCE TO HAVE MEETINGS OF THIS SORT ON A REGULAR BASIS SO THAT I CAN GET FEEDBACK FROM ALL OF YOU. I AM HERE BY THE GOOD GRACES OF A SYSTEM THAT SEEMED TO THINK I COULD DO THIS JOB, BUT I KNOW I SURE CAN'T DO IT ALL BY MYSELF. I NEED THE HELP OF THE SCIENTIFIC COMMUNITY. I NEED THE HELP OF THE CONGRESS AND THE ADMINISTRATION, AND I NEED YOUR HELP. THANK YOU ALL VERY MUCH.

[APPLAUSE]

# # #