

S. HRG. 114-606

**CONTINUING AMERICA'S LEADERSHIP: REALIZING
THE PROMISE OF PRECISION MEDICINE FOR
PATIENTS**

HEARING
OF THE
**COMMITTEE ON HEALTH, EDUCATION,
LABOR, AND PENSIONS**
UNITED STATES SENATE
ONE HUNDRED FOURTEENTH CONGRESS
FIRST SESSION
ON
EXAMINING PRECISION MEDICINE FOR PATIENTS

MAY 5, 2015

Printed for the use of the Committee on Health, Education, Labor, and Pensions



Available via the World Wide Web: <http://www.gpo.gov/fdsys/>

U.S. GOVERNMENT PUBLISHING OFFICE

94-601 PDF

WASHINGTON : 2017

For sale by the Superintendent of Documents, U.S. Government Publishing Office
Internet: bookstore.gpo.gov Phone: toll free (866) 512-1800; DC area (202) 512-1800
Fax: (202) 512-2104 Mail: Stop IDCC, Washington, DC 20402-0001

COMMITTEE ON HEALTH, EDUCATION, LABOR, AND PENSIONS

LAMAR ALEXANDER, Tennessee, *Chairman*

MICHAEL B. ENZI, Wyoming
RICHARD BURR, North Carolina
JOHNNY ISAKSON, Georgia
RAND PAUL, Kentucky
SUSAN COLLINS, Maine
LISA MURKOWSKI, Alaska
MARK KIRK, Illinois
TIM SCOTT, South Carolina
ORRIN G. HATCH, Utah
PAT ROBERTS, Kansas
BILL CASSIDY, M.D., Louisiana

PATTY MURRAY, Washington
BARBARA A. MIKULSKI, Maryland
BERNARD SANDERS (I), Vermont
ROBERT P. CASEY, JR., Pennsylvania
AL FRANKEN, Minnesota
MICHAEL F. BENNET, Colorado
SHELDON WHITEHOUSE, Rhode Island
TAMMY BALDWIN, Wisconsin
CHRISTOPHER S. MURPHY, Connecticut
ELIZABETH WARREN, Massachusetts

DAVID P. CLEARY, *Republican Staff Director*
EVAN SCHATZ, *Minority Staff Director*
JOHN RIGHTER, *Minority Deputy Staff Director*

C O N T E N T S

STATEMENTS

TUESDAY, MAY 5, 2015

Page

COMMITTEE MEMBERS

Alexander, Hon. Lamar, Chairman, Committee on Health, Education, Labor, and Pensions, opening statement	1
Murray, Hon. Patty, a U.S. Senator from the State of Washington	3
Hatch, Hon. Orrin G., a U.S. Senator from the State of Utah	22
Franken, Hon. Al, a U.S. Senator from the State of Minnesota	23
Cassidy, Hon. Bill, a U.S. Senator from the State of Louisiana	25
Bennet, Hon. Michael F., a U.S. Senator from the State of Colorado	27
Collins, Hon. Susan M., a U.S. Senator from the State of Maine	29
Warren, Hon. Elizabeth, a U.S. Senator from the State of Massachusetts	30
Whitehouse, Hon. Sheldon, a U.S. Senator from the State of Rhode Island	32
Baldwin, Hon. Tammy, a U.S. Senator from the State of Wisconsin	34

WITNESSES

Collins, Francis S., M.D., Ph.D., Director, National Institutes of Health, Be- thesda, MD	6
Prepared statement	8
DeSalvo, Karen B., M.D., MPH, MSc, National Coordinator for Health Infor- mation Technology, Washington, DC	10
Prepared statement	12
Shuren, Jeffrey, M.D., J.D., Director, Center for Devices and Radiological Health, Food and Drug Administration, Silver Spring, MD	13
Prepared statement	15

ADDITIONAL MATERIAL

Statements, articles, publications, letters, etc.:	
Senator Klobuchar	40

CONTINUING AMERICA'S LEADERSHIP: REALIZING THE PROMISE OF PRECISION MEDICINE FOR PATIENTS

TUESDAY, MAY 5, 2015

U.S. SENATE,
COMMITTEE ON HEALTH, EDUCATION, LABOR, AND PENSIONS,
Washington, DC.

The committee met, pursuant to notice, at 2:41 p.m., in room SD-430, Dirksen Senate Office Building, Hon. Lamar Alexander, chairman of the committee, presiding.

Present: Senators Alexander, Collins, Hatch, Cassidy, Murray, Casey, Franken, Bennet, Whitehouse, Baldwin, Murphy, and Warren.

OPENING STATEMENT OF SENATOR ALEXANDER

The CHAIRMAN. The Senate Committee on Health, Education, Labor, and Pensions will please come to order.

This morning, we're holding a hearing on Continuing America's Leadership: Realizing the Promise of Precision Medicine for Patients. Senator Murray and I will each have an opening statement. Then we'll introduce our panel of witnesses, who are getting to be very familiar to us.

We're very grateful to you for coming.

After that, we'll have time to ask 5-minute rounds of questions or two, depending on how many Senators are here.

We're here today to discuss an exciting new direction in our healthcare called precision medicine. What does that mean?

Well, if those of us in this room were a good representation of the U.S. population, the Centers for Disease Control and Prevention estimates that nearly 1 in 10 of us would have diabetes. If doctors could use precision medicine—that is, if they could look at our individual DNA and the genetic and molecular makeup of our disease—perhaps they could then potentially tailor treatments to each individual, rather than to the more general category of diabetes.

I was visited this morning by the head of Philadelphia Children's Hospital, who talked about their work in identifying a genetic defect that causes blindness and how they have developed a therapy that restores the sight in a child because the therapy is directed for that specific genetic defect.

This is happening with cancer treatment. Doctors can look at the mutations of the cancer cell and assess how to treat it. Newsweek reports that genetic sequencing of tumors is already starting to become the norm. In the big cancer hospitals like Sloan-Kettering,

Dana-Farber, and MD Anderson, all incoming patients automatically have their tumors sequenced.

This is all possible because of the extraordinary achievement by a great many individuals of sequencing the human genome, none more important than Dr. Collins, who is here with us today, and we are grateful for that.

Today, we want to discuss what the National Institutes of Health and private industry are doing in precision medicine, how the Food and Drug Administration will regulate these innovations, how electronic health records can affect our ability to innovate, and what this means for the American patient and for our health care system. This is one of the most exciting new frontiers in medicine.

Senator Murray and I are working on an initiative to ensure that our Federal agencies are equipped to review the medical products and processes produced by this kind of cutting edge medicine, so that American patients aren't waiting on the sidelines because regulatory science can't keep up. Our innovation initiative is not just about precision medicine, but precision medicine is an important part of our initiative.

President Obama announced a Precision Medicine Initiative in the State of the Union this year. He detailed his plans in an event at the White House. I attended that to demonstrate my support for it.

The President has proposed, as part of his plan, mapping the genomes of 1 million individuals and making that information available to medical researchers across the country. I look forward to hearing more about that from our witnesses.

I also know there are similar private efforts underway and I am interested in hearing about that competition and about possible collaboration. For example, the Children's Hospital of Philadelphia that I mentioned also has genome sequences of children at their hospital, and I wonder how useful that would be to the 1 million that Dr. Collins is putting together and how these scientific entrepreneurs, Dr. Venter in California, the doctors at CHOP in Philadelphia, and other places—how that relates to the President's proposal for 1 million individuals.

I look forward to hearing more about the potential cost of precision medicine. We know that costs to sequence the human genome have been reduced significantly in the last two decades. Dr. Collins testified—I believe that he said 15 years ago, it cost us about \$400 million to sequence the first human genome, whereas today it's about \$1,000.

Very often in health care, innovation initially increases our costs. That doesn't mean we shouldn't innovate. Innovative new products can increase costs, but in the long term actually decrease health care costs.

Take Alzheimer's, which, according to the Alzheimer's Association, will cost us \$226 billion this year along with other dementias. If we could use precision medicine to delay onset or cure that disease, we could save precious dollars in our healthcare system and alleviate some of the grief and pain associated with it.

The committee has also spent some time and will spend more on improving electronic health records. The Federal Government has spent \$28 billion to drive the adoption of these records systems,

and the result is that doctors don't like the systems. Many say they disrupt workflow, they interrupt the doctor-patient relationship, and that haven't been worth the effort.

Senator Murray and I have begun a working group to identify the five or six things we can do to help make the failed promise of electronic health records something that physicians and providers look forward to instead of something they endure.

Dr. DeSalvo, we look forward to working with you on that and with Secretary Burwell and being able to report maybe early next year some results, either that you take administratively or that we do legislatively or some of both. We have to get to a place where the systems can talk to one another—interoperability—and where doctors, particularly the smaller physicians' offices, want to adopt these systems, can afford the cost, and can be confident that their investment will be of value.

Dr. Collins has told us—and I've heard from many others—that a properly functioning electronic medical records system is tremendously important to the President's Precision Medicine Initiative. No. 1, it can help to assemble the genomes of the 1 million individuals; and, second, if we want to make genetic information useful it's going to take computers that operate easily and with the click of a mouse to help make it possible for doctors to actually prescribe prescriptions for individual patients.

I also would like to hear if we know, Dr. DeSalvo, at some point, how the \$11 billion effort by the Defense Department on electronic medical records would relate to the \$28 billion we've already spent and whether those will be compatible and whether you'll be working with them.

There's a lot to talk about today. This is a tremendously interesting and important effort, and I look forward to the witnesses' comments.

Senator Murray.

STATEMENT OF SENATOR MURRAY

Senator MURRAY. Well, thank you very much, Mr. Chairman.

Thank you to all of our witnesses for being here today. Each of your agencies plays a critical role in the topic we're going to be talking about, and I'm grateful to have you all here to share your expertise.

I've approached our bipartisan effort to advance medical innovation focused on one question in particular, and that is: What can Congress do to help all patients and families get the safest, most effective treatments and cures more quickly? Our conversation today is about the promise of precision medicine, and it is a crucial and truly exciting piece of the puzzle.

There's no question we are at a critical moment in the medical field. Researchers and medical experts are increasingly finding ways to treat patients not just as the average patient but, instead, based on their own unique characteristics and history.

This is like the difference between getting eyeglasses based on the average prescription and getting eyeglasses based on your own prescription. It's huge, especially for patients and families across the country who are waiting and hoping for better treatments and cures.

I'm proud that my home State of Washington is home to several institutions that have been pioneers in this area. These include the Fred Hutchinson Cancer Research Center and the University of Washington, which are using precision medicine technology to tackle breast cancer, eye disease, and Alzheimer's disease, among others.

I'm glad we have the opportunity today to discuss the ways in which precision medicine is changing and improving lives and how Congress can help advance this new frontier in biomedical innovation for patients and families.

The President has proposed making significant investments in precision medicine. His fiscal year 2016 budget supports a bold new initiative to exploit the recent advances in genomics, molecular biology, and data management to support the shift away from this one-size-fits-all medicine and toward treatment tailored to specific individuals. This proposal could do an enormous amount to accelerate the advancement of precision medicine. But as I discussed with Dr. Collins in our appropriations hearing last week, I am deeply troubled by the steady erosion of NIH's purchasing power over the last decade.

Last Congress, Democrats and Republicans were able to come together to replace harmful sequestration cuts to investments in NIH, FDA, and other critical priorities, like education, infrastructure, and defense. I am really hopeful that this year, despite the budget proposals put forward by my Republican colleagues, we will be able to work across the aisle and find a way to prevent these shortsighted cuts from kicking in again.

This is absolutely critical to the kinds of investments we need to make to help families and grow our economy, including precision medicine. One of my top priorities on this committee is looking for ways to continue improving the quality of care patients receive, and supporting precision medicine is essential to this goal.

By offering patients and providers more and much better health information, patients, in consultation with their doctors, will be empowered to make informed decisions about their care. Our health care system will be better equipped to put their needs first.

I do want to note that protecting privacy will be an important challenge throughout this process. Just in the last few months we have seen serious security breaches impacting families' personal health information, and that is unacceptable.

As researchers, providers, and patients gather and use more health information, we need to be aware that data is being created that cyber criminals will want to exploit, and that means we will need to develop strategies to protect privacy that meet today's challenges. Chairman Alexander and I are investigating the current state of cyber security in the health sector, and it is clear that this needs to be an all-hands-on-deck effort with providers, insurers, and government working together.

Again, thank you to all our witnesses for being here today. I want to thank Chairman Alexander for holding this hearing on a topic of such importance for patients and families in Washington State and across the country.

I look forward to working together, Mr. Chairman, with you and other members of the committee to support this important initiative.

The CHAIRMAN. Thank you, Senator Murray.

To underscore what Senator Murray said, this specific topic is an initiative of the President in which the committee, in a bipartisan way, is very interested. We expect to get a result, and we welcome the expert advice.

We have three witnesses, and I'll ask Senator Cassidy if he'd like to introduce the first one.

Senator CASSIDY. Yes, Dr. Karen DeSalvo. Dr. DeSalvo and I know each other from way back when I was full-time with LSU and she with Tulane, and I told her that just in her honor, we made the spread Tulane green today.

Dr. DeSalvo is the National Coordinator for Health Information Technology at the Office of the National Coordinator of Health Information Technology, or ONC. ONC is the lead agency charged with formulating the Federal Government's health IT strategy and coordinating Federal health IT policy, standards, programs, and investment.

I've been impressed. Dr. DeSalvo has come to me personally. There's a friend back home who is having a lot of problems with her electronic medical record and adapting to it. She called her, and they spoke at length. She clearly recognizes interoperability as key. She is working with and listening to physicians and developers of these products.

Before joining HHS, Dr. DeSalvo was the Health Commissioner for the city of New Orleans, including and after Hurricane Katrina.

The CHAIRMAN. Thank you, Senator Cassidy.

Dr. DeSalvo, we look forward to your testimony. We expect to get to know you pretty well here, because all of us are interested in fixing the electronic medical record system, and you're on point for that, according to Secretary Burwell. We look forward to that.

Our other two witnesses are here about every other day, it seems, and we're grateful for that. Dr. Collins, the Director of the National Institutes of Health, who oversees the work of the largest supporter of biomedical research in the world, has been the Director since 2009. Of course, he is known, among other things, for his leadership of the International Human Genome Project, completely sequencing the human genome in 2003.

Dr. Jeff Shuren was here just last week. He has been the Director of the Center for Devices and Radiological Health at the Food and Drug Administration for more than 5 years. They're responsible for assuring the safety, effectiveness, and quality of medical devices; assuring the safety of radiation-emitting products; and fostering device innovation.

He's had a lot of experience, and 1 year of that experience was being detailed to this committee as a part of Senator Kennedy's staff. So we welcome him back.

If the witnesses would summarize their remarks in about 5 minutes, we would appreciate it. We have Senators here who want to have a conversation with you. Let's start with Dr. Collins.

**STATEMENT OF FRANCIS S. COLLINS, M.D., Ph.D., DIRECTOR,
NATIONAL INSTITUTES OF HEALTH, BETHESDA, MD**

Dr. COLLINS. Well, good afternoon, Chairman Alexander, Ranking Member Murray, and distinguished committee members. It's an honor to appear before you today to discuss how we can advance America's health by accelerating progress toward a new era of precision medicine.

Earlier this year, the administration unveiled the Precision Medicine Initiative, a bold new research effort to revolutionize how we diagnose and treat disease. We believe the time is right for this ambitious initiative, and the NIH and our partners, the FDA and ONC, will work hard to achieve this vision.

Historically, physicians have had to make most recommendations about disease prevention and treatment based on the expected response of the average patient. This one-size-fits-all approach works for some patients and some conditions but not others.

Precision medicine is an innovative approach that takes into account individual differences in patients' genes, environments, and lifestyles. The concept is not entirely new. Blood typing, for example, has been used to guide blood transfusions for almost a century.

The identification of the BRCA1 and BRCA2 genes has made it possible to provide options for women at high risk of breast or ovarian cancer. The gene implicated in cystic fibrosis, discovered in my own laboratory 25 years ago, has led to widespread availability of carrier screening and targeted therapeutics.

The prospect of applying this concept broadly has been dramatically improved by the development of powerful and affordable methods for characterizing personal biological information. That includes genomics, the widespread adoption of electronic health records, the recent revolution in mobile health technologies, and the emergence of computational tools for analyzing large biomedical datasets. Furthermore, patients are increasingly interested in taking part in research.

All of these developments will help make possible the dream of personalizing a wide range of health applications. With this in mind, we are thrilled to take a lead role in the multiagency Precision Medicine Initiative.

In the near term, this initiative will focus on cancer, accelerating efforts to develop precision medicine strategies for a wide range of adult and pediatric cancers. This component will include the molecular analysis of large numbers of individual tumors to see what gene mutations are actually driving the malignancy, and then matching that information with available targeted therapeutics provided by pharmaceutical industry partners to optimize responses for the individual.

Simple blood tests will be developed that can detect early response or resistance to drug therapy. Combinations of targeted drugs will be tested to see how best to achieve not just a remission, but a cure.

To put a human face on this, I'd like to paint you a forward-looking picture of what the Precision Medicine Initiative could deliver for cancer in a few years. Consider the hypothetical case of Lily, a 52-year-old woman of Asian descent. In 2018—this is a hypothetical case—after battling bronchitis and a persistent cough for

several months, Lily goes to her doctor, who orders a lung CT scan, along with a new blood test developed through research supported by the Precision Medicine Initiative, to look for DNA and other biomarkers circulating in her blood.

The CT scan, as you can see from the arrow there, detects a tiny spot that could be either inflammation from bronchitis or cancer—not clear. The biomarker test clinches the diagnosis, revealing a genetic mutation that occurs only in patients with cancer.

In 2015, today, her prognosis from this cancer would likely be pretty grim. In 2018, that could all change. Lily is treated with surgery to remove the tumor, and the tumor DNA then undergoes additional molecular analysis.

Based on those results, Lily is treated with a targeted drug that was originally developed for skin cancer but has just the right properties for her tumor. She also receives a course of immunotherapy specifically designed to kill any tumor cells that may still be lurking in her body. With this treatment, a decade later, Lily remains cancer free. That is a hypothetical but quite realistic example of what the cancer component of this initiative could achieve.

As a longer term goal of this initiative, NIH will launch a National Research Cohort of 1 million or more volunteers who will play an active role in how their medical, genetic, and environmental information is used to prevent and manage a broad array of diseases. Participants, some recruited from existing NIH-supported cohorts and some new volunteers, will be centrally involved in the design and implementation of this process. They will be true partners.

With appropriate privacy protections, they will be able to share genomic data, lifestyle information, and biological samples, all linked to their electronic health records. Participants will be able to have access to their own health-related information.

New approaches for detecting and analyzing a wide array of biomedical variables will be initially tested in small pilot studies focused both on prevention and management of disease. Ultimately, the most promising approaches will be utilized in greater numbers of people over longer periods of time to collect valuable data that will be of great benefit to both researchers and patient partners.

Let me quickly give you an example of how this could benefit a specific participant in the Precision Medicine Initiative but could also provide evidence for a new strategy for health maintenance that could be extended across the Nation.

Consider the case of 38-year-old Precision Medicine Initiative participant Carla. It's 2020. Carla feels perfectly healthy. She welcomes the chance to try out a wearable sensor that continuously monitors her pulse, blood pressure, physical activity, and sleep patterns, but discovers that her blood pressure usually runs about 150 over 100, too high, increasing her risk of stroke, heart attack, kidney failure, and other life-threatening conditions.

Carla is not alone, by the way. Nationwide, about 78 million Americans, one out of three adults, have high blood pressure. Many, like Carla, don't even know it. What's worse, nearly 50 percent of those diagnosed with hypertension do not have it under control.

Carla consults with her doctor who confirms the need for treatment and suggests she take an inexpensive diuretic drug. Carla obtains a smart bottle that sends a message to her smart phone if a dose is missed, so she takes her pills on schedule, returning her blood pressure to the normal range, avoiding future medical crises.

The Precision Medicine Initiative will also bring many other types of healthcare monitoring into this new century. Current evidence suggests that the venerated annual physical exam and associated screening lab tests may not be as useful as one would hope.

New opportunities to incorporate much more sensitive and specific indicators of individual health are emerging. This National Research Cohort will provide a powerful opportunity to assess such strategies rigorously to see if they really provide clinically valid information and, most importantly, to better health outcomes for the American people.

In closing, let me emphasize that the impact of the Precision Medicine Initiative will extend far beyond the individuals who volunteered to participate. It will push the frontiers of discovery across the entire spectrum of biomedical research, from basic science aimed at finding new therapeutic targets to translational science intent on moving research discoveries into practice for maximum public health benefit.

Given the size of the project and its real-world nature, evidence of improved health outcomes derived from this initiative will be attractive for immediate application across U.S. medical care. With sufficient resources and a strong, sustained commitment of time, energy, and ingenuity from the scientific, medical, and participant communities, the future of precision medicine appears very bright. We really look forward to working together to make stories like those of Lily and Carla a reality.

That concludes my testimony. I look forward to answering your questions.

[The prepared statement of Dr. Collins follows:]

PREPARED STATEMENT OF FRANCIS S. COLLINS, M.D., PH.D.

Good afternoon, Chairman Alexander, Ranking Member Murray, and distinguished members of the committee. I am Francis S. Collins, M.D., Ph.D., and I am the Director of the National Institutes of Health (NIH).

It is an honor to appear before you today, alongside my dedicated colleagues, to discuss how we, as a Nation, can advance the health of the American public by accelerating progress toward a new era of precision medicine.

As the Nation's premier biomedical research agency, NIH's mission is to seek fundamental knowledge about the nature and behavior of living systems, and to apply that knowledge to enhance human health, lengthen life, and reduce illness and disability. I can report to you today that NIH leadership, employees, and grantees continue to believe passionately in that mission.

In January of this year, the President announced a new Precision Medicine Initiative—a bold, new research effort to revolutionize how we diagnose and treat disease, including a \$215 million investment in the President's fiscal year (FY) 2016 Budget. We believe that the time is right for this ambitious initiative, and the NIH and our partners, the U.S. Food and Drug Administration (FDA) and the Office of the National Coordinator for Health Information Technology (ONC), will work hard to achieve this vision.

Historically, physicians have had to make most recommendations about disease prevention and treatment based on the expected response of the average patient. This one-size-fits-all approach works for some patients and some conditions, but not others. Precision medicine is an innovative approach that takes into account individual differences in patients' genes, environments, and lifestyles. This concept is not new; blood typing, for example, has been used to guide blood transfusions for

more than a century. Prescription eyeglasses are tailored specifically to the patient's individual needs. Moreover, the identification of the *BRCA1* and *BRCA2* genes has made it possible to provide options for women at high risk for breast and ovarian cancers. The gene implicated in cystic fibrosis has led to widespread availability of screening and targeted therapeutics.

The prospect of applying this concept broadly has been dramatically improved by the development of powerful and affordable methods for characterizing personal biological attributes (such as genomics and metabolomics), the widespread adoption of electronic health records, the recent revolution in mobile health technologies, and the emergence of computational tools for analyzing large biomedical data sets. These advances will help make possible the dream of personalizing a wide range of health applications.

With this in mind, we are excited to take a lead in the two key components of the President's Precision Medicine Initiative that will be managed by NIH: a near-term goal that will focus on cancer and a longer term aim to generate knowledge applicable to the whole range of health and disease. Both components are within reach, due in large part to scientific breakthroughs in basic research. Furthermore, the initiative will tap into converging trends in connectivity, through social media and mobile devices, and Americans' growing desire to be active partners in medical research in a way that protects their privacy.

Oncology is the clear choice for enhancing the near-term impact of precision medicine. Cancers are common diseases and are among the leading causes of death nationally and worldwide, and their prevalence is increasing as the population ages. They are especially feared because of their lethality, their symptoms, and the often toxic therapies used to treat them. Cancer research has been leading the way in precision medicine for many years. Thanks to advances in DNA sequencing and efforts such as The Cancer Genome Atlas project, we now have a better understanding of the molecular changes that drive many cancers and we can define the driver mutations in individual tumors and use this information to design the ideal therapy for each patient. Genomic information has already helped shape the development of some cancer treatments. For example, the drug, imatinib (Gleevec), was designed to inhibit an altered enzyme produced by a fused version of two genes found in chronic myelogenous leukemia.

While we've made significant strides in recent years to learn the molecular signatures of many cancers, much more remains to be done. The National Cancer Institute will accelerate the design and testing of effective, tailored treatments for cancer by expanding genetically based clinical cancer trials, exploring fundamental aspects of cancer biology, and establishing a national "cancer knowledge network" that will generate and share new knowledge to fuel scientific discovery and guide treatment decisions. Furthermore, we aim to understand the development of resistance to targeted therapy, apply non-invasive methods to track patients' responses to treatment such as liquid biopsies, and explore the efficacy of new drug combinations targeted to specific tumor mutations.

As a longer term goal of this initiative, NIH will launch a national research cohort of one million or more volunteers who will play an active role in how their genetic, environmental, and medical information is used for the prevention of illness and management of a wide array of chronic diseases. This component will pioneer a new model for doing research; one in which people who participate are true partners. Not subjects, not patients—partners. The goal will be to expand the benefits of precision medicine into myriad aspects of health and health care. Participants will voluntarily share clinical data from electronic health records, results of imaging and laboratory tests, lifestyle data and environmental exposure recordings tracked through real-time mobile health devices, and genomic information—all with appropriate privacy protections.

Participants will be at the center of the project design, and they will have access to their own health data, as well as research using their data, to help inform their own health decisions. As volunteers, each individual will participate because they choose to be a partner in this bold research effort. Through this dynamic community, researchers will be able to advance the information derived from this cohort into new knowledge, approaches, and treatments. Researchers from many organizations will, with proper protection of patient information, have access to the cohort's data so that the world's brightest, scientific and clinical minds can contribute insights.

In order to help inform the vision for building the national research cohort of one million or more volunteers, a Precision Medicine Initiative Working Group was recently created. This group of experts in precision medicine and large clinical research studies is seeking public input from the diverse stakeholder community interested in the development of this initiative, including the patient community, and

will articulate the vision for advancing participant engagement. They will help define what can be learned from a study of this scale and scope, what issues will need to be addressed as part of the study design, and what success would look like in the near and longer term. With the guidance from this team of experts, we will move ever closer to realizing the goals of this ambitious research program.

A project of this magnitude will lay the foundation for a myriad of new prevention strategies and novel therapeutics. Although the initiative will likely yield its greatest benefits years down the road, there will be successes in the relatively near future as well, especially in the areas of cancer and pharmacogenomics—how to provide the right drug at the right dose to the right person at the right time. Moving forward, this pioneering research initiative will require the involvement of many different sectors of science and society, including biologists, physicians, technology developers, data scientists, and especially the American people. Given related efforts in a few other countries, we will aim to forge collaborations on a global scale.

With sufficient resources and a strong, sustained commitment of time, energy, and ingenuity from the scientific, medical, and participant communities, precision medicine's full potential can be realized to give everyone the best chance at good health. There's no better time than now to embark on this ambitious new enterprise to revolutionize medicine and generate the scientific evidence necessary to move this individualized approach into everyday clinical practice.

With your support, the future of medicine can be very bright. This concludes my testimony, and I look forward to answering your questions.

The CHAIRMAN. Thank you, Dr. Collins.
Dr. DeSalvo, welcome.

STATEMENT OF KAREN B. DeSALVO, M.D., MPH, MSc, NATIONAL COORDINATOR FOR HEALTH INFORMATION TECHNOLOGY, WASHINGTON, DC

Dr. DeSALVO. Thank you, Senator Alexander and Ranking Member Murray and to the other distinguished Senators. Thank you for the opportunity to be here today with my colleagues, Dr. Collins and Dr. Shuren.

I'm Karen DeSalvo. I'm the National Coordinator for Health Information Technology at the Department of Health and Human Services.

When I was a medical student at Tulane, I could have never imagined that in my career, I would see medicine on the frontier of such a significant transformation. As a still practicing doctor, it is thrilling for me to know that we are on the cusp of being able to customize treatment for the patient in front of me based on their genetics, preferences, and other key information instead of having to treat them as the average patient.

What is even more exciting is that precision medicine is not just a theory. It's already changing practice and saving lives in the United States. We wouldn't be on this cusp but for health information technology, which is foundational to the President's Precision Medicine Initiative.

The Office of the National Coordinator is the Federal lead for health information technology, and ONC's responsibility is to advance the health IT infrastructure for what is a sixth of the U.S. economy—healthcare. We do this work through a mixture of programs, convenings, and technical assistance aimed at catalyzing the marketplace. We seek to spur and support innovation to help address important advancements like precision medicine.

At the same time, we want to provide clear and steady direction. ONC also has responsibility to ensure that all consumers are engaged and their interests are protected.

Congress created significant momentum in health IT when it passed the HITECH Act in 2009. The act provided funding to support the adoption of electronic health records and technical supports for doctors and hospitals on the front lines as they made the transition to use them.

As a result of the HITECH program and the hard work of providers, we are bringing healthcare into the digital age, and we have reached a tipping point. The strong foundation of health information technology makes it possible to bring to the bedside personalized treatment through precision medicine.

The data in electronic health records, married with advanced analytics, information from mobile health devices, and other sources of data, including patient preferences, will provide the full picture of a person's health and needs. This comprehensive data picture is necessary to identify the right prevention and treatment that is not only the most effective, but also most desired by the patient.

This is not just a vision about what might come but a reality already. In places like Tennessee and Maryland, Nebraska, Florida—I could go on—thanks to the tools built into the electronic health record, doctors are able to tailor treatment today.

I spoke to folks at the University of Florida Health, where a patient's cardiologist can order a test to see if they carry a particular variant of a gene. This test will help the cardiologist know if they are using the best medicine to prevent a future clot in the patient's heart. At a critical time in someone's life, a doctor is choosing the right lifesaving blood thinner, tailored and specific for them.

Though this kind of treatment is exciting, it is, indeed, only the beginning. We have much work to do ahead to see that this is available to everyone in this country as part of routine care, and ONC stands ready to undertake this work.

To get there, we will need to stay the course in adoption to see that every American has an electronic health record. We also need to go beyond the pockets of data exchange and achieve true interoperability as described in our nationwide roadmap.

We will need to establish standards for the most fundamental clinical information that are shared by all. We will also need to establish standards for new data necessary for precision medicine, including genomics, but also environmental exposures and patient-generated information.

We will build a trust framework that respects individual privacy and establishes strong security protections. We will work with the private sector to establish openly available APIs, which are doorways to unlock data.

In all of this work, we will remember what I hear consistently from consumers who are our principal customer. They want to be able to access and share their health information, including with scientists if they wish, without blocking or delay.

The President's Precision Medicine Initiative is one of the most exciting ways that we can bring the right care, the right prevention to the right patient, only imagined a few years ago. It's because of advances like this and the underlying technology that we have to support it that we are on the cusp of realizing better care and

health for everyone. ONC stands ready to help further precision with our colleagues at HHS and with Congress.

Thank you, and we look forward to your questions.
[The prepared statement of Dr. DeSalvo follows:]

PREPARED STATEMENT OF KAREN B. DESALVO, M.D., MPH, MSC

Chairman Alexander, Ranking Member Murray, and distinguished committee members, thank you for the opportunity to appear today. My name is Dr. Karen DeSalvo and I am the National Coordinator for Health Information Technology. I appreciate the invitation to be here to discuss how health information technology plays a necessary role in our Nation's precision medicine efforts.

For far too many diseases, there is no proven means of prevention or effective treatment. We must gain better insights into the biology of these diseases to make a difference for the millions of Americans who suffer from them. Precision medicine is an emerging approach for disease treatment and prevention that takes into account variability in genes, environment, and lifestyle for each person instead of a "one-size-fits-all-approach." While significant advances in precision medicine have been made for select cancers, the practice is not currently in use for most diseases. Many efforts are underway to help make precision medicine the norm rather than the exception. To accelerate the pace, President Obama has unveiled the Precision Medicine Initiative—a bold new enterprise to pioneer a new model of patient-powered research that promises to accelerate biomedical discoveries and provide clinicians with new tools, knowledge, and therapies to select the treatments that will work best for individual patients. Working with our colleagues at the NIH and FDA, our mission is to improve the lives of all Americans by empowering patients, research participants, scientists, and providers to work together and turn new knowledge into individualized treatment and prevention strategies for a new era of precision care.

To turn the promise of precision medicine into the reality of better health, we require robust and useful information tools, systems and practices for participants, providers, and more. As President Obama stated when he unveiled this initiative,

"This helps us find new cures but also helps us create a genuine health care system as opposed to a disease care system. We want each of us to have sufficient information about our individual differences so that we can make better life decisions."

This goal cannot be realized without unlocking the data stored in various health information technology tools to ensure consumers have access to their own health data—and to the applications and services that can safely and accurately analyze it—so that in addition to treating disease, we can empower individuals and families to invest in and manage their health in partnership with their clinicians.

Health information technology is the foundation required to bring precision medicine to operational life. Genomics helps to define us as individuals, and is one kind of important data for precision medicine. Other data of emerging importance to our health include microbiomes or bacteria and other microbes that share our bodies, environmental exposures or "exposome," social determinants of health, and personal lifestyle choices—all of which provide us with more detailed knowledge about ourselves.

We at the Department of Health and Human Services have recognized the importance of this data to advance better health and a healthier Nation, and have been working over the years to further refine our information infrastructure through health information technology adoption, policy development, and innovation by both the public and private sector. The Office of the National Coordinator for Health Information Technology (ONC) was established by Executive order in 2004, charged with the mission of giving every American access to their electronic health information when and where they need it most. In 2009, ONC was statutorily established in the Health Information Technology for Economic and Clinical Health Act (HITECH), enacted as part of the American Reinvestment and Recovery Act of 2009 (ARRA). HITECH also provided the resources and infrastructure needed to stimulate the rapid, nationwide adoption and use of health IT, especially electronic health records (EHRs). In the 6 years since the HITECH Act was enacted, we have seen dramatic advancement in the use and adoption of health information technology. The combined efforts of initiatives like the Regional Extension Centers, the ONC Health IT Certification Program, use of standard terminologies, and the CMS Medicare and Medicaid EHR Incentives Programs have brought us past a tipping point in the use of health information technology. Today, we are irreversibly on the path to a digital health care system.

Since I became the National Coordinator in January of last year, HHS has been working intensely to harness both the health care industry's energy and consumer demands for interoperability to drive improvement in health—we feel the strong sense of urgency and have acted on it quickly. The Nation asked for a clear strategy to get to interoperability and a learning health system, and we delivered that plan in *Connecting Health and Care for the Nation: A Shared nationwide Interoperability Roadmap Draft Version 1.0*. We received broad feedback and have heard agreement from critical stakeholders like developers, consumers, providers, technologists, and others that this plan is the right path forward, and that they would like to work with us to advance interoperability. The roadmap explains that to get to interoperability as quickly and safely as possible we need to build upon the current infrastructure and we need to pursue three immediate goals.

First, we need to focus on ensuring that applicable standards are consistently used, including standards for application programming interfaces, health care terminology, implementation, and security. Second, we need to foster an environment of trust where individuals can access their data, and where that data is kept private and secure. Third, we need to incent, through consumer demand and delivery system reform, interoperable movement and use of electronic health information that endures and is self-sustaining. These three goals will ultimately advance health care and health.

Our work in interoperability matters because it is what the Nation expects, but also because, in order for physicians, scientists, researchers, individuals and other partners to provide care tailored to the specific needs and characteristics of individuals, they will need to be able to access individual level information to learn more about how to treat patients, and ultimately improve the diagnosis, treatment, and prevention of diseases. This information cannot flow in the form of mail or fax to partners across the country—it must be quickly, efficiently and appropriately available electronically, and with patient consent when required by law, we must be able to apply the incredible speed and computing power available in the 21st century to help us analyze the data.

To advance this work in precision medicine, ONC will build on our strong foundation through our standards advancement authorities, our regulatory authorities, our policy expertise and our deep connection with the private sector and consumers, in close coordination with our Federal partners. As proposed in the President's fiscal year 2016 budget, ONC would fund standards—coordination and development—to advance the basis on which precision-based medicine can be practiced. ONC's \$5.0 million funding proposal will lay the groundwork to achieve many of the milestones included in the Interoperability Roadmap's milestones for how health IT can support a learning health system. ONC will engage industry stakeholders to identify the standards, technology, and policy necessary to support big data analyses and precision medicine with appropriate privacy protections. Working closely with our many partners, ONC will aggressively pursue a portfolio of standards and technology initiatives that support precision medicine and protect user privacy, such as the standardization and use with consent of patient-generated health data from non-clinical settings; the incorporation of genomic data into health IT with appropriate protections; patient identity management and matching with consent to permit linked analyses; a patient's ability to access their data and contribute it to research projects, and new platforms for clinical trial recruitment through the use of health IT.

Health information technology and information sharing plays a fundamental role in the President's Precision Medicine Initiative to improve care and speed the development of new treatments. We look forward to building on our current foundation and reaching for the future of better health for all Americans. Thank you again for inviting me today.

The CHAIRMAN. Thank you, Dr. DeSalvo.
Dr. Shuren.

STATEMENT OF JEFFREY SHUREN, M.D., J.D., DIRECTOR, CENTER FOR DEVICES AND RADIOLOGICAL HEALTH, FOOD AND DRUG ADMINISTRATION, SILVER SPRING, MD

Dr. SHUREN. Chairman Alexander, Ranking Member Murray, and distinguished members of this committee, thank you for the opportunity to testify regarding FDA's role in the administration's Precision Medicine Initiative.

The success of precision medicine depends upon having accurate, reliable, and clinically meaningful tests, because it's the results of the tests that determine which patients get which drugs or treatment and whether or not they get them. Imprecise medicine results from bad tests. You have misdiagnosis, you get the wrong treatment, or you get no treatment at all when you should, and as a result, patients get harmed and healthcare costs go up.

FDA's role in the Precision Medicine Initiative is primarily focused on advancing an emerging technology called next-generation sequencing or NGS. NGS tests can sequence long segments of a patient's DNA or even the entire genome. As a result, we're moving away from the model of one test, one disease, but a test that can identify one of many different diseases or even the risk of developing that disease.

Today, there are significant barriers in place for advancing that technology. It affects our research, it affects development, and it affects our ability to use this optimally in healthcare. Let me tell you what some of those barriers are and what we're doing about it.

If you're making a test, and you want to know if it's accurate, reliable, and clinically meaningful, two of the things you need to know are the following. One, does it accurately measure what you're trying to measure, in this case, genetic variants, to identify the right variants? We call that analytical validity.

Second, you want to know: If there is a good relationship between what you measure and the particular disease? To test for breast cancer, is that variant, in fact, associated with breast cancer? We call that clinical validity.

Today, that's difficult to do for next-generation sequencing, and here's why. Think about the human genome. You have about 3 million variants, and your genes are made up of components called base pairs. There are 3 billion of them. You want to know: How accurate is next-generation sequencing tests to measure all of that?

Normally, you would look at each of the variants. You can't assess the accuracy of 3 million variants. It would take forever. There aren't good standards out there to assess it, so people are struggling to make sure their tests are accurate.

Then you want to know: Is it clinically meaningful? Well, you need data for that. The problem is many of these variants are uncommon, so it's hard to get a lot of clinical data. It's difficult to do clinical studies, and that data tends to be siloed in the institutions that are doing the testing.

In December, we proposed an entirely different framework for the oversight of next-generation sequencing tests, tests we've regulated for a long time, but we and the developers had struggled on what to do with it. So for analytical validity, we need to have reference standards, essentially subsets of genetic variants that if you can show accuracy in measuring those, it is reasonable to infer you're good at measuring the other variants.

In fact, we gave \$2 million to the National Institute for Standards and Technology, NIST, to work with the scientific community to come up with the very first reference standard for the genome, and they just released that last week. Under this initiative and with additional funding support, we will continue to work with NIST and the scientific community on developing additional ref-

erence standards, and as a result, developers wouldn't have to come to the FDA to show they're analytically valid. They would just meet that standard.

For clinical validity, we need to leverage databases, curated databases, where we get all of that siloed genetic information, and then make sure it's standardized and it's of sufficient quality that we can make decisions on it. We're partnering with Francis and his team to take advantage of a database they have called ClinVar and the curation activity behind it called ClinGen, and then working with them and the scientific community to develop standards and best practices for having these databases, for doing the curation, and then having consistent clinical interpretation.

Because you know what happens today? You can send your blood to different genetic testing labs, and you can get different results. That's what happens. It may be because you missed the particular genetic variant, or you interpret it differently. With those standards in place, we can now have consistent accuracy in testing and consistent clinical interpretation and reduce the time and cost to spur research, to advance technology development, and ultimately to achieve better health outcomes.

Thank you.

[The prepared statement of Dr. Shuren follows:]

PREPARED STATEMENT OF JEFFREY SHUREN, M.D., J.D.

Chairman Alexander, Ranking Member Murray, and members of the committee, I am Dr. Jeffrey Shuren, Director, Center for Devices and Radiological Health (CDRH) at the Food and Drug Administration (FDA or the Agency). Thank you for the opportunity to be here today to discuss the important role FDA is playing in the Administration's Precision Medicine Initiative as part of our mission to protect and promote the public health by ensuring the safety, efficacy, and quality of medical products.

The President's Precision Medicine Initiative, launched in January 2015, is a new effort to revolutionize how we improve health and treat disease in the United States. The initiative will pioneer a new model of patient-powered research that promises to accelerate biomedical discoveries and equip clinicians with new tools, knowledge, and therapies to select which treatments will work best for which patients. Additionally, through collaborative public and private efforts, the initiative will leverage advances in genomics, emerging methods for managing and analyzing large data sets, and health information technology to accelerate biomedical discoveries, all while protecting patient privacy.

A key technology that will advance the Precision Medicine Initiative is Next-Generation Sequencing (NGS) technology.¹ NGS tests can rapidly sequence large segments of an individual's DNA and even an individual's entire genome. In fact, an NGS test is capable of detecting the billions of bases in the human genome, and in doing so identify the approximately 3 million genetic variants an individual may have. A single use of an NGS test could enable the diagnosis of any one, or more, diseases or conditions a patient presents with or help to predict a patient's risk for numerous conditions.

The use of NGS tests also is accelerating the pace of scientific discovery, as the compilation of large amounts of genetic information in scientific databases and electronic health records enables scientists to perform observational studies and computer modeling to better understand whether and how certain genetic variants, including very rare variants, are linked to certain conditions and diseases. As the Initiative moves forward, we expect NGS technologies to play a central role in both research and clinical practice.

For precision medicine to succeed, NGS tests must be accurate, reliable, and clinically meaningful. As with other diagnostic tests, an inaccurate NGS test can lead

¹Next-Generation Sequencing, also referred to as "massively parallel sequencing" or "high-throughput sequencing," refers to technologies that perform DNA sequencing in parallel, allowing for the production of thousands or millions of sequences concurrently.

to patients receiving the wrong diagnosis,² the wrong treatment, or no treatment at all, even when effective therapy is available. Inaccurate NGS tests can impose unnecessary costs on the health care system. Inaccurate tests could cause healthy individuals to seek further testing and treatment to address an erroneous belief that they have, or could develop, a certain condition or disease. As an example, if a patient was informed that she had a dominant mutation that confers increased risk for breast and ovarian cancer, that patient might choose to have complete mastectomy and hysterectomy, in order to prevent future cancer. In addition, the patient's family would be alerted to their own genetic risk. If the test results were inaccurate, the prophylactic surgery and all the family followup may not have been necessary. As treatment for cancer becomes more influenced by genetic testing of the tumor, and treatment based on type of mutation, it is increasingly important to ensure accurate and reliable tests. Thus, FDA oversight is critical to protect the public health and to maximize the benefits of precision medicine.

The capabilities of NGS tests and their rapid evolution, however, pose unique challenges to applying FDA's traditional regulatory approach for determining whether a diagnostic test is accurate and reliable (analytical performance) and if the results from the test correctly identify the relevant disease or condition (clinical performance) on a test-by-test basis. Specific challenges related to NGS tests include:

- The need to evaluate the ability of the test to produce accurate and reliable results. Because NGS can identify an essentially unlimited number of variants, it would be difficult, if not impossible, to demonstrate performance on every possible detectable variant, as it would for other tests. Instead, FDA has accepted novel strategies to demonstrate the analytical performance of NGS tests while maintaining appropriate oversight to protect patients.
- Because NGS tests can routinely identify variants that are shared by only a few individuals, traditional clinical studies establishing the link of such variants to disease are not feasible. Instead, the clinical performance of NGS tests will rest in many cases on the ability to aggregate evidence from many diverse sources.

Although the unique features of NGS tests create regulatory challenges, these same features also provide opportunities for novel solutions to regulatory oversight:

- The accumulation of data from NGS testing is enabling scientists, clinical labs, and regulators to better understand NGS outputs and error modes. NGS used in research and in diagnostic testing is generating a large amount of data that can be leveraged in further research, clinical trials, databases, and learning health systems to further evaluate the analytical and clinical performance of NGS tests.
- The large amount of cross-genome data generated by NGS tests could allow unique approaches, such as novel metrics and computational approaches, for assessing test performance.
- More generally, the cumulative generation of data through the increased use of NGS testing could help spur additional research in genomics and precision medicine.

The challenges and opportunities described above are now presenting themselves as realities because of the critical mass of genomic data that has been accumulated by researchers and clinicians. Thus, it is clear that new regulatory approaches will be needed to enable the Agency to provide appropriate oversight, in a way that is more suitable to the complexity and data-richness of this new technology, and to ensure that NGS tests have adequate analytical and clinical performance.

Recognizing the importance of NGS tests under the President's Precision Medicine Initiative, FDA is committed to developing a new approach for evaluating NGS tests. The work under the President's Precision Medicine Initiative builds off of efforts FDA has taken in the last several years to understand NGS technologies and to identify a regulatory framework that ensures safety and effectiveness while enabling innovation in the field.

Since 2011, FDA has hosted several public workshops examining various aspects of NGS, and has interacted extensively with scientists and other subject matter experts at conferences and in other professional venues. In addition, FDA personnel have also participated in developing standards and tools for the scientific community, such as the Next-Generation Sequencing: Standardization of Clinical Testing (Nex-StoCT) Workgroup and the Genome in a Bottle Consortium. These efforts helped to inform the essential elements of a new regulatory approach to NGS technologies.

²Here, "diagnosis" refers to the "diagnosis of disease and other conditions, including a determination of the state of health, in order to cure, mitigate, treat, or prevent disease" (21 *Code of Federal Regulations* 809.3(a)), and includes but is not limited to diagnosis, aid in diagnosis, prognosis, therapy selection/dosing, monitoring, and risk prediction.

In 2013, FDA cleared the first NGS instrument as well as two NGS tests for cystic fibrosis. In doing so, the Agency adapted its traditional regulatory approach to diagnostics. For instance, FDA was able to rely on a well-curated, shared database in assessing the validity of the 139 genetic variants involved in the assay, rather than requiring the test's manufacturer to independently generate data to support each variant's association with the disease. This not only reduced the burden for the manufacturer, it significantly improved the timeliness with which the product was able to be made available to clinicians and the public.

FDA now seeks to build on its successful past approaches to create an efficient and dynamic system for providing regulatory oversight of NGS tests. In December 2014, the Agency issued a discussion paper, *Optimizing FDA's regulatory oversight of next generation sequencing diagnostic tests—preliminary discussion paper*,³ to gain public feedback. The paper outlines new regulatory approaches under consideration for both analytical and clinical performance of NGS tests.

For analytical performance, the paper discusses an approach based on the development of quality-based standards⁴ for NGS test performance. These standards would be created in collaboration with the leading experts from the field of genomics. Conformance to such a standard could potentially provide assurance that an NGS test meets an acceptable level of performance, and that the results generated are reliable and accurate.

For clinical performance, the paper discusses the use of high-quality curated genetic databases that provide information on genetic variants and their association with disease to better establish the clinical performance of NGS tests by providing evidence about such associations and the strength of that evidence. As an example, NIH has created the ClinVar database, which houses information about genetic variants and their association with disease that has been shared by clinical laboratories, researchers, and other sources. Recently, NIH has funded external geneticists to curate entries in the ClinVar databases, under a program called ClinGen. FDA is now collaborating with NIH to understand how to use the curated data in ClinVar to support regulatory review of NGS tests. Use of curated databases, such as ClinVar, can provide a dynamic system for test developers to capture and update the clinical meaning of their tests, based on the latest evidence.

Both of these components—a standards-based approach to test performance and the use of community-generated evidence—could provide a dynamic and efficient regulatory system that could enable developers and users to seamlessly alter and improve their NGS tests as needed to advance the practice of precision medicine to benefit patients.

A key component of FDA's work under the President's Precision Medicine Initiative is to engage with stakeholders to inform any new regulatory approach adopted for NGS tests. Moreover, FDA is committed to drawing on the knowledge of the scientific community to help inform the Agency's approach to NGS oversight. Thus, the first action taken by FDA, after the launch of the Precision Medicine Initiative, was holding a public meeting in February 2015, with a broad range of stakeholders to discuss the regulatory approaches outlined in FDA's NGS discussion paper, and to hear experiences and ideas on NGS from the clinical and research communities. Nearly 1,000 individuals attended the meeting, and there was general consensus that an innovative regulatory approach was needed in order to balance NGS innovation and appropriate oversight. FDA is now reviewing feedback from the stakeholders to inform the development of more specific regulatory proposals that will be released for public comment.

As a first step to creating these proposals, FDA is meeting with the scientific community and other stakeholders to develop the standards, technical solutions, and best practices necessary to create a comprehensive proposal. In fiscal year 2015 and fiscal year 2016, FDA plans to issue additional white papers, and, if necessary, guidance, and convene further public workshops to work out the specifics.

To support this essential work, the President's fiscal year 2016 budget includes \$10 million to FDA to acquire additional expertise and advance the development of the regulatory structure needed to advance innovation in precision medicine and protect public health.

We now are entering a time of rapid scientific advancement with an eye toward precision medicine occurring in everyday clinical practice. For precision medicine to

³ Food and Drug Administration. *Optimizing FDA's regulatory oversight of next generation sequencing diagnostic tests—preliminary discussion paper*. <http://www.fda.gov/downloads/MedicalDevices/NewsEvents/WorkshopsConferences/UCM427869.pdf>.

⁴ Here, the term "standards" encompasses: metrics and tools that can assess the metrics, best practices, and more specific technical or other standards that would be developed by a recognized body.

fully succeed, our regulatory approach must be crafted in a manner that facilitates innovation, is sufficiently nimble to new scientific and technological advances, allows the public to have timely access to newly developed tests, and ensures that those tests are accurate, reliable, and clinically relevant.

Thank you for your continued interest in this important topic and for the opportunity to testify regarding FDA's contributions to progress on this issue. I am happy to answer any questions you may have.

The CHAIRMAN. Thank you, Dr. Shuren.

We'll now have a round of 5-minute questioning.

Dr. DeSalvo, in 1980, when I was Governor of Tennessee, I had the big idea that all eighth graders would become computer literate. I flew to San Francisco, met with Steve Jobs, and bought enough Mac computers—they were big tall things then—to put in all the middle schools. It was a great idea. It sounded good.

I forgot something. I forgot teacher training. Nobody really knew how to do it. I didn't think it all the way through the end. We have something of the same problem with our electronic healthcare record system. We spent \$28 billion. It's a great idea. It holds great promise. It's not working the way it's supposed to.

The current standards for meaningful use aren't clear. Upgrades are expensive. The systems don't work well enough to share the data. We hear it's expensive to share the data because of the relationships between vendors and doctors. Some of the doctors call this data blocking. You just released a report on data blocking describing these concerns.

Senator Murray and I have set up a working group to work on this because of the large amount of interest in our committee on the subject. My question is: Will you work with us, this committee, to identify the five or six steps we could take to get our electronic medical records system functioning well enough so that it supports not just the precision medicine effort that we have, but so that it functions and it's something that physicians and providers can look forward to using instead of enduring?

Dr. DESALVO. Yes, Senator. I very much look forward to working with you all on identifying ways that we can make this work for doctors and others on the ground, on the front lines, because that's where it really matters, where the workflows are sometimes not the way they ought to be. They can be clunky. We look forward to that, and you can count on our participation very actively.

We have some efforts underway. As the Senator is likely aware, we've been working through our rules for meaningful use, for certification, the blocking reported and other strategies. We know there's more work to be done, and we look forward to that.

The CHAIRMAN. Well, good. What I'm talking about here is actually beginning to get some results. I mean, identifying the five or six steps we should take—you should know them better than we, really, although Dr. Cassidy has some personal experience in this he will give us, I'm sure—and then going step by step to get them done. If you can do them by administrative order, terrific. If we need to do something, we'll include them as part of our innovation initiative and begin to do what we ought to do.

Dr. Collins, 1 million genomes—I mentioned I was visited by the head of Philadelphia's Children's Hospital. They have 250,000 sequenced genomes. I have two questions. Well, one question. How many of these are already out there? I mean, you want to assemble

a million. They've got 250,000 in Philadelphia. Dr. Venter wants to assemble a million.

Can you get your 1 million genomes simply by going to places like the Philadelphia Children's Hospital and using some of theirs?

Dr. COLLINS. That's a great question, Senator. We are, in fact, trying to come up with every possible way to assemble this million-strong cohort by taking advantage of things that have already been done instead of having to start from scratch. We will, in fact, have a major meeting in your State on May 27 and 28 with a number of those who have been managing these large-scale cohorts, gathering together to see if there is a way to put them together in a way that would prevent us from—

The CHAIRMAN. Can you estimate the number of genomes that have been sequenced?

Dr. COLLINS. I should be clear. When you say genome sequencing, some people are referring to a sampling of some of the base pairs in the genome, a genotype. A snip chip is the term that's often used. Some are talking about sequencing just the parts of the genome that code for protein, and we call that the exome, e-x-o-m-e.

To do a whole genome sequence is substantially more expensive but is becoming now quite affordable. Most of the cohorts that are out there have not yet done whole genome sequencing. The group in Philadelphia is doing that with some of their patients, but not with many of them. Dr. Venter certainly has the intention in his Human Longevity Institute of doing a lot of whole genome sequencing. We believe that to get the maximum information, you want that.

The CHAIRMAN. Let me ask you quickly—Senator Murray has emphasized the importance of making sure everyone or a representative group of everyone is included in this. What about children? I mean, the suggestion was made this morning that sequencing the genome of a child for a genetic defect leading to a particular disease can be less complicated than for an older person who may have a more complex disease. Will you include children and the single gene therapy treatment as part of what you do?

Dr. COLLINS. That is an active area of investigation by our working group. Let me explain that. We assembled a group of both public and private experts on this whole question of this million-strong cohort. They have met once, last week. They will be meeting again, specifically, to talk about what should be the constitution of this cohort. Should children be included? What should we do about individuals that may not otherwise be asked to participate?

We want to be sure this covers diversity of our population as well. Some of the cohorts that are already out there may not be as diverse as what we need. We will figure this out. There is a desire, however, Senator, to have this be something that represents the broad swath of our country. There will be a strong motivation for many people to include children.

The CHAIRMAN. Thank you.

Senator Murray.

Senator MURRAY. Well, thank you again to all of you for being with us.

Dr. Collins, we've heard a lot about how precision medicine is revolutionizing the practice of medicine, allowing for development of targeted cures for individuals. I'm also interested in the economic impact of this work.

We know that precision medicine is not only about treatment but about prevention, and we have a lot to learn about how factors like environment and nutrition impact individual health outcomes. How might these discoveries impact healthcare costs?

Dr. COLLINS. Well, I do appreciate that question, because we all agree that healthcare costs need to be brought under control, and a lot of the concern about our current system is that it is more a sick care system than it is a healthcare system. If we had the opportunity to focus more on prevention instead of waiting for illness to strike, we would both improve the health of the Nation and save money.

The Precision Medicine Initiative aims with this million-strong cohort to focus very intensively on prevention and to find out what actually works. I mentioned in my opening statement something about the fact that our annual physicals, which many of us sign up for, probably don't collect the kind of data that ultimately you'd like to have that might be a tip-off to something that needs attention.

The opportunity to begin to use many of these new tools, as well as these wearable sensors that are reporting on the environmental exposures and the body's performance under various situations, should put us in a much better situation to monitor individual health before an illness strikes. I don't want to over-promise the value that this will result in as far as cutting healthcare costs and bending that curve that we all want to see start downward again, because I think it's a longer term initiative. I would think over the course of time, this is one of the best opportunities we will have to cut our healthcare costs.

Senator MURRAY. You know, one of the exciting things about precision medicine is that it's empowering patients and people to participate and be full partners in discovery of new treatments. You mentioned the new mobile and wearable health technologies that are out there that allow researchers to collect data on how participants' behaviors impact their health outcome.

I know that all of your agencies are working hard to find new ways to engage patients in their own health and sustain participation among people that are involved in research studies. What are the best practices in patient engagement to ensure sustained participation throughout these precision medicine studies?

Dr. COLLINS. Another great question. Certainly, there are a number of cohorts that have already engaged a lot of patients that we are going to be consulting with about what their experience has been. Kaiser Permanente has a large cohort. The Mayo Clinic, Marshfield, has one, and Geisinger in Pennsylvania. All of these have done a lot of work to figure out what it is that people are looking for if they're going to participate.

An important part of what we're trying with this initiative is not to think of the individuals who take part as patients. They're really partners. They're participants. We want to have them at the table. We will have a workshop July 1st and 2d which is focused specifi-

cally on trying to get input from individuals about what they're looking for.

What we could already say is that people expect, if they're going to be part of this, that their information is going to help people. There's a lot of altruism involved in taking part.

They also would like to get information back about themselves, in terms of what's been learned about their own state of health and what has this study led to in terms of broader discoveries that might not have happened otherwise. They want to be included. They want to be informed. They want to be at the table. We promise that is the attitude we will bring to this.

Senator MURRAY. Very good.

Let me ask you, Dr. DeSalvo—we've seen several high-profile sophisticated attacks on healthcare organizations in the last few months. Americans expect that healthcare providers and researchers are taking the necessary precautions to protect their data. That's why I mentioned working with Senator Alexander on the current State of cyber security in the healthcare industry.

Can you tell us what steps ONC is taking to help researchers keep the large amount of genetic and other health information that they are collecting secure?

Dr. DESALVO. Thank you for the question, Senator. We agree with you. It's a major issue. It's something that's on top of our mind every day. The steps we have taken most recently, for example, are to require in the electronic health records that data is encrypted at rest and in motion so as you move to interoperability and data is moving across systems it also needs to be secure and encrypted.

We are working with, for example, the Department of Homeland Security, with the National Security Council and others to ramp up the additional security expectations, because, again, as data begins to move and be more liquid, there's more opportunity for there to be security issues. It's a top priority. We have taken some actions, and we have some additional ones that are underway.

Senator MURRAY. Dr. Collins, what is NIH doing to protect patient privacy?

Dr. COLLINS. We've already initiated a genome data sharing policy which has been in place for several years, because we have been conducting studies, as you might know, to try to understand genetic contributions to diseases like Alzheimer's or schizophrenia or heart disease. The conditions under which then that data can be shared is rather carefully overseen.

Qualified researchers can apply to see that data, because we think much is gained by having it accessible. It has to be overseen in a way to make sure that the individuals who are looking at the data are appropriately signing on to various restrictions, such as not sharing it with third parties and acknowledging where it came from. That has been very successful over several years. I think we have a pretty good framework there.

We do think there are some things that are needed in order to protect genetic privacy and make sure that it is not acquired by individuals who do not have the right to do so, that there ought to be something to avoid surreptitious genetic testing of individuals without their consent.

Senator MURRAY. Thank you very much.

Thank you, Mr. Chairman.

The CHAIRMAN. Thank you, Senator Murray.

Senator Cassidy has deferred to Senator Hatch, and then Senator Franken.

STATEMENT OF SENATOR HATCH

Senator HATCH. Well, thank you, Mr. Chairman.

Thanks to all of you. We appreciate the work that you're doing. We've been following it for many years and really appreciate it. In Utah, we have a large database, too, and I wonder if that could be part of the million-person cohort, because the Utah population database is the world's largest repository of computerized family histories, and it's linked with more than 22 million public health and clinical records.

Scientists at the University of Utah have been able to use this non-commercial resource to identify dozens of genes responsible for diseases. The Utah Genome Project is harnessing the power of Utah's large families to discover new disease-causing genes that underlie conditions such as diabetes, heart disease, obesity, and cancer.

These large families accelerate the pace of genetic discovery by magnifying our ability to identify disease-causing genes. By harnessing the advantages gained through using these large families and large cohorts, our folks in Utah can make significant contributions to what you're trying to do here. I would just like to know if you think they would be useful, and if I can play a role in getting the University of Utah and you to work together.

Dr. COLLINS. We are, indeed, and, Senator, thank you for the question. Utah has been in a wonderful place as far as the ability to do remarkable research in human genetics over many decades, research that I've personally benefited from collaborating with over decades of my own research career. You're right. You have an unprecedented level of depth in terms of family collections.

One of the things that we are going to be wrestling with a bit in terms of this cohort is exactly what ought to be the involvement of multigenerational pedigrees. It does bring considerable strength to the effort, and that will be a topic of discussion also at this workshop later this month in Nashville.

I would say that the Intermountain Healthcare system, which obviously involves lots of folks in Utah—I should have mentioned it on my list a minute ago of those that have already generated cohorts. They have a very strong presence in this as well. I'm quite sure when the dust all settles, this Precision Medicine Initiative will have a Utah connection.

I saw that wonderful piece this morning in the Deseret News talking about some of this, and I think there's a lot of excitement across the scientific community and across the country about what this might lead to. I appreciate you volunteering to help us.

Senator HATCH. Well, thank you so much, and we do want to help you. The University of Utah has a genetics department about as good as anybody can have. In fact, we've irritated Harvard to death by enticing a number of very top researchers to Utah. They like the mountains and the skiing as much as anything, but they

wouldn't leave once they get there. We'd love to be of great assistance to you.

I, personally, appreciate all three of you. I know a little bit about what each of you do, and I've taken a great interest in what you do over these many years. As I've been chairman of the Finance Committee, I haven't had as much time to spend in this committee, which I used to chair.

I just want to compliment the distinguished chairman and Ranking Member here for the good work that they're doing. They're terrific leaders in the U.S. Senate, and I just want to personally express that.

Thank you. I appreciate all three of you being here, and I appreciate the work you're doing.

Dr. COLLINS. Thank you, Senator.

Senator HATCH. Thank you.

The CHAIRMAN. Thank you, Senator Hatch.

I almost had to give Senator Warren equal time there, but she'll have—just so you'll know, the order I'm calling on Senators is based on who was here at the time the gavel went down. Based on that, on the Republican side, Cassidy and Collins are next, and on the Democratic side, it's Franken, Bennet, Warren, and Murphy.

Senator Franken.

STATEMENT OF SENATOR FRANKEN

Senator FRANKEN. Thank you, Mr. Chairman, and thank you to the Ranking Member for holding this hearing. Precision medicine is extremely exciting.

Those of us in Minnesota thank you, Dr. Collins, because Mayo and the University of Minnesota have been doing a genomics project funded by you—so very smart on your part.

This is exciting, because there's really been a paradigm shift in the way we think of healthcare in this country. In some part, due to the healthcare reform law, healthcare providers and insurers are moving more toward person-centered care, and I'm talking about coordinated care, medical homes, ACOs that provide incentives and information that help doctors tailor their practice, their treatments, their therapies to meet the needs of individual patients.

That's, of course, what precision medicine is all about, making sure the right patients get the right treatment. The right treatment doesn't, as you were saying, Dr. Collins—doesn't necessarily mean—it isn't treating people when they're sick. It's about healthcare, not sick care, and using personalized medicine should improve prevention, so that we are doing healthcare and not sick care.

Dr. DeSalvo, I do want to talk to you about what you said, which is we're at a tipping point in the adoption of medical health records and electronic health records. I just want to talk about that, because we had a hearing on that not so long ago, and there are some barriers to adoption by certain medical providers, some resistance, some because of doctors who feel like "I've got 20 minutes with this patient, and I don't want to spend eight of it inputting data."

On the other hand, this is where we're going. What are you doing to address that, and what are some good models? I've heard of things like having a scribe whose job—like a medical student who

is there with you and who is taking down the information. We need to get there. What are we doing to get there?

Dr. DESALVO. Well, Senator, thank you for raising the voice of a lot of doctors in this country. I hear similar things when I travel and talk and from my own family members, including my husband, that there are—it's been a great advancement. We're going forward. Folks want to go there. However, the systems are not a part of the workflow in the way that we want or expect in clinical practice.

The opportunities there include giving more time for providers to be able to implement the systems on the front line. The Senator may be aware that in the last year, we have put forward some rules with CMS to provide additional flexibility in timing of the adoption of records or upgrading to new ones and also to propose in this last set of rules giving doctors the option of a more streamlined approach to the kinds of ways that they have to report, so reducing the burden or the expectation on the amount of clicks that they must do to show that they're functionally using the records.

We are working toward a goal of a shared expectation that this is going to be an enabler and really support them, and I'm committed to continuing in that path.

Your point about successes on the ground and tools that doctors have used in their office practice and otherwise—is it really important we collect those—there are health IT fellows program, as an example—and share those. It varies by doc and by office what's going to work for them—sometimes using a dictation system and then having some assistants to transcribe over it. Sometimes it's a scribe.

Sometimes working with the electronic health record, they have had so much time that they've been able to make the systems as seamless as possible for them. A State like Minnesota is so far advanced in health IT, as I'm sure you're aware, and has had many more years in to make sure the systems are working. We're not finished with—

Senator FRANKEN. Thank you. I don't mean to interrupt you.

Dr. DESALVO. Yes. I'm sorry.

Senator FRANKEN. We can talk about this for a long time, but I want to get one quick question to Dr. Shuren.

I have a question about how the tests at FDA—those tests that the FDA is going to be assessing. One thing that concerns me is that some tests may get quite expensive, and I want to make sure that I understand how they and the highly personalized care that precision medicine can provide will benefit everyone and won't contribute to health disparities in our country.

My questions are: Will these tests be considered diagnostic tests or preventative tests? Who is going to be paying for them?

Dr. SHUREN. Well, in the case of next-generation sequencing, they can be used potentially for both diagnosis and for predicting and, therefore, prevention purposes. It all depends upon—do you have the data to show that that particular test can perform in such a way.

In terms of reducing cost, there's the opportunity for reducing cost for those technologies to be developed, because if we have the standards I talked about, and we have those databases of informa-

tion, it will be a lot less expensive to have the science. In the past, you do a clinical study to show if your test actually predicts or diagnoses that disease. With the databases, you might be able to point to that data. Essentially, the clinical community is crowd sourcing the evidence.

We just did that recently with—two years ago with a test for cystic fibrosis, where first, we approved that NGS test based upon a subset of variants. And, second, they were able to use data in a database at Johns Hopkins that was supported by the Cystic Fibrosis Foundation and didn't have to do a clinical study, dramatically reducing the cost of bringing that test to market.

Who pays for it? Hopefully, the insurers will pay for it at the end of the day, because if you have good technology, it's of no value to patients if they don't have access to it. If they can't afford it, they won't have access.

Senator FRANKEN. There's no question that precision medicine can bring down our costs, and, certainly, as I just don't—I worry about a brave new world where certain people have access to certain things. I'm out of time. I would just ask unanimous consent to submit a statement from Senator Klobuchar to the hearing record.

The CHAIRMAN. Of course. It will be done. Thank you, Senator Franken.

[The information referred to may be found in Additional Materials.]

Senator Cassidy.

STATEMENT OF SENATOR CASSIDY

Senator CASSIDY. Dr. DeSalvo, we've had a GAO report in the past that the VA and the DOD needed to do much better to coordinate their records. Clearly, electronic medical records are critical to precision medicine, both the research thereof and the implementation.

I hear that there's an \$11 billion DOD contract going out, and I'm not quite sure that it's coordinating with the VA. We had testimony recently about how there's a lack of interoperability, so one of the systems being considered or two of the systems being considered by DOD are those mentioned as lacking interoperability.

I feel like we're in a thicket here, and we can't get out. All we know is that we're about to spend \$11 billion on something that the VA system is not interoperable with. Please tell me that I'm absolutely wrong.

Dr. DESALVO. Senator, the DOD's acquisition of a new electronic health record—you are correct—is one of the most important things that's going to happen on the health IT landscape, and we are intimately involved in that. The Department of Defense, for example, has embedded staff with us at ONC to see that we're communicating. The Department of Defense has agreed to lead the way in pointing to the standards.

The Senator asked me earlier what are the steps we should take. If I could, just for a second—

Senator CASSIDY. I only have 3½ minutes.

Dr. DESALVO. One of the most important things is to move away from proprietary standards, which is getting in the way of the systems being able to—

Senator CASSIDY. There are some open source bidders—Epic is one of them. So is Cerner. They are not open source. Correct?

Dr. DESALVO. Those vendors will have to agree to use the standards that the Department of Defense wants to use, which are the ones that ONC has published. We are very pleased that we're all moving in a direction to have a core set of standards that everyone will agree to so that we don't run into a problem where the system is not interoperable.

Senator CASSIDY. So you're telling us that the VA will be able to share records or that the local hospital will be able to share records with the DOD?

Dr. DESALVO. The VA and the DOD is a separate issue, because they have a different kind of technology. Yes, sir, the goal is that that becomes not only exchange, but interoperability.

Senator CASSIDY. Again, I talk to medical students all the time, and I don't mean to offend. When you mention the goal, I accept that it is a goal, but how likely is it to happen? Because it seems like you left some wiggle room, that, indeed, the VA has a different system and it may not yet communicate with DOD.

Dr. DESALVO. What the VA and the DOD have done now is they have found a solution to exchange information. If you're at the bedside with the patient, you can see the records from the VA and DOD. So they've taken that first step.

The integrating of the data requires having the same core data elements, so there's a technology issue, which is solvable. There is also a policy and a culture issue, which, honestly, is generally the harder one and what we're facing in circumstances like information blocking which is one of the things getting in the way of interoperability in the broader community.

Senator CASSIDY. You spoke of the open source, though. That seems kind of, by definition, not to include information blocking. Who is blocking the info?

Dr. DESALVO. So information blocking can happen sometimes from technology, but what we're seeing commonly is that the vendor systems will charge—

Senator CASSIDY. I accept that. We've had those hearings about how the vendors are blocking. I'm going to take it back to where we started. My fear is that the very vendors who are blocking data are the ones bidding. Then you mentioned it's going to be open source, but you returned to the fact that there could be blocking. I guess I'm not clear.

Will the final \$11 billion project be something that I, at Our Lady of the Lake in Baton Rouge, can access data? Or will there be a problem with vendor blocking?

Dr. DESALVO. I would need the DOD to confirm the answer, if you would. However, what I would share with you is that since we described blocking and since we put out the report, the vendors have begun to pull down the fees to make this problem start to go away, and we have to keep putting on the pressure. I do not think the work is done.

Senator CASSIDY. I apologize, and I'm already over time. If I can just ask one—no, I'm almost out of time. Is it part of the initial RFP that they cannot block, and that the VA has to be able to share, as does the community hospital? Let me ask it that way.

Dr. DESALVO. That is the intention of the DOD. Yes, that is correct.

Senator CASSIDY. It is the intention, but is it part of the RFP?

Dr. DESALVO. Yes. That's where they're going. That is what I understand from our—what we have recommended as ONC to the DOD. I would have to defer to the DOD.

Senator CASSIDY. Can you? Because I sometimes find that recommendations are not adopted. I find that commonly.

Dr. DESALVO. We can certainly get back with you on that.

Senator CASSIDY. Yes. I'm almost out of time. I yield back.

Dr. DESALVO. Thank you.

The CHAIRMAN. Thank you, Senator Cassidy.

Senator Bennet.

STATEMENT OF SENATOR BENNET

Senator BENNET. Thank you, Mr. Chairman.

I thank the witnesses for your testimony. You're fortunate to be on the cutting edge of all this stuff.

Dr. Shuren, as I know you know, there is a thriving movement of innovation in molecular diagnostics underway, thanks to the Human Genome Project and investment over the past decade. There are a number of Colorado companies like Corgenix and SomaLogic and Biodesix, which are developing remarkable new advanced diagnostics in areas like Ebola, cardiovascular disease, and lung cancer.

The FDA recently released a draft framework to regulate lab-developed tests. As I wrote in a letter to you a couple of weeks ago, as always, we need to balance both innovation and safety to ensure that we create a fair and workable process. There's some concern that the draft framework could require the FDA to register and approve thousands of labs or, at a minimum, thousands of tests.

I just wonder if you could speak to this a little bit. Given the size and scope of the issue, do you intend to propose more formal regulations in this space? Are you open to congressional action here? Would that be useful? How do we get a handle on this and create predictability for the people that are doing this work?

Dr. SHUREN. Well, first, let me say that laboratory-developed tests play an important role in our healthcare system today, and our goal here is not shutting down laboratory-developed tests, but, in fact, making sure that we are both facilitating innovation and that those tests are accurate, reliable, and clinically meaningful. Under our proposal, we tried to strike that balance.

I don't know that we'll actually receive thousands of tests, because what we've heard from the lab community is that a lot of the tests they make are to address unmet needs. One of the things we put out in our proposal is to say,

“Look, if you're making a true laboratory-developed test, your healthcare facility, healthcare system, is doing this and treating patients, and there isn't a test out there like that that

FDA has approved or cleared, you don't come in the door for premarket review. We'll address that unmet need."

If, subsequently, someone has that test, and they send us the data, and we look at it, and it turns out we know this test works, then our expectation is other people who are making that test should do the same, because we now have data that that test, in fact, is accurate, reliable, and clinically meaningful. I don't know that we will actually receive thousands.

That said, we received a lot of comments on the proposal. We're working on it, and we will be making changes before we have the final policy.

Senator BENNET. If there is legislative work that needs to be done around this, I hope you'll let the committee know. I'm sure that you will.

I want to turn also to one other topic around innovation. Dr. Collins mentioned earlier how important mobile technologies have become. Really, in the blink of an eye, this is all changing the way doctors practice medicine and patients monitor their own well-being.

As you know, probably, Senator Hatch and I reintroduced the Med Tech Act yesterday to ensure that lower-risk medical software and mobile apps are not regulated by the FDA. I think we share the same goals on this, and I want to thank your team for giving us technical advice all the way through.

Can you talk a little bit about FDA's thinking in this area?

Dr. SHUREN. Well, first, let me thank you and Senator Hatch and your staffs for the opportunity to work together on the Med Tech Act. We agree—as we looked at this space, we were looking at functions, device functions, that we had been regulating for a long time, and now some of them are being put on mobile platforms.

What we found as we looked at it is that some of these lower risk functions we may better serve by no longer actively regulating them and spur a little bit more innovation. They're sufficiently low-risk. We don't need to provide that additional FDA oversight. Instead, focus on higher-risk medical device functions in this space, and that is kind of a nice balance on that—facilitate innovation, but still assure good patient safety.

Senator BENNET. Thank you.

I don't know, Dr. Collins, if you have anything you'd want to add.

Dr. COLLINS. Well, only that your point about mobile health technologies is extremely well-taken. The proliferation of really exciting opportunities is happening all around us, and we certainly see this Precision Medicine Initiative as a great opportunity to test those out, because you not only want to have an application that's kind of cool and gives you interesting information, but you want to know does it actually improve health and does it change outcomes.

If we have a million individuals who are excited about participating in research, who are essentially volunteering to become users of these kinds of technologies, whether it's the next version of a watch that measures all kinds of aspects of your body's physiology or something that's detecting in the air around you what kind of exposures you're having, this would be a great opportunity to find out what works, what actually improves healthcare, and then

what ought to then be extrapolated and utilized across medical care for the whole country.

Senator BENNET. Thank you.

Thank you, Mr. Chairman.

The CHAIRMAN. Thank you, Senator Bennet.

Senator Collins.

STATEMENT OF SENATOR COLLINS

Senator COLLINS. Thank you very much, Mr. Chairman.

Dr. Collins, just this morning, I met with some advocates from Maine who are pushing for more research into brain cancers such as glioblastoma. You mentioned in your written testimony that oncology is the clear choice for enhancing the near-term impact of precision medicine and that important advances have already been made in this area. I very much look forward to sharing your testimony with this group of people from Maine who are concerned about such devastating brain cancers as glioblastoma.

I'm wondering if you also see a role for precision medicine in neurodegenerative diseases, like Alzheimer's, Parkinson's, and ALS. Are investments in these important areas also being considered as part of the Precision Medicine Initiative?

Dr. COLLINS. Thank you, Senator Collins, for the question. Absolutely. We are learning that disorders like Parkinson's and Alzheimer's and other neurodegenerative conditions do, in fact, have multiple contributions to whether they happen or not to a given individual.

For Alzheimer's disease, we now know of 35 individual places in the genome where variations place an individual at higher risk. We know about one or two where individual variations actually are protective, which is an even more potentially actionable finding, because you'd like to understand that in order to develop the next generation of preventive strategies for people who weren't so lucky as to inherit that preventive kind of genetic variation.

There is, especially for any disease that is common enough that you're going to have thousands of individuals in your 1-million-strong cohort, an opportunity to study those at a scale that has not previously been possible and to try to put together all of the things we can learn about their genetic inheritance using whole genome sequencing.

Also, their environmental exposures, also everything we can learn about their electronic health record experience, also using mobile health are the ways that we can come up with better detection systems of early trouble in terms of cognitive changes. That is very much an intention of this.

One of the exciting aspects of having this very large cohort is that it doesn't have to be just about heart disease or just about diabetes or just about Alzheimer's disease. It can be about all of those things, because it will have the scale to do so.

We have waited a long time to reach the point where the technology would make that possible, and the time is now. We've really reached a remarkable inflection point in the potential of medical research, and we should not let this moment pass.

Senator COLLINS. I could not agree more. Truly, it's so exciting.

Dr. DeSalvo, despite the Federal support that you mentioned in your testimony, I continue to hear from smaller healthcare providers about the barriers that they face with electronic health record implementation. Just yesterday, I met with a physician from Bangor, ME, who shared with me that putting in place a comprehensive electronic data collection system for his small practice was going to cost in excess of \$230,000. This was just for the software, not for the hardware. That's no small amount, particularly for a smaller, independent practice that is not hospital-owned.

To access information about individuals to improve diagnoses, treatment, and prevention of diseases, you discussed the important role, the absolutely essential role, of health information technology and interoperability, including the standards and technology that are going to be needed. As you work to build these health IT systems for precision medicine, how can we assure that we're not leaving out rural America, smaller practices, rural hospitals, health clinics, because of the cost?

Dr. DESALVO. Senator Collins, thank you for the question and for particularly spotlighting one of the challenges of small practices and rural. As the Senator may be aware, that was a particular focus that we had early on in the HITECH funding. That was prior to me joining the Administration, but that team really wanted to see that rural America was not left behind, and there was great success, actually, in the adoption in many of those communities across the country in partnership with USDA, as an example.

They are facing now a challenge of upgrading technology, and it is one of the reasons that last year, because of some challenges they were having, we put out this flexibility rule that gave them some more time to be able to advance. The cost that you're describing for that physician sounds fairly exorbitant, and I would be very happy to followup with your staff and that physician to see if we can understand what is happening there, and perhaps the regional extension centers that are in your communities could followup.

You are exactly correct. It's critical that we get this. It's critical that nobody is left behind and that we find a way to make it successful for everyone.

Senator COLLINS. Thank you very much. I'm going to take you up on that offer. Thank you.

Thank you, Mr. Chairman.

The CHAIRMAN. Thank you, Senator Collins.

Senator Warren.

STATEMENT OF SENATOR WARREN

Senator WARREN. Thank you, Mr. Chairman. The President's Precision Medicine Initiative could be a big step forward for more targeted and more effective therapies for any number of conditions, and I think it's a great idea. We should have started years ago.

Dr. Collins, you first advocated for a national genetic study to examine how people's genes and environments contribute to diseases over a decade ago, in 2004. Is that right?

Dr. COLLINS. In fact, that is exactly right, and it landed with a thud at that point. That's actually the article up there on the screen that I published in 2004. In retrospect, this was probably a bit ahead of its time because we didn't have the technology at the

point where this would have been affordable or practical. But it is now.

Senator WARREN. I'm glad to hear that it is now, although if we had started pushing and funding back then, we can only wonder how much further we would be ahead right now. Congress didn't make those investments, and, in fact, over the past decade, NIH funding hasn't even kept pace with inflation. That means we are years behind in doing this work.

If we are serious about speeding up biomedical innovation, about improving health, about reducing long-term costs, we start by investing in NIH. The House has a proposal called 21st Century Cures that's supposed to accelerate biomedical innovation. When it was first released by the Republicans a few months ago, it didn't include a single dime of new NIH funding for Congress.

Last week's new bipartisan draft of this bill very much seems to be moving in the right direction. It has \$2 billion in new mandatory funding for the NIH every year for 5 years. I applaud the House Republicans for acknowledging what so many of us, including Newt Gingrich and the drug industry, have been saying for years. NIH funding is critical to accelerating cures.

Let's be clear. A few billion dollars in temporary funding will not solve a decade of neglect, much less build the future that we need.

Dr. COLLINS, in the late 1990s, Congress doubled the budget of NIH, and then agency funding was left to shrink back down. If Congress had never doubled the budget of NIH and had simply kept pace with prior investments, where would the NIH budget be today?

Dr. COLLINS. Well, Senator, I keep a graph in front of me all the time about this very question, and I'll just put it up on the screen, because this is a documentation of the problems that we are now facing. What you're seeing on that screen there—the yellow line is basically what NIH has had as far as our purchasing power for research, so it's the appropriation, but as adjusted by the effects of inflation.

The dotted green line is the trajectory that NIH was on going back to 1970 until 1998 when we had that wonderful doubling. Then we've been getting undoubled ever since. If you follow the dotted green line, and we had stayed on that smoother trajectory, we would be substantially higher, up in the neighborhood of a little over \$40 billion.

Senator WARREN. Just to get back on track and to reverse the damage of the last decade, NIH, if I'm reading this right, would need more than \$12 billion in just the first year, and the House proposal doesn't even put that much in over the space of 5 years. Let me just ask, based on what you've got here, in your expert judgment, what's the annual rate of increase that NIH needs to get back on track on its funding?

Dr. COLLINS. Well, first, let me say we were thrilled also to see what's in the 21st Century Cures. The \$2 billion a year of mandatory gave a great jolt of excitement and some relief to a community that's been really quite stressed over the past 12 years as we've been losing ground.

To get back on a stable trajectory that would result in a healthy biomedical research ecosystem which our country has depended on

with great success over 50 years, I would estimate, in my professional judgment, that we need to be in the space of inflation plus 4 percent or 5 percent per year. That's pretty much—that dotted line was inflation plus 3.7 percent, I guess. That was a healthy way to be sure that all the talent and capabilities of this country, in terms of biomedical research, where we have led the world for decades, could be sustained, encouraged, and innovation could go forth in all the ways that we want it to.

Senator WARREN. Well, thank you, Dr. Collins. Two billion dollars a year for 5 years is certainly better than nothing. Let's not pretend that a small, temporary investment that falls billions of dollars short of what we're going to need will do the job. There is a gaping hole in our NIH budget, and we need a serious plan to fix it.

There are many ways to make that happen. I have a Medical Innovation Act, for example, that could add another \$6 billion a year—wouldn't cost taxpayers a dime. But, whatever we do, this committee has to get serious about medical innovation, and that means we have to do better than the House proposal on this.

Thank you, Mr. Chairman.

The CHAIRMAN. Thank you, Senator Warren.
Senator Whitehouse.

STATEMENT OF SENATOR WHITEHOUSE

Senator WHITEHOUSE. Thank you, Mr. Chairman.

Let me start by echoing the chairman's interest in having a review of where we are on health information technology. I read with interest the *Wall Street Journal* piece by, Dr. DeSalvo, your predecessor, David Brailer, and it makes a lot of sense and provides, I hope, some bipartisan foundation for us to work forward.

I think that the meaningful use program has become obsolete and needs to be tuned up to meet the new challenges that the progress over the last years since it was passed now present to us. Thank you for agreeing to work with the chairman and the committee on the four or five key goals that we should be achieving, and I would urge you to think big in accomplishing that. Let's not twiddle around the edges. Let's get this right.

Dr. Collins, to followup a little bit on what Senator Warren was saying, I'm interested in what you've been able to document by way of consequences for failures to adequately fund our scientific and medical research. It strikes me that you could probably tell me that there's a return on investment from the research that we do, and if we don't fund the research, we lose that return on investment.

It strikes me also that you probably have examples of human benefits from the scientific research which, if foregone, become human costs. You probably also pay some attention to the country's global competitiveness in this field.

Could you comment specifically in those three areas on what you think is the payback for investment in scientific research, or, if you want to put it contrarily, the cost of not funding scientific research?

Dr. COLLINS. Well, I very much appreciate the opportunity to talk about some of those consequences. I'll just put up another graph which, in many ways, reveals the difficulties that are present in this country, beginning back in 2003. What I'm showing

you there is the opportunity that an investigator who comes to NIH with their best ideas has of actually getting funded.

Most biomedical research done in this country in our Nation's finest universities and institutes is supported by NIH. This is the main place where this work gets done. For most of our history, that has been in the space of 25 percent to 35 percent success rate, about one in three. That's not easy. That means two-thirds of the people are sent away. Now it's about one in six, and that's very unhealthy.

We have looked at what happened in the past when we could fund up to 30 percent, and there's a lot of great science that falls in that space between the 16th percentile and the 30th percentile, and we're not funding that now. About half of what we should be supporting by historical trends is left on the table.

We don't know what we're missing in that regard. The next great idea about cancer may have been one of those things that didn't quite make the cut.

In terms of your question about medical consequences, I'm a physician. The reason I love being at NIH and love what we do is the hope that this is going to change things for the better for people's health. Our track record there is striking in terms of what's happened in terms of longevity and prevention of disease. It is frustrating that we are going more slowly.

I promise you that the institute directors and I, when we sit around the table and try to figure out what to do in these constrained circumstances, we still prioritize, we still try to push forward, but we're just going more slowly. We need more advances in cancer. We need a universal influenza vaccine. We're working on all those things, but we could be going faster than we are right now.

In terms of the financial return on investment, that's been documented over and over again. A dollar of NIH grant money returns about \$2.20 in the first year to the local economy because of the goods and services that are generated as a result. We support about 400,000 jobs directly across the country in all 50 States on the basis of the grants that we give out. Those are high-quality jobs.

You asked about global competitiveness. We were the unquestioned leader of the world in biomedical research until recently. That is no longer to be taken for granted. When you see us losing ground, and we see countries like China and India and Singapore and South Korea upping their investment sometimes in double digits, we are losing that leadership.

Senator WHITEHOUSE. A quick question on that. When they are making those increases in investments, do they have an eye on us as a target—

Dr. COLLINS. You bet.

Senator WHITEHOUSE [continuing]. Or are they just doing this in a sort of a general eleemosynary way?

Dr. COLLINS. Well, it's a little of both. They basically read our playbook from 20 years ago, and they saw what it did for America's economy and for the spinning off of small businesses that come out of this effort, and they want to do what we did. I don't know if

you'd say they're gunning for us, but they're basically trying to learn from our experience and recreate that in their environment.

One statistic that particularly renders this very serious—last year, China filed more patents in bioscience than the United States did. That was not even a close competition a couple of years ago, and they have now jumped ahead of us. Those patents result in intellectual property claims that are going to ultimately spin off new businesses. We have to take that more seriously.

Senator WHITEHOUSE. Thank you, Chairman.

The CHAIRMAN. Does that mean China will start respecting patents more?

Dr. COLLINS. I'd better not comment on that.

Senator WHITEHOUSE. Don't get the man in trouble.

[Laughter.]

The CHAIRMAN. Thank you, Senator Whitehouse.

Senator Baldwin.

STATEMENT OF SENATOR BALDWIN

Senator BALDWIN. Thank you, Mr. Chairman, and I very much appreciate you and the Ranking Member holding this hearing, giving us the opportunity to learn more about the administration's Precision Medicine Initiative. We're excited about it because of its lifesaving potential. We're excited about it because of its breakthrough potential.

I'm excited about it, having the honor of representing a State that's been a leader in setting the stage for some of the things we're talking about today in precision medicine, from the isolation of the first embryonic stem cells to the discovery of short tandem repeat polymorphisms at Marshfield Clinic, which is a major discovery that has had a big impact on the study of human genetics. The clinic has since developed a very significant genetic biobank, one of the larger ones with information from over 20,000 central Wisconsin residents.

Dr. Collins, I know you've been asked sort of iterations of this question before by my colleagues on the committee. If you have more to add, I would like to hear more about how you will utilize the existing data, like the data that I just described that was collected by the Marshfield Clinic and real-world clinical data. How will you use those and share those in new ways to create personalized therapies?

Dr. COLLINS. That's a great question. Yes, Marshfield is a wonderful leader in this enterprise. I visited there myself several times, and Dr. Murray Brilliant, who's the person there who's overseeing their large cohort, their precision medicine effort, is somebody that we are all looking to for his experience to share with us. He was at the White House when the President announced this on January 30.

In this workshop we're going to hold at the end of this month at Vanderbilt, we will really look hard at the ways in which Marshfield, Mayo, Intermountain, Kaiser Permanente, Geisinger, and perhaps the Million Veterans Project as well, could, in fact, be assembled into a synthetic kind of cohort, not having to do all of the work from scratch, but making the whole greater than the sum of the parts, because this kind of initiative really builds power by

numbers, and that's one of the reasons we're so excited about being able to say that word, million, which would not have been in the vocabulary of most people planning these things until fairly recently.

We want to take every kind of opportunity to build on the experience that's already been obtained in places like Marshfield. One of the things they've done, by the way, is they found individuals who, by looking at their DNA sequence, ought to be sick, but they're not.

Senator BALDWIN. Right.

Dr. COLLINS. They have some kind resilience. Some people call them genetic heroes. There's something about them that we need to understand, because they have that resistance to disease that we perhaps could learn more about and figure out how to share with other people by development of new therapeutics. That's just one kind of insight that they've got a start on, but if you had a million people, you could find a lot more.

Senator BALDWIN. I want to followup on a discussion that you were just having about the research workforce. You were talking about funding and the reducing percentage of research grant applications that are actually funded. I'm curious to know what impact this initiative may have on the changing nature of the research workforce.

It strikes me that there are doctors who happened on discoveries of novel therapies in the course of treating patients, but others may not know that their patients' unique reaction to a treatment holds the potential for a breakthrough in this field. What opportunities for new and nontraditional researchers are presented through this initiative?

Dr. COLLINS. Oh, Senator, that's another great question, and I'm glad you brought it up. This Precision Medicine Initiative will not reach its full potential if it doesn't lure and recruit all kinds of people from different disciplines to get together to work on this. I think of a parallel here with the Human Genome Project that I had the privilege of leading, where it was such a historic opportunity that people who never really thought of themselves as working in that space decided to make it their passion.

The same can happen here. We would want to have computational experts, because we have very large datasets. This is the world of big data and the best way. We would want to have technology developers of all sorts who can figure out ways not only to look at your DNA sequence, but what about all of those metabolites that are floating around in your system. We can begin to look at hundreds of those in a given situation.

All the technologies we mentioned about mobile health—there's still lots of opportunities there as well, and physicians who can begin to figure out how do you take this kind of data and implement that in a real world setting in order to improve health outcomes.

You know, I have to say when I look at the way we practice medicine today compared to when I was a resident in medicine in 1979 or 1980, it's not that different. We have such a long way to go here in terms of really incorporating all the new technologies that are coming along.

This is going to be a wonderful laboratory for all kinds of people to get involved in and figure out what kind of discoveries can be made and what use we can put them to. I hope it's going to be filled with that kind of innovative talent.

Senator BALDWIN. I appreciate that.

Mr. Chairman, with your indulgence, one quick question.

Dr. Shuren, you can answer this for the record. There's incredible potential in what we're talking about. It also strikes me that there's incredible potential for fraud, for folks, as this develops, offering and selling fraudulent interventions that claim to be personalized medicine. I would like—and, again, it can be after this hearing—to hear from you more about how the FDA will work to prevent fraud and ensure patient safety in the age of precision medicine.

Dr. SHUREN. I'll take a quick moment, if it's OK, just to say—

The CHAIRMAN. Sure. Senator Murray and I would like to know the answer, too.

Dr. SHUREN. All right. Well, thinking even just beyond fraud, the whole point of having a test out there that really doesn't work and it's being sold—this is one of the impetuses in terms of our now deciding to actively regulate that subset of in vitro diagnostics laboratory-developed tests, because there are some great tests out there, but there are some bad tests out there.

Let me give you an example of one that's in the precision medicine space—and there are several—something called KIF6, and it's used for predicting people—for people having coronary heart disease and their response to statin treatment. This is a test where we had seen data on it. We saw the test didn't work. Laboratory-developed tests—they're selling it.

There was a meta-analysis of 19 clinical studies. The test doesn't work. Then they performed a randomized placebo control trial on over 18,000 patients. The test doesn't work. When this was reported out around 2010, at that point, over 150,000 people got that test. We estimate the cost to the healthcare system may have been upwards of \$2.4 billion, and that test is still available today.

That doesn't serve patients well, and it doesn't serve precision medicine, because it undermines our efforts to make sure that we get accurate and reliable clinically meaningful tests out there, and we get the right treatment to the right patient.

The CHAIRMAN. Thank you, Senator Baldwin.

Senator Murray, do you have any further remarks?

Senator MURRAY. How are consumers supposed to know that?

Dr. SHUREN. Right now, they can't. They don't—because you don't have that oversight for some of these tests to know if they are, in fact—

Senator MURRAY. Does FDA not have oversight for this?

Dr. SHUREN. We do have the authority, and what we proposed is it's time for us to exercise it. Years ago, when we set up the program, laboratory-developed tests tended to be very simple. They were used locally for often rare conditions. In setting up the program, we said we would exercise what we call enforcement discretion. You are subject to our requirements. We're not enforcing them.

Over time, particularly without our being there, these tests have become increasingly more complex. They're being used nationally. They've become increasingly more important and being relied on for healthcare decisions. As a result of that and our seeing some of the bad tests out there is why we moved forward to regulate them.

I've got to tell you, this question has come up since the 1990s. We had NIH back then and the Department of Energy saying the FDA needs to get involved. The Institute of Medicine came out in the 2000s to say that—two advisory committees to the Secretary of Health and Human Services. We've been trying to move forward.

In 2007, we put out a guidance to say we'll start regulating a subset, and what we heard from the lab community was, "Don't pick away, don't chip away at this. Put out an overarching framework, make it risk-based, and phase in implementation."

We held a public meeting in 2010. We got that input, and the guidances we put out, the framework we proposed late last year, was our attempt to do that, to have a risk-based program, to try to balance innovation with patient safety and phase it in over a period of time. Right now, we're addressing comments. We're still working with the community on what that right policy should be, and then come up with a final—

Senator MURRAY. So you're engaging providers and patient groups as you work through that?

Dr. SHUREN. Yes. We've gotten a lot of feedback on it. An example is the American Cancer Society has said they have seen tests where they're incorrect. Patients are getting diagnosed with cancer when they don't have it, and people who have cancer are getting told they don't have cancer. They've said, too, that we need better oversight here. We need to make sure we have accurate tests and the tests do what they claim to do.

Senator MURRAY. Important question. I just have one more question.

Dr. Collins, why is 1 million the right number?

Dr. COLLINS. Well, that's a great question. There's nothing magic about 1 million except it's a nice round number that we could aim for. I actually will admit that I would love it if we could go beyond that. As I said a minute ago in the conversation with Senator Baldwin, this is all about numbers. That's where you get the power of the analysis to find out what works.

A million is very ambitious. It seemed like a goal we could set for ourselves to try to achieve. Given the fact that we already know there are cohorts out there which collectively have enrolled more than a million people, if we can figure out how to do this, maybe we'll do better than that.

Senator MURRAY. Well, just listening to the committee members that you've been talking to over the last few weeks, every one of our States apparently has some—we have Fred Hutch in my State that is developing a database.

Dr. COLLINS. Yes.

Senator MURRAY. I don't know how you're going to work through all of this to get your cohort. Again, diversity, making sure that we represent everybody is really important. I'm going to look forward to hearing how you do that.

Dr. COLLINS. I totally agree with you on that, because if we just tack together the existing cohorts, I don't think we would have the kind of representation that we need of the country.

Senator MURRAY. Thank you.

Thank you, Mr. Chairman.

The CHAIRMAN. Dr. Shuren, following up Senator Baldwin and Senator Murray, this was the high-risk, low-risk difference you were talking about in these laboratory tests. You're focusing your attention on the higher risk areas. Is that right?

Dr. SHUREN. Well, the high-risk, low-risk we were talking about earlier was on mobile technologies. We try to put a focus on—when we implement this, try to focus first on the higher risk tests as we phase that in.

The CHAIRMAN. Oh, I see, as you phase it in.

Dr. SHUREN. As we phase that in.

The CHAIRMAN. Is any of your enforcement—is it all prior approval, or is it where you might be acting on a complaint? In other words, to let the marketplace run for a while on the lower risk items and police it, in effect.

Dr. SHUREN. For the lower risk tests, we would not enforce requirements on them, other than tell us what you are, and if there are problems reported—we also wouldn't enforce requirements on tests for rare disorders and, again, some of these tests for unmet needs as well.

The CHAIRMAN. Well, this has been very useful. As I said to Senator Murray, this is kind of like going back to college. It's actually very interesting, and we're very privileged to be students in a classroom with such distinguished teachers and witnesses on a subject that's so important and one that the President, the House of Representatives, and this committee all are committed to work on.

It is my hope—and Senator Murray and I will work out exactly how we will do this, but it is my hope that we can finish our work on our innovation initiative this year and report it to the full Senate early next year so it can be acted on, or some schedule like that. We've got some other things we have to do as well. The precision medicine proposal by the President would be incorporated within that, so it'll be a part of all of it.

We didn't talk about privacy today. Who's going to figure that out?

Dr. COLLINS. A very important question. ONC and NIH and the White House OSTP have been engaged in this. We will have a deep conversation about this July 1st and 2d with the participants at the workshop that's coming forward at that point.

The CHAIRMAN. Well, what we're talking about—if you're going to have a million participants or more, and you use all their data, you've got to figure out some way to protect that.

Dr. COLLINS. To protect that, yes. We are deeply serious about doing that in the most high-tech, thoughtful, capable way, again, with a fair amount of experience to build on. We have to take that with great seriousness.

The CHAIRMAN. Dr. DeSalvo, you heard from a variety of Senators in regards to our interest in helping you figure out what the steps are to actually improve the electronic medical records system,

to get to see some real results, coordinate properly with whatever the Defense Department is doing. There's a lot of work to do there.

We're not trying to catch anybody here. We're just trying to fix a problem, and we'd like to work with you to do that and to do it soon, because it affects many, many physicians, many, many hospitals, and as we've heard today in a couple of important ways, it's absolutely essential to the Precision Medicine Initiative.

If there's no other—I've got a final page I'm supposed to read. The hearing record will remain open for 10 days. Members may submit additional information for the record within that time if they would like.

I want to thank Senator Murray again for the way she has conducted—helped us do this in a bipartisan way. We learn a lot more that way.

The next HELP hearing will be tomorrow on higher education. The committee will stand adjourned.

Additional Material follows.]

ADDITIONAL MATERIAL

PREPARED STATEMENT OF SENATOR KLOBUCHAR

Chairman Alexander and Ranking Member Murray, thank you for holding this important hearing today exploring how precision medicine can improve care for patients. I would also like to thank Dr. Collins, Dr. DeSalvo, and Dr. Shuren for being here to offer their views. Your agencies will be at the forefront of our Nation's work on precision medicine. I feel confident in the future of precision medicine with you three at the helm.

Without a doubt, doctors have always aimed to treat the individual patient, not the disease. Our medical technology might finally be catching up with our intentions as medical innovation hones in on the level of the individual patient through precision medicine.

In 2003, the National Institutes of Health and their international partners completed the mapping of the human genome—a scientific achievement equivalent to landing on the moon. The Human Genome Project jump-started the field of medical genomics and opened up new opportunities to improve how we care for patients.

We have already seen the extraordinary results that precision medicine—health care tailored to a person's genes, environment and lifestyle—can have, for example, in a breakthrough drug to treat cystic fibrosis.

Great work in the field of precision medicine is already being done in research centers around the country, including at the Mayo Clinic in Rochester, MN. I had the opportunity to see this work first hand when I visited Mayo's Center for Individualized Medicine. One great project is Mayo's BEAUTY study, which is working to understand why chemotherapy treats breast cancer in some women but fails in others. In addition, Mayo has a robust biobank with almost 50,000 samples that researchers can use to solve the mystery of a host of diseases.

The United States should remain on the cutting edge of health care innovation. Today we have the opportunity to build on the success of the Human Genome Project and lead the global effort to encourage scientific discovery and clinical implementation of precision medicine. To accomplish those goals, we need strong leadership from the Administration, continued investment in research, and the proper regulatory framework to encourage innovation.

With precision medicine, the health care of our future is closer than ever before. It will transform the way doctors provide care, making our health care system more efficient by providing improved, targeted treatments. Precision medicine is just beginning to show its promise, and it offers opportunities not just for patients in need of better treatments, but for the future of medicine as well. I know the committee and the Administration share the goal of supporting precision medicine, and that is why you are here today. I look forward to working with you toward accomplishing these shared goals.

[Whereupon, at 4:21 p.m., the hearing was adjourned.]

41

○