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THE FUTURE OF BIOMEDICINE: TRANSLATING BIOMEDICAL RESEARCH INTO PERSONALIZED HEALTH CARE

WEDNESDAY, DECEMBER 8, 2021

House of Representatives,

Subcommittee on Health,

Committee on Energy and Commerce,

Washington, D.C.

The subcommittee met, pursuant to call, at 10:30 a.m., in Room 2123 Rayburn House Office Building, Hon. Anna G. Eshoo, [chairwoman of the subcommittee] presiding.

Present: Representatives Eshoo, Matsui, Castor, Sarbanes, Schrader, Cardenas, Dingell, Kuster, Kelly, Schrier, Trahan, Fletcher, Pallone, ex officio; Guthrie, Upton, Burgess, Griffith, Bilirakis, Long, Bucshon, Mullin, Carter, Dunn, Curtis, Crenshaw, Joyce, and McMorris Rodgers, ex officio.

Also present: Representatives DeGette and Schakowsky.

Staff Present: Waverly Gordon, Deputy Staff Director and

General Counsel; Tiffany Guarascio, Staff Director; Zach Kahan, Deputy Director Outreach and Member Service; Mackenzie Kuhl, Press Assistant; Meghan Mullon, Policy Analyst; Juan Negrete, Junior Professional Staff Member; Kaitlyn Peel, Digital Director; Tim Robinson, Chief Counsel; Chloe Rodriguez, Clerk; Kylea Rogers, Staff Assistant; Andrew Souvall, Director of Communications, Outreach, and Member Services; Kimberlee Trzeciak, Chief Health Advisor; C.J. Young, Deputy Communications Director; Alec Aramanda, Minority Staff Member; Sarah Burke, Minority Deputy Staff Director; Grade Graham, Minority Chief Counsel, Health; Nate Hodson, Minority Staff Director; Peter Kielty, Minority General Counsel; Emily King, Minority Member Services Director; Bijan Koohmaraie, Minority Chief Counsel, O&I Chief Counsel; Clare Paoletta, Minority Policy Analyst, Health; Kristin Seum, Minority Counsel, Health; and Michael Taggart, Minority Policy Director.

*Ms. Eshoo. Good morning, everyone. Good morning, colleagues. The Subcommittee on Health will now come to order.

And due to COVID-19, today's hearing is being held remotely as well as in person.

For members and witnesses taking part in person, we are following the guidance of the CDC and the Office of the Attending Physician. So please wear a mask when you are not speaking.

For members and witnesses taking part remotely, microphones will be set on mute to eliminate background notice. Members and witnesses, you will need to unmute your microphone when you wish to speak.

Since members are participating from different locations at today's hearing, recognition of members for questions will be in the order of subcommittee seniority.

Documents for the record should be sent to Meghan Mullon at the email address we have provided to your staff, and all documents will be entered into the record at the conclusion of our hearing.

The chair now recognizes herself for five minutes for an opening statement.

Colleagues, we are here today to hear from our country's leading researchers about where biomedical innovation is headed and what we can do to accelerate innovation to improve

the health and the lives of every American.

This is, I believe, one of the most important topics we could be discussing at our subcommittee. This year marks 20 years since the initial results of the Human Genome Project were first published. The outcomes of the project provided a glimpse into DNA's potential for advancing research and launched a new era of biomedicine where genetic discoveries paved the way for new treatment options and improved human health.

The Human Genome Project was and remains the world's largest collaborative biological project. Ambitious for its time, the project sequenced the three billion pairs of DNA letters of the human genome in just over ten years, with $2.7 billion in funding.

This success is due to the multidisciplinary research efforts of 20 international institutions, the coordination of high-performance computing centers, and the successful management by the NIH and the Department of Energy.

Incredible advances in the field of genomics and the creation of state-of-the-art technologies now allow us to understand human biology much better than ever before. A human genome can now be sequenced in a matter of days for less than $1,000 on a single deep sequencing machine.

Genetic testing can now be done at home, to find increased risk for certain health problems, and CRISPR gene

editing can uniquely modify genetic code, offering hope for the time for treating rare genetic disorders in clever ways.

It is through groundbreaking scientific breakthroughs like these that the U.S. continues to be on the cutting edge of discovery. Fundamental discoveries and basic research continue to help scientists identify genetic variants that increase the risk of diseases like cancer and diabetes. And novel discoveries and translational research will pave the way toward innovative treatments.

As we meet today, Americans still face the highest disease burden and the highest rate of avoidable deaths when compared to similarly large and wealthy countries. Traditional medicine's approach of treating the average patient with a one-size-fits-all approach does not appropriately serve our country's diverse patient population.

We need to capitalize on the new tools and technologies that are being created to treat each patient as what they are, a unique individual.

I am greatly looking forward to hearing from today's witnesses about where they see the biomedical sciences heading and what Congress should be investing in to accelerate innovation for the betterment of the American people in the third decade of the 21st century.

[The prepared statement of Ms. Eshoo follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. The chair now recognizes Mr. Guthrie, the distinguished ranking member of our subcommittee, for five minutes for his opening statement.

*Mr. Guthrie. Thank you, thank you, Chair Eshoo for holding this hearing.

And today we are discussing ways to promote and advance American biomedical innovation.

I thank the chair for holding this hearing, and I look forward to hearing from our witnesses today about how health care delivery can be transformed through data generation and innovative technologies.

However, we cannot talk about advancing health care innovation without addressing the harmful drug pricing provision the Democrats' reckless tax and spending spree bill that will ultimately restrict patients' access to timely care and lead to less cures.

This past year several indicators have shown the Biden administration is moving the country in the wrong direction. We are facing the highest levels of drug overdoses in our Nation's history, in part, because of the influx of deadly drugs, including fentanyl, exacerbated by the Biden administration's failure to secure our southern border.

Additionally, Americans are experiencing the highest levels of inflation in over 30 years. This is the most expensive Thanksgiving holiday most have seen.

Despite all of this, the White House and Democrats in Congress continue to ignore these flashing red lights and are downplaying the true risks that increasing Federal spending will have on American households.

Even the former Chair of the White House Counsel of Economic Advisors in the Obama administration is sounding alarm bells on inflation by stating that the Biden administration's officials are systematically underestimating inflation and further saying they poured kerosene on the fire by signing the massive $1.9 trillion American Rescue Plan.

The Democrats' most recent partisan effort would force drug manufacturers to accept a government-mandated price of a drug or potentially face up to a 95 percent excise tax for refusing to accept the government's bad deal. Make no mistake. This is not a negotiation. This is government price setting.

The University of Chicago published an issue brief last week on the impact of this tax and spending bill on biopharmaceutical innovation and patient health. The studies in the summary brief found the drug pricing provisions would lead to a decrease in research and development investments by over 660 billion through 2039, resulting in 135 fewer drugs brought to the market during this time.

Most consequentially, they found it could lead to the loss of approximately 331 million life-years, which is 31

times higher than loss due to COVID.

Most Americans are against this. A survey from the Kaiser Family Foundation found that 72 percent of Americans oppose drug price negotiation if it leads to fewer medications being developed in the future.

The bill punishes our innovators and undermines the significant strides of this committee. Operation Warp Speed under President Trump's leadership, and others have made throughout the pandemic to get needed treatments to health care settings as quickly and safely as possible.

The good news is there are existing bipartisan proposals introduced by members of this committee to address the rising cost of prescription drug medications for patients without harming innovation.

H.R. 19, the Lower Cost, More Cures Act, includes 40 bipartisan proposals that would bring needed market-based reforms like addressing pay-for delay tactics in order to lower the cost of prescription medications without threatening the development of future cures.

H.R. 19 would specifically give seniors relief by capping their annual out-of-pocket spending and reducing the cost of insulin for seniors by capping monthly insulin costs, once deductibles are met. In fact, Build Back Better does have some of these proposals from H.R. 19, which shows that there are areas of agreement on how to lower drug costs for

Americans.

Where we do not and we will never agree is on the idea that forcing companies to accept whatever price the government feels like paying for prescription medications is the correct way to lower the cost of prescription medications.

I am deeply concerned about the estimated loss of more than 100 new cures if Build Back Better is signed into law and hope this bill ultimately fails.

Moving forward, I encourage my colleagues to work with me on bipartisan reforms to lower drug prices and find solutions that prioritize getting more affordable treatments to market for patients living with life-threatening and debilitating diseases like ALS and other neurodegenerative diseases. Doing so can improve the quality of life for millions across the country.

Thank you, Madam Chair, and I yield back.

[The prepared statement of Mr. Guthrie follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. The gentleman yields back.

The chair is now pleased to recognize the chairman of the full committee, Mr. Pallone, for his five minutes for an opening statement.

*The Chairman. Thank you, Chairwoman Eshoo.

I know this is an issue that is very important to you and you have been involved with for a long time, and that is the cutting edge of biomedical research.

As we celebrate the 20th anniversary of the mapping of the human genome this year, it is an opportunity to examine how far we have come and how scientists are charting a path forward to lead to new discoveries to improve public health.

The purpose of today's hearing is to examine the current state of biomedical research in the U.S. and explore the opportunities for the future of innovation, investment, and equity in health care.

Our Nation is fortunate to have the greatest biomedical researchers in the world, working every day in clinics and labs to advance our basic understanding of disease in living organisms and apply that foundational knowledge to the development of treatments and cures.

Historically, our health system has focused on treating or preventing diseases broadly in the average patient. This has resulted in treatments and drugs that work well for some but have little to no effect in others.

In the last decade, however, we have seen transformative changes in the field of biomedical research. One such example is the advancement of precision medicine, which seeks to individualize treatment and care by accounting for patient-specific genes, environment, and lifestyle.

For example, research and development in precision medicine has helped advance immunotherapy treatments for oncology. If we are to continue to build on this work, we will need to leverage new technological tools and methods of study, such as genetic phenotyping, quantum computing, novel clinical trial designs, as well as traditional basic and translational research.

As we examine the current state of biomedical research, we must keep equity at the forefront of our efforts. The ongoing COVID-19 pandemic has demonstrated what many have known all along, that our health system disadvantages minority communities and inadequately addresses their needs.

We must examine and account for diverse populations in data collection, as well as recognition of potential biases in artificial intelligence, biomedical research, and the development of drugs, devices, and treatments.

It is also important that we ensure Americans can access these drugs and treatments, and that is a critical component of the Build Back Better Act that the House passed last month. Today, far too many Americans are being forced to

ration their medications, go without needed treatments, or exhaust their life savings because prescription drug costs are too high.

It is simply not fair that Americans pay three, four, or ten times as much for the exact same drugs as people in other countries pay.

The Build Back Better Act will make prescription drugs more affordable by finally giving Medicare the ability to negotiate lower drug prices with the pharmaceutical companies. Seniors will also pay no more than $2,000 a year in out-of-pocket costs for their drugs, and the legislation penalizes pharmaceutical companies that unfairly raise prices.

The bill also allows the Federal Government to negotiate insulin prices and lowers those prices to no more than $35 a month for Americans with diabetes.

And this legislation finally begins to provide relief to Americans at the pharmacy counter without threatening innovation.

So I look forward to hearing from our panel of experts who have significant experience in academic, clinical, regulatory, and commercial settings. The future of biomedical research depends on the synergy between these fields, and the fruits of their labor will transform our health system to promote wellness for all Americans.

So, again, I thank the chairwoman for convening this hearing and continuing her work to move the ball forward on such an important topic.

And I yield back.

[The prepared statement of the Chairman follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. The gentleman yields back.

The chair is now pleased to recognize Representative Cathy McMorris Rodgers, the ranking member of our full committee, for your five minutes for an opening statement.

*Mrs. Rodgers. Thank you, Madam Chair.

And to our witnesses, I want to extend this special thank you to Dr. Leroy Hood for making the trip all the way from the great State of Washington to be with us.

The story of American biomedical innovation is one that should be celebrated. Through the NIH's Human Genome Project, we know that there are over 20,000 human genes.

To help discover new cures, this information is being used to identify genes found in conditions like Alzheimer's, cancer, and rare diseases.

The 21st Century Cures Act gave the NIH the resources to advance basic biomedical research across the spectrum.

As co-chair of the Neuroscience Caucus, I have been a strong supporter of the BRAIN Initiative, which is aimed at finding new ways to treat, cure, and prevent brain disorders by exploring how the brain enables the body to store and retrieve information quickly.

The All of Us Research Program is also revitalizing the health care system by teaching us more about precision medicine and personalized care plans. I am excited about these researchers' innovative work that will reduce cost and,

more importantly, save lives and improve people's quality of life.

America's biopharmaceutical sector is vital to our global competitiveness. There are over 4,000 cancer drugs in the R&D pipeline, 700 for neurological conditions, and 450 for cardiovascular disease.

We are on the verge of amazing breakthroughs. America is leading the way in bringing hope to patients here and around the world.

Unfortunately, in the reconciliation package pending before Congress, this would be reversed, and the incredible work would be lost. And it would eliminate hope for future cures.

Price controls, price controls that are being included right now in President Biden's plan will kill innovation and lurch us more toward government-controlled health care. We see in countries like Canada and the U.K. the power rests with the government to measure lives in dollars and cents before politicians decide whether a cure is worth it.

It would mean no hope for many people who deserve a fighting chance at life. It would also push private innovators further overseas and empower countries like China, which is already racing to lead the world in biotechnology.

We all sadly saw what happened during the pandemic when China dominated the market for certain medical supplies.

Surely, we all agree that less innovation, fewer cures, and a dependency on China cannot be America's future.

There was bipartisan agreement on this just a few months ago. That is why H.R. 3, government price control, failed in this committee. Unfortunately, this policy has been resurrected in the bill that has now passed the House floor.

I look forward to hearing today about how we can unleash more biomedical information, not destroy it with government price controls.

The Congressional Budget Office confirmed that there will be fewer new medicines as a result of government price controls that ultimately passed the House. Hopefully it will not pass the Senate.

A University of Chicago study estimates that it would shrink R&D spending by 18, 18.5 percent and lead to 135 fewer new cures. The study found price controls would generate a loss 331 million life-years, which measures the lost potential of saved lives and longer years lived.

This study found that government price controls would lead to 21 to 43 fewer new antiviral drugs. They estimate four to nine fewer new HIV drug approvals, and about two to five million life-years lost as a result of price controls.

We have heard some suggest that this reduction in cures and treatments is just a feature of built-in cost of bringing down drug cost. It has been suggested that it is, quote,

worth it and a tradeoff Americans are willing to accept.

I do not believe it. It is a false choice. It is a false choice on families like Crystal Davis who believed in the promise of America so that her son with SMA can live a full life. We should be doing all that we can to encourage hope in the next generation of cures.

Let's reject price controls and focus on bipartisan work like solutions in H.R. 19, which will result in increased competition and lower patient cost without sacrificing the future of biomedical innovation in the United States.

Now more than ever we should be working together on uniquely American solutions to save lives, lower cost, and uphold the dignity and right of every person to live a full life. Energy and Commerce can lead the way.

I look forward to the discussion today.

I yield back.

[The prepared statement of Mrs. Rodgers follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. The gentlewoman yields back.

Pursuant to committee rules, all members' written opening statements shall be made part of the record.

I now would like to introduce our witnesses. First, Dr. Amy Abernethy is the President of Clinical Studies Platforms at Verily Life Sciences.

Welcome to you and thank you for being with us today.

Remotely, Dr. Atul Butte is the Priscilla Chan and Mark Zuckerberg Distinguished Professor and the Inaugural Director of the Bakar Computational Health Sciences Institute at UCSF. That is University of California at San Francisco. He is also the Chief Data Scientist for the entire University of California Health System.

Welcome to you, Doctor, and thank you very much for being with us.

Mr. Adolph Falcon is the Executive Vice President of the National Alliance for Hispanic Health.

Welcome to you, and thank you for being with us.

Dr. Leroy Hood is here at the witness table. He is the President of the Institute for Systems Biology and an Affiliate Professor of Immunology at the University of Washington.

Welcome to you, Dr. Hood, and thank you.

And last but certainly not least, Dr. Lloyd Minor who is the Dean of the Stanford University School of Medicine, which

I have the privilege of representing.

Welcome to you, Dr. Minor, and thank you for the on again, off or putting up with the on again, off again changes in schedules for this hearing. I appreciate it. We all do, and it is an honor to have you with us this morning.

So thank you for joining us today. We look forward to your testimony.

You are probably familiar with the lights that are in front of you. You have a minute remaining when the light turns yellow, and we all know what red means.

So let's begin with Dr. Abernethy. You are recognized for five minutes for your testimony.

STATEMENT OF AMY ABERNETHY, M.D., Ph.D., PRESIDENT OF CLINICAL STUDIES PLATFORMS, VERILY LIFE SCIENCES; ATUL BUTTE, M.D., Ph.D. DISTINGUISHED PROFESSOR AND DIRECTOR OF THE BAKAR COMPUTATIONAL HEALTH SCIENCE INSTITUTE, UCSF, AND CHIEF DATA SCIENTIST, UC HEALTH; ADOLPH P. FALCON, M.P.P., EXECUTIVE VICE PRESIDENT, NATIONAL ALLIANCE FOR HISPANIC HEALTH; LEROY HOOD, M.D., Ph.D., PRESIDENT, INSTITUTE FOR SYSTEMS BIOLOGY, AFFILIATE PROFESSOR OF IMMUNOLOGY, UNIVERSITY OF WASHINGTON; AND LLOYD B. MINOR, M.D., DEAN, STANFORD UNIVERSITY SCHOOL OF MEDICINE

STATEMENT OF AMY ABERNETHY

*Dr. Abernethy. Thank you, Chair Eshoo, Ranking Member Guthrie, and members of the Health Subcommittee.

Thank you for inviting me to speak with you today about personalized health care. I have spent my career working on ways to make sure patients are getting the care that is tailored to their unique circumstances and to make sure that that care is based on the best available evidence.

For many years before my tour at FDA, I was a Professor of Medicine at Duke University. My clinical training is in oncology and palliative care. I focused on patients with melanoma.

I also directed the Center for Learning Health Care at

Duke, where the vision was to enable whole-person care to meet the individual where they are in their health journey and seamlessly bring together research and clinical care so that they inform each other in a patient-centric way.

It is, therefore, a real honor and humbling that after many years I am here with this panel discussing ways that we can come together to make personalized health care a reality.

If there is one message I want to emphasize today, it is that we are not going to reach the goal of personalized health care unless we make big gains in how we generate and analyze health care data, data that tell us how new treatments work at the individual personalized level.

Data and the clinical evidence generated from these data are the fundamental underpinning of personalized health care decisions.

Let's take cancer diagnosis as one example. With personalized health care, it will not be just a matter of matching a certain cancer mutation with the appropriate drug for that cancer mutation, but rather a selection of the appropriate intervention, and it will depend on many features blended together, including a person's symptom experience, such as what symptoms are bothering them the most; the genetic basis for the disease; and lots of additional details, such as the likelihood for the intervention to work based on a person's background, genetics or environmental

exposures.

Finally, the selection of the intervention should account for the personal values of the patient.

Personalization in health care means far more than just matching a treatment to specific biologic markers. It is the ability to consider many features and circumstances together to support ultra-tailoring, matching to the intervention and the patient.

What gaps do we have before this vision can be realized? Diversity in clinical trials, a topic I hope that we cover today, is just one example.

Traditional clinical trials are an extremely powerful way for understanding what treatment works. But the ways we have historically done clinical trials also have significant drawbacks, not the least of which is that these trials only capture a segment of the population.

As a result, such trials typically cannot account for the great diversity of people in our society. We can do better. Diversity in clinical trials is only one of the challenges that we need to address, but it is a big one.

When I say that we need to address the gaps in evidence generation, I do mean "we.'' Congress and the Executive Branch agencies involved, including HHS, FDA, CMS, and others, are extremely important for setting the requirements and goal posts for developing health care evidence.

For example, 21st Century Cures represent a big leap in the right direction and included a multitude of provisions that have helped to put us on a course to make better use of real-world data and real-world evidence.

The Cures 2.0 Act has some very important proposals that can continue the momentum, including on themes like decentralized trials, use of real-world data, and diversity in clinical trials.

But I will reiterate a point that I made over and over again when I was at FDA. In addition to government, industry also has a critical responsibility to push the field forward, to generate the tools needed to translate biomedical discoveries into personalized health care.

Different components of industry are now playing different roles in this work. On the discovery side, there are many companies, large and small, doing revolutionary work with gene and cell-based therapies, advanced diagnostics, and digital therapeutics. These are just a few categories where we are seeing solutions.

On the health tech side, we, including the company where I now work, Verily, are working to develop the machinery of evidence generation. We are hyper focused on making clinical trials run more efficiently and reach a broader, more diverse set of patients than ever before.

We are building data sets that allow us to combine the

best features of clinical trials and real-world data, and we are developing methods for monitoring the performance of health care products when they are deployed in the real world, especially important for monitoring tools, such as artificial intelligence-based tools of medicine.

And we need to do all of this with robust, transparent, and secure works in a way that protects personal privacy. This work is complex and takes collaboration between clinicians, data scientists, privacy experts, and of course, excellent software energy and engineers.

I look forward to talking with you today. Thank you.

[The prepared statement of Dr. Abernethy follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. Thank you very much, Doctor.

The chair is now pleased to introduce Dr. Butte for five minutes for your testimony.

STATEMENT OF ATUL BUTTE

*Dr. Butte. Thank you, Chair Eshoo, Ranking Member Guthrie, and committee members.

My name is Atul Butte. I am a physician scientist and institute Director at the University of California at San Francisco and the Chief Data Scientist for the whole UC Health System.

I have to start by saying the views expressed here are my own and do not represent the views of the University of California or any of these organizations.

Since 2013, when White House Office of Science and Technology Policy, Dr. John Holdren directed Federal agencies to enable the results of federally funded research to be made freely available to the public, our national data resources are growing, but we do not often think about this biomedical infrastructure like we think about other national resources, like our national labs or our national parks or our roads and bridges.

I will highlight a few examples here, all of which are housed at or through NIH of this national data resource. GenBank contains all publicly available DNA sequences. While nearly 40 years old, it is still a relevant --

*Ms. Eshoo. Doctor, I am sorry to interrupt. The sound is a bit jerky. So can you just speak a little slower so

that we can absorb every word. I am not catching it all, and I think it may be a little difficult for the rest of the members as well. Every word you say counts. So if you could just slow down a little bit and if you need a little more time, I will certainly grant it. Okay?

Thank you.

*Dr. Butte. Surely. I will slow down.

I am highlighting a few examples of our national data resources, which are growing, and we should think of these as national resources, like our national labs and national parks, to be protected.

GenBank, for example, nearly 40 years old now, is a relevant worldwide home for even SARS-CoV-2 sequences.

Cancer Genome Atlas, one of many disease-specific databases funded by NIH, has led to tens of thousands of cancer patients being studied, led to many discoveries, and while the program has formally ended, the data is still there publicly accessible.

And many others, tens of thousands of data resources around the world emphasizing the volume and complexity of data needed to understand the human condition. And all of this is just the tip of an iceberg of the data we are going to need to develop the next generation of cures and treatments.

And we are expecting much more to come. Starting in

January 2023, it is great that NIH will be executing on their new policy requiring all NIH supported research include a data management and sharing plan. It will be important to ensure these plans are good plans and in force.

A newer source of biomedical data is electronic health record data surrounding clinical care. One of the most exciting roles I have now is that of Chief Data Scientist for the entirety of the University of California Health System, and across our six medical schools and 12 hospitals, we have treated over seven million patients in the past ten years.

We have a secure data warehouse now that we use to improve the quality of our care and, when deidentified, to enable the next generation of clinical research with data on hundreds of millions of encounters, procedures, and nearly a billion medication orders and prescriptions.

The narrative I want to make sure I communicate is that we have spent billions of dollars to acquire this data on patients. In fact, I call it the most expensive data in America now. It will be a national tragedy if we do not use this data, of course, safely and responsibly; if we do not use this data to improve the practice of medicine.

This clinical data can inform patients as to the detail of what is going on in their care and what is next. Data on the pricing of services can help patients select the right level of care at an affordable price, and we can start to use

this data to eliminate unnecessary use of specific medications.

And I am proud that the University of California Health System recently signed a health equity pledge, along with 40 other institutions, to leverage our clinical data to document and address health equity or specifically inequities.

I am going to end with some specific recommendations. First, more funding should be made available for training in biomedical data sciences at all stages, teaching statistics, programming, and database skills, design and visualization.

Second, Federal funding for these important biomedical data repositories remains quite variable, too arbitrary, and too fragile, and needs to be stabilized.

Third, can we open more Federal Government-related data to others? Imagine if the millions of chest X-ray images, for example, from federally run hospitals and clinics were carefully and safely shared with available AI engineers to build novel tools to help lead them and then companies around those schools.

We should invest in technological solutions, enable broader and better use of our national data resources.

Fourth, let's ensure that the 2023 NIH policies for data sharing do carry through, and that we create a better culture of research data that is disseminated with the public.

And fifth and final, we need to build on programs like

the new NIH AIM-AHEAD to not only make sure diversity is properly covered in our biomedical data set and artificial intelligence models, but diversity is promoted and enhanced among the data scientists themselves.

Thank you for enabling me to give my signature. Thank you.

[The prepared statement of Dr. Butte follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. Thank you, Dr. Butte.

It is almost encyclopedic what you just shared with us, and I look forward to the questions that we are going to ask you to answer.

Next, Mr. Falcon. You are recognized for five minutes, and thank you, again, for being with us today.

STATEMENT OF ADOLPH P. FALCON

*Mr. Falcon. Well, thank you, Chairwoman Eshoo and Ranking Member Guthrie and members of the Health Subcommittee. I thank you for the opportunity to testify today on behalf of the National Alliance for Hispanic Health, the Alliance and the Healthy Americas Foundation, which is the supporting organization of the Alliance.

The Alliance is the Nation's premier science-based and community-driven organization that focuses on the best health for all. We work to ensure that health incorporates the best of science, culture, and community.

Our community-based members I am proud to report deliver health and human services to over 15 million in underserved communities every year, and over the past two years, we have been at the front line of our Nation's COVID-19 response.

We, as an organization, know the benefit of biomedical research, but we have challenges, and I have submitted written testimony to the subcommittee, but I would just like to cover a few of those challenges.

One is the ongoing inadequate inclusion of underrepresented population groups, and the lack of inclusion is not a new issue. Dealing with the lack of inclusion was a central recommendation of the 1985 report of Secretary Heckler's Task Force on Black and Minority Health.

In fact, out of that task force work in 1989, we added a Hispanic identifier for the first time to the model death certificate. Adding that data was transformative. It showed us that regardless of country of heritage Hispanics actually live longer than non-Hispanic whites, and that is true despite additional risk factors like diabetes, excess weight, lack of health insurance.

The one-size model never served anyone, and it only created distorted models of health. Good science, good epidemiological practice, and development of safe products require adequate inclusion of all.

But we are not there. For example, Hispanics represent one in five persons in the U.S., but we only represent about five percent of participants in clinical trials. An analysis of a decade of clinical trials that led to approved cancer drugs found that only one in ten of those trials reported data for Hispanics.

And tragically, Hispanics represent only one percent of individuals in genome-wide association studies.

This lack of inclusion not only limits our ability to translate biomedical research into health care. It also is ignoring the law. The 1993 NIH Revitalization Act required inclusion of all groups.

The good news is that we know it works. We have existing standards of community-based participatory research

where researchers and community members collaborate as equal partners in design, carrying out the assessment and analysis of research. We know these standards work and deliver inclusive science.

For example, the Hispanic Community Health Study at NIH has already enrolled 16,000 Hispanic adults from four diverse communities in a long-term study, and the NIH's All of Us Research Program is a shining example of inclusion of community-based participatory research.

Right now with all of us we have over 400,000 participants and participants from racial and ethnic groups underrepresented in biomedical research represent a majority of those participants.

And we have seen the importance of this kind of research in a response to COVID-19. With All of Us, we were able to quickly test over 24,000 participant samples to look for antibodies against SARS-CoV-2, providing significant information to our Nation's response.

We can do better in terms of inclusion. Three items I would draw your attention to are: one, the importance of passing the Diversifying Investigation Via Equitable Research Study for Everyone, DIVERSE Trials Act.

And I thank many of the members of this committee for their support of that Act.

Two, we should be mandating inclusion of community-based

participatory research standards, both as a part of FDA review of new drug applications and as a part of NIH's review of research funding proposals.

And, thirdly, it is time to require the establishment of a task force and a report by HHS on efforts for collecting and analyzing data for population underrepresentation in biomedical research. It has been 36 years since the report of Secretary Heckler's Task Force on Black and Minority Health. The time for an update is long overdue.

I thank you for the opportunity to present testimony to you here today.

[The prepared statement of Mr. Falcon follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. Thank you for your excellent testimony.

The chair is now pleased to recognize Dr. Hood for your five minutes of testimony.

STATEMENT OF LEROY HOOD

*Dr. Hood. Thank you, Chairman Eshoo, for inviting me to testify today.

I would also like to commend you for authorizing the ARPA-H Program. With this initiative, I think we can say comes big, bold, paradigm-changing effort to transform health care.

And one such program is the Beyond Human Genome I will speak about that will bring actionable outcomes to patients from the very beginning.

*Ms. Eshoo. Dr. Hood, can you move the microphone a little closer?

*Dr. Hood. Yes.

*Ms. Eshoo. That is it. Good. Thank you.

*Dr. Hood. We face in health care today five major challenges: one, the quality of health care; two, the escalating cost of health care; three, an aging population; four, an explosion of chronic diseases, and five, the need to have equity in outcomes and opportunity for health care.

I am going to discuss this program Beyond the Human Genome Project, and I will argue it will bring novel approaches for each of these challenges.

The essence is the following. We know with the data-driven approach can follow the health trajectory of each

individual over time and optimize their health, minimizing disease, and bringing people hopefully into their 90s or 100s mentally alert and physically capable.

To execute this kind of program, I have proposed this initiative Beyond the Human Genome, and my suggestion is it be directed initially by a nonprofit I have created called Genome Health, and that we analyze the genomes and phenomes of a million persons over a ten-year period, i.e., the second genome project supported by the Federal Government just as we did for the first.

The genome is a static, unchanging digital source code of life. The phenome represents the state of an individual as you change across your lifetime, and that is determined by three things: your genome, your lifestyle, and the environmental exposures that you have.

We can assay this phenome at different times by measuring and quantifying up to 1,000 or more protein analytes; by looking at your gut microbiome, the species in your gut that determine very much about your health; and digital cognition measurements and digital physical health measurements.

And these are the tools we will use to follow trajectories. Thus, the longitudinal phenome is what is Beyond the Human Genome Project, and it provides deep actionable insights into body and brain.

We have two proofs of principles. One, we have looked at 5,000 people over four years, and by using this data-driven approach, we have been able to identify a powerful set of actionable possibilities we call scientific wellness that are validated by the literature.

Number two, we can create a metric that measures how rapidly you age and makes suggestions about how to optimize aging.

And there we have seen now the ability to discover the earliest transitions of chronic disease years before they manifest themselves, giving us the opportunity to reverse them when they are at simple stage.

Our partner Posit has actually done the same kind of thing for the brain, that is, 40 digital brain measurements that assess 25 different cognitive elements in a way that measures them and optimizes them for optimal brain health.

We have three key partners in this endeavor. Guardian Research Network, they are connected to 100 hospitals, 30 million patients in 13 States across the South and the Southeast. The important point is they lie across populations, Latino, Black, and economically disadvantaged, and we plan in the million-person project to have the proper ratios of all of these individuals in the program.

The second partner is Providence with coverage in brain health.

And the third partner just announced to us recently is Google that has made available to us its search, its cloud, its digital health, and its hyper scale AI techniques to optimize the platforms we need to make this program a reality.

If you look around the world, all --

*Ms. Eshoo. You need to wrap up, Doctor.

*Dr. Hood. Oh, okay. I have got much more to say. We will answer any questions.

[The prepared statement of Dr. Hood follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. Wonderful. Thank you very much.

Dr. Minor, it is a pleasure to welcome you to our subcommittee. Thank you for your special leadership, and you members think I am biased. You are absolutely right. I am.

You are recognized for five minutes for your testimony.

STATEMENT OF LLOYD B. MINOR

*Dr. Minor. Thank you.

Good morning, Chairwoman Eshoo, Ranking Member Guthrie, Chairman Pallone, Ranking Member McMorris Rodgers, and members of the subcommittee. I am honored to appear before you on behalf of the Stanford University School of Medicine.

I would like to express my gratitude to Representative Eshoo for her years of support for Stanford Medicine. I also thank this committee and Congress for your leadership and longstanding investments in biomedical research through the National Institutes of Health.

This bipartisan support of biomedicine is enabling people to live healthier lives while strengthening our Nation's economy and its future competitiveness.

At the heart of this progress is basic science, which provides the foundational knowledge upon which all novel therapeutics, interventions, and diagnostics are developed.

NIH funding drives our Nation's preeminence in basic science research and robust investment is critically important to our health, our economy, and our global standing.

COVID-19 mRNA-based vaccines underscore the extraordinary return on basic science investment. Beyond the hundreds of thousands of lives saved, these vaccines, built

on decades of research, are blunting the pandemic's financial burden, which some economists estimate could reach $16 trillion if left unchecked.

These vaccines also exemplify the power of preventive medicine, a central focus of Stanford Medine's precision health vision. This proactive approach to health care seeks to transform our system of sick care into true health care, using Data Health, using health data, AI, emerging technologies, and lab-based discovery. Precision Health emphasizes predicting, preventing, and curing disease precisely, critically in that order.

More than ever we are challenged by diseases that demand Precision Health solutions. Consider the growing physical and economic burdens of mental illness in the United States. From 2009 to 2019, spending on mental health treatment and services increased 52 percent, reaching $225 billion.

SAINT, or Stanford Accelerated Intelligent Neuromodulation Therapy, exemplifies one Precision Health approach to addressing this challenge. This experimental treatment uses a magnetic coil to directly stimulate underactive parts of the brain in people with clinical depression.

In October, results from the latest clinical trial showed that 80 percent of participants went into remission after receiving the therapy.

NIH grants have made this promising endeavor possible, among many others at Stanford Medicine, which are detailed in my written testimony. And though we are fortunate to have this support, the funding landscape grows ever more competitive.

Since 2000, NIH applications have doubled. The success rates have declined sharply. Research proposals that Federal agencies rate as excellent are often not funded due to limited resources.

For this reason, I urge you to continue to strongly increase funding for basic research. Supporting this pursuit of knowledge has produced stunning medical advances, generated new fields of research, and made the unimaginable possible. And it will continue to so long as scientists are supported in exploring the unknown.

Basic research remains the bedrock of innovation, but translational research is also critical. At Stanford Medicine, we are encouraged by the promise of a model that supports basic science and the translation of discovery through creation of an Advanced Research Project Agency for Health, or ARPA, also known as ARPA-H.

Legislative efforts to fund and establish ARPA-H, such as Chairwoman Eshoo's recently introduced authorizing legislation, recognize the critical importance of our country's biomedical enterprise and reflect our aspirations

to move discoveries from lab bench to bedside.

To help bridge this gap and accelerate the translation of promising therapies, we recently launched the Innovative Medicines Accelerator, or IMA. Serving as an ARPA-H of sorts, the IMA provides researchers from across Stanford University access to the technology, resources, expertise, and funding needed to advance their discoveries to improve human health.

Though only recently formed, the IMA has already had a significant impact. Originally designed to aid development of medicines for diseases such as cancer and rare disorders, the IMA pivoted early in the pandemic, enabling our faculty to better address the public health crisis.

In the months following the beginning of the pandemic, the IMA awarded research grants, supported two trials on repurposed drugs, and initiated two industry-sponsored trials, all the while building out the infrastructure to enable further research.

I am more optimistic than ever about the future of biomedicine in the United States for many reasons. Our world class academic medical centers, longstanding congressional support, and a diverse population uniquely position us to continue to lead the scientific revolution.

However, as competing economies around the world pump money into biomedical research, remaining at the forefront

will require increased investment in the research that fuels our technological and scientific progress.

Moreover, it is critical that we continue to invest in the diversity of our scientific research community and support those from underrepresented groups, which I described in my written testimony.

I hope you agree that these are urgent issues for our Nation's health, our economy, and our standing as a global leader, and our future.

Thank you.

[The prepared statement of Dr. Minor follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. Thank you, Dr. Minor.

We will now move to members' questions, and the chair recognizes herself for five minutes to do so.

I will go to Dr. Minor first.

Obviously, I take great pride in representing Stanford because it is an institution where you, Dr. Minor, and your colleagues are at the cutting edge of so many fields.

Can you broaden out what you state in your testimony and tell us what key enabling technologies you think will play the biggest role in medical innovation in the coming decade and where we should be directing Federal investments?

Is it AI? Is it quantum computing? Is it something else completely? Is it all of the above?

And also, your testimony and others as well discuss the importance of diversity. What is your experience with what works for increasing diversity in biomedical and life sciences research?

*Dr. Minor. Well, thank you, Congresswoman Eshoo.

On the first question, and we heard from the distinguished experts giving testimony this morning in many of the areas that I think are important for advancing the future of biomedical research and improving the health of Americans, indeed, the health of everyone in the world.

I see the future as really recommending the convergence of related but distinct areas, the first being biomedical

science, biotech, med tech., the second being information science, and the third technology.

It is the fusion and the virtuous triangle created by the synergies among these three disciplines that I think will transform the biomedical landscape in the next decade-plus and will help us to bring about even greater improvements than we have seen in the past in predicting, preventing, and curing disease precisely.

On your second question with regard to diversity, I think the first point and the first important point is being very intentional about how we as leaders in higher education, leaders in academic medical center, how we reach out, how we design training programs, how we create the environment that attracts and nurtures the careers of young people from diverse backgrounds.

At Stanford, one of the things we have been able to do, thanks to generous philanthropic gifts, is to establish a program that is going to bring people from historically underrepresented communities to post-baccalaureate programs at Stanford as well as increase the recruitment of underrepresented minorities to our faculty and our outreach through our programs with historically black colleges and universities, our outreach to institutions that have not had the opportunity that many institutions in our country have had because of our country's history of racism and

disparities that we really have to take a proactive stance in curing and correcting moving forward.

*Ms. Eshoo. Thank you, Dr. Minor.

To Dr. Falcon, thank you for your excellent testimony.

I want to stick with the last point around diversity. The statistics you shared about the lack of diversity in clinical trials I found really chilling, and your testimony points to several efforts that increase inclusion and diversity in research.

Why do you think to date the efforts have fallen short so far? What have we been doing wrong?

There have been efforts. Tell us what you think about that.

*Mr. Falcon. Sure. Clearly, we have put out standards. FDA has standards for inclusion. NIH has standards for inclusion, and this body has mandated development of those standards.

But standards are not enough. It has come time to move beyond standards and start mandating inclusion, and we have seen that work. For example, four years ago, as part of FDA's appropriation, FDA was required to start reporting data for inclusion of Hispanics as part of their clinical trials drug snapshot.

Simply knowing that that data was going to start being reported publicly made a change. We have seen the numbers

for inclusion of Hispanics in FDA-reviewed clinical trials improve since that was required.

We need to require that any reviews of FDA clinical trial research have a score with regard to inclusion that as those clinical trials are conducted, they report regularly to FDA whether or not they are meeting their inclusion standards, and if not, we should be kicking new efforts to bring those trial, while they are in the field, up to the standards of inclusion.

Similarly, NIH, as part of their review of proposals for funded research, should have a relevancy score that would look at its not only inclusion by race, ethnicity, sex, gender, but also whether or not the studies are powered to report that data separately.

We have seen, for example, just last month the New England Journal of Medicine is now going to require all published research to include a table that specifically states what populations were included and in order to increase diversity in all of New England Journal of Medicine's publications.

*Ms. Eshoo. I think I have to ask you to stop because I am way over my time, but each of us has five minutes to ask questions. I could easily use a half hour to field questions to those that have testified today, but we can submit question, written questions, to our witnesses.

So thank you to each one of you.

The chair now recognizes Mr. Guthrie, the wonderful ranking member of our subcommittee, for his five minutes to ask questions.

*Mr. Guthrie. Thank you, Madam Chair. I appreciate it.

And before I begin my questions, I want to mention how important it is for the Biden administration to keep crucial components of the Trump administration era, Medical Coverage for Innovative Technology, or the MCIT, interim final rule intact to ensure timely access for care for Medicare beneficiaries.

I recently read a letter with over 660 bipartisan members calling for the Biden administration to work with industry partners to resolve outstanding implementation issues while providing temporary coverage, both prospective and retroactive, of these approved devices to allow patients access to care.

Dr. Abernethy, how can we build off policies like MCIT, the MCIT rule to ensure we are incentivizing future investments in innovative technologies and apply requisite data to make informed coverage decisions and subsequently advancing future investments in novel health care technologies?

*Dr. Abernethy. Thank you, Mr. Guthrie. This is an important question.

Really as we think about the goal of getting innovative technologies as another intervention to patients, the real question is how do we make sure we have the mechanisms in place to provide those innovative therapies and also the mechanisms in place to evaluate how they perform, including in the real world.

I think the MCIT discussion or proposal really brought to the forefront that critical discussion that, for Medicare beneficiaries and beyond, how do we really think beyond even the approval state to continue to evaluate how these interventions perform for all people.

I think one of the things that I saw when I was at FDA, the importance also that if we are going to have continued evaluation of interventions, that CMS has the capabilities and resources to also support that evaluation across time.

*Mr. Guthrie. Unlike better integration of the FDA approval that you were involved in, CMS coverage and data collection increase our potential for truly personalized medicine, and what more can Congress do to make that happen?

*Dr. Abernethy. This is certainly an area, sir, that was of great interest to me when I was in FDA. We were thinking about how could we use data, the same data, in support of questions of safety and effectiveness that FDA has while also questions about coverage and implementation for Medicare beneficiaries that CMS has.

I see that we can really help FDA and CMS understand the value of working together with the tools needed and help to develop both the tools and the methodologies needed to do that and also to be able to evaluate data from those different perspectives across time.

There is a huge opportunity here to not only make this continuous evaluation of interventions become a reality, but also develop the method that we can apply to other parts of the personalized health care setting.

*Mr. Guthrie. Okay. Thank you.

Dr. Hood, you mentioned your partners as you were moving forward. I think some nonprofit and Google for-profit. Can you explain why it is so important to take a public-private approach whenever we are advancing complex health care research initiatives that involve large data sets?

*Dr. Hood. [Microphone turned off.]

*Ms. Eshoo. Doctor, you need to turn your microphone on.

*Dr. Hood. Whether --

*Ms. Eshoo. There you go.

*Dr. Hood. It is going to require fundamental changes in how industry looks at this, and we see this project as an opportunity to recruit industry in to catalyze this change, have them be a participant of it, and put it in the context of how in the future they are going to direct their efforts

to be a part of this big swing from disease orientation to wellness orientation.

And I will tell you it is going to come. It is a question of how long, and together we can really accelerate this process.

The really important point is if you move to wellness, you create enormous cost savings, and you can transfer what you have saved into creating active opportunities to really emphasize wellness and emphasize prevention.

And I think this is the opportunity Google saw. This is the opportunity Kavoit, one of our partners, has seen, and I think this is the opportunity that Guardian Research Network and Posit have all seen. They will be a part of the new revolution in medicine that is pointed toward health care.

And the only question is are you going to get in at the leading edge or are you going to be dragged in behind.

*Mr. Guthrie. Thank you.

My time has expired.

*Ms. Eshoo. The gentleman yields back.

The chair is pleased to recognize the chairman of full committee, Mr. Pallone, for his five minutes of questions.

*The Chairman. Thank you, Chairwoman Eshoo.

Modern medical researchers have a tremendous amount of data available to them, but not all data is relevant, reliable, or useful evidence for proving a product is safe

and effective to regulators.

So, Dr. Abernethy, my questions are to you. I wanted to ask you about how FDA can determine the appropriate use for available data when making regulatory decisions.

And first, you mentioned in your testimony that considering real-world data in addition to randomized clinical trial data can improve scientific decision making.

However, real-world data introduces a number of variables including data sources and quality, and while one size does not always fit all, it's important for regulators like FDA to have standards for data quality before considering it in the context of a regulatory decision.

So let me ask first how can FDA develop clear standards for how the agency should assess real-world data and should these standards differ by the type or source of data?

*Dr. Abernethy. Thank you very much for this important question.

Well, so first of all, 21st century cures compelled FDA to start to sort out to use real-world data; did so within the context of two critical use cases, extending labels, making label expansions and also post marketing commitments and requirements.

In December of 2018, FDA, therefore, put forward a framework of how to start to consider to use real-world data, specifically understanding data that are fit for purpose,

understanding analyses are fit for purpose, and demonstration of when these analyses could be done.

But this was not FDA saying we will use real-world data, but to learn how.

In September of this year, FDA put forth a guidance on the expectations around using real-world data in the context of regulatory decision making, and that guidance came back to exactly what you said, Mr. Pallone, the importance of understanding data quality and understanding when data can be fit for purpose, for especially high-risk questions and concerns that FDA has.

I still think we have a lot of work to do to develop the methods, develop the data sets, to develop an understanding of when we can leverage both real-world data and real-world data combined with clinical trial data to make confident decisions.

But I see progress, and I see that we have the opportunity to do this better and better in the future, and FDA will be a critical part of making that happen.

*The Chairman. Well, thank you.

Now, your testimony also mentioned the use of patient experience data to generate evidence. However, here, too, FDA has reported that patient experience data submitted with applications can vary widely in quality and completeness and relevance.

So is there anything that can be done? Well, two questions. Is there anything that can be done to improve the quality and relevance of patient experience data?

And how can regulators ensure the patient experience data is free from bias?

*Dr. Abernethy. So patient experience data is critical for hoping to put the medical interventions into context and also understanding how do medical interventions change our experience within the context of our own health, our own lives.

The challenge with patient experience data is that it is by definition subjective that is about experience, and so we have critical work to do to both make sure that we can collect patient experience information, for example, direct patient reports, in as complete of a way as possible.

So leveraging, for example, software technologies that meet patients where they are and allow the collection of complete information directly from people in their homes rather than needing to come to clinic.

We need to make sure that as patient personal information or patient experience is being collected, we understand the reliability and the credibility of that information and how it, for example, corresponds with increasing fatigue and decreasing ability to function and move along in real life.

And then how can that information be used to make credible assessments of the performance of medical interventions?

This is an area that we cannot let go but is going to need continued technology development, scientific methodology development, and understanding of how to integrate that into regulatory decisions.

*The Chairman. Now, you may have touched upon the last question, but let me ask it an easy way. Well, what lines, if any, should FDA draw around patient experience data?

And, you know, should it be considered in regulatory decisions?

*Dr. Abernethy. So, first of all, the ability to consider patient experience data should be in the context of any regulatory decision, at least to help understand and put that decision into context.

Whether patient experience data, patient reported information is the specific endpoint that the regulator makes the decision on for that particular treatment really depends on the credibility and essentially the qualification of that endpoint using rigorous scientific methods.

But the ability to incorporate the understanding of how an intervention works for real people in their experience can be a part of the complete story for any particular evaluation.

*The Chairman. Oh, thank you so much.

Thank you, Madam Chair.

*Ms. Eshoo. The gentleman yields back.

The chair is pleased to recognize the ranking member of the full committee, Mrs. McMorris Rodgers, for her five minutes of questions.

*Mrs. Rodgers. Thank you, Madam Chair.

Dr. Hood, your innovative approach to predictive preventive, personalized, and participatory framework that embraces wellness and early detection is fascinating. It is encouraging to imagine a future in the United States where biomedical leadership syncs up with a community-based, personalized and preventative system to capitalize on our country's leadership in biomedical foundations.

I am concerned that that foundation is in jeopardy and potentially being dismantled by the proposed government price controls. It selectively applies a negotiating process to certain drugs at staggered intervals and cumbersomely applies these price controls to new indications as well.

Would you share more with us of what you mentioned in your testimony as developing clinical research approaches and data resources that are disease agnostic, and any insights you might be willing to give as far as the impact of price controls that push investment in certain diseases over others and certain indications over others, and how much that might

disrupt the opportunity ahead of us?

*Ms. Eshoo. Doctor, your microphone please.

*Dr. Hood. [Microphone turned off.]

*Ms. Eshoo. Is your microphone on, Doctor? Oh, okay.

*Dr. Hood. Can you hear me now?

*Ms. Eshoo. Yes.

*Dr. Hood. Okay. Sorry.

*Ms. Eshoo. You have to turn it on each time.

*Dr. Hood. With regard to drug-controlled prices, I think there is a much better way to approach it, and I will tell you. Out of the data we are going to be generating on each individual, we will be able to get biomarkers that tell which patients can respond to which drugs.

The fact is if you take the ten most common drugs today sold in the U.S., less than ten percent of the patients respond properly to those drugs.

With these biomarkers, we will be able to match patients against drugs and get 100 percent results.

The cost of drugs today is roughly $600 billion a year. So if we could save 90 percent of that, and that is, you know, a hypothetical stream, that would be a very powerful way to deal with drug efficiency and to think about changing the price of drugs. Okay?

I think the second question of this program being disease agnostic is really an important point because, for

example, there are 7,000 rare diseases that have been defined to date. Ten percent of the American population has one of these rare diseases. Eighty percent of those diseases comes from a single defective gene in the cases that have been studied.

And often if you know the gene at birth, you can begin to make accommodations that do not imprison the patient in that defective gene for the rest of their life.

And we will be looking at entire populations with no bias for disease whatsoever, and we will identify the correlations, and we will have the data to begin getting even deeper insights into therapies for all of these rare diseases.

Exactly the same is true for rare recessive mandilion diseases like hemochromatosis or so forth. At birth we will be able to diagnose those and we will be able to make modifications that will improve the health.

One of the things that is really missing in today's data is the deep data approach from the infant up to 16. Those early stages of childhood development we have very little data on, and if we were to have a similar program on that initiative, it could be transformational for how we deal with kids.

So the agnostic disease looking kind of at everything -- and you can do that with a million patients,

that is why we chose the number -- is, I think, going to transform the whole landscape of how we deal with diseases.

*Mrs. Rodgers. That is great. I appreciate your insights.

As the chair said, I think we all have more questions that we could ask. I think I am going to yield back at this point and look forward to hearing from more.

Thanks.

*Ms. Eshoo. The gentlewoman yields back.

The chair is now pleased to recognize the gentlewoman from California, Ms. Matsui, for her five minutes of questions.

*Ms. Matsui. Thank you very much, Madam Chair, and I want to thank the witnesses for being with us here today to testify at this very important hearing.

I want to ask about something that we have not talked about here. Advancement in the field of computer science and artificial intelligence paves the way for health care innovation, delivery, and outcomes.

For example, just a few years ago researchers at UC-Davis Health and UC-San Francisco developed an artificial intelligence algorithm to teach a computer to define the hallmarks of Alzheimer's disease and human brain tissue.

During the pandemic, engineering researchers for UC-Davis demonstrated that artificial intelligence algorithms

may be useful in protecting newly infected COVID-19 patients on ventilators from developing serious long-term lung injuries.

These are really noteworthy achievements and hold a lot of promise for the future of health care. However, challenges remain in addressing biases within the machine learning and artificial intelligence.

Dr. Butte, there have been calls for increased accountability and transparency in coding and computer science. What biases exist in artificial intelligence and what are their effects on health care?

Dr. Butte.

*Dr. Butte. All right. Thank you. Great. Thank you for the question.

So all of us as computational innovators have to do better by working with data in an open, fair, accountable, and governed way. And as your question states, collecting more data is not just the challenge. Making sure we collect data in a fair, responsible, and transparent way and ensuring the data collected properly represents all of our patients is of utmost importance.

As an analogy that I use, imagine considering the purchase of a self-driving car that was only trained on roads in Mountain View, California. You would never accept such a car that did not know how to run in deep snow or blinding

rain.

So similarly, we should never utilize a self-driving medical algorithm trained only on a quarter of American patients.

*Ms. Matsui. Okay.

*Dr. Butte. Go ahead.

*Ms. Matsui. Mr. Butte, thank you.

I can understand that analogy, but how can these biases be addressed and eventually eliminated?

*Dr. Butte. We have got to know; we should know and document what is in the algorithms we are building, sharing, and buying, and ensure that they are trained on data covering the diversity of Americans. That is of utmost importance.

*Ms. Matsui. Okay.

*Dr. Butte. Further, we need to make sure they are engineered by data scientists that cover the diversity of America as well, and I think the NIH AIM-AHEAD program is a great start by NIH and should be continued, renewed, and grown.

*Ms. Matsui. Okay. Thank you, Dr. Butte.

And while I have you, I want to quickly touch on related and important work coming from my district. I am enormously proud of UC-Davis, how it participates in NIH and all of its precision medicine network.

Can you share how the use of data from initiatives like

All of Us is improving care for patients?

How do we make sure all of these networks are tools to address health disparities?

*Dr. Butte. I am really proud of what we have accomplished in the All of Us Research Program in the State of California. Dr. Anton Colburn and others have led this effort to register nearly 50,000 participants so far at UC-Davis and across the UC Health System, and importantly 80 percent are considered underrepresented in biomedical research and 53 percent are racial minorities.

We ourselves have already used this data to study how specific drugs are being used and prescribed in Americans, especially looking at the differences in how these drugs are prescribed across races and ethnicities.

We can study these drugs like antihypertensives and diabetes drugs and compare them with each other to find the better, safer, and more cost-effective drugs.

*Ms. Matsui. Okay. Thank you, Dr. Butte, very much.

A technology like this brings tremendous potential to advancing health equities.

I have a question here. Dr. Abernethy, I want to follow up on the chairman's questions about patient experience data. This Congress I have introduced the BENEFIT Act, legislation that would require FDA to consider patient experience and patient-focused drug development, PFDD, data within the

benefit-risk framework for drug approval.

Stakeholders, particularly patient groups play a big role in collecting this data. You mentioned the importance of credibility in collecting patient experience information. What suggestions do you have for how these groups can better work with FDA to ensure data collected is useful to overall evaluations.

Dr. Abernethy, quickly.

*Dr. Abernethy. Thank you.

Patient experience data is critical, and the patient advocacy space as well as public-private partnerships that incorporate FDA is critical to not only make sure it is clear what FDA needs to understand better and how to prioritize the patient's voice, but also to figure out how do we develop the tools and methodologies going forward in the future. The patient voice is critical.

*Ms. Matsui. Okay. Thank you very much.

And I yield back.

*Ms. Eshoo. The gentlewoman yields back.

It is a pleasure to recognize the gentleman from Michigan, Mr. Upton, who has been at the forefront of helping to make the investments so that we have a brighter health future in our country.

You are recognized for your five minutes, sir.

*Mr. Upton. Well, thank you, Madam Chair.

Earlier this week I had the chance to visit with some of the NIH folks and others, and I am so excited about what is coming about.

And for me in my district, it just so happens Pfizer is my largest employer, and that is where they package and manufacture and put together the vaccine, and because of the 21st Century Cures Act, it is pretty clear that we actually got a vaccine approved probably eight or ten months earlier than it otherwise would have been, saving literally hundreds of thousands of Americans.

So it is really exciting for me to be there as they play such a prominent role.

Dr. Abernethy, as a follow-up to the 21st Century Cures Act, as you know, we have introduced H.R. 6000, 2.0. It has a real focus on increasing the use of real-world evidence in the regulatory decision-making.

Can you tell us a little bit about how we can further maximize and expand the use of RWE to help new treatments for patients?

*Dr. Abernethy. Thank you very much.

Thank you for 21st Century Cures and --

*Mr. Upton. You can thank everybody here. It passed in this committee 53 to nothing.

*Dr. Abernethy. I am so proud to be sitting with all of you here today. So thank you for 21st Century Cures.

And thank you for the conversation around the potential of extending into the future.

Real world data can be a part of the conversation and the decision making at FDA and really across personalized health care in a number of ways.

First of all, as highlighted, we need to continue to develop the data sets and make sure the data sets are appropriately fit for purpose.

We need to make sure that we continue innovation in that space and that FDA has the opportunity to see not only what is possible today but comment on and continue to point forward with what types of innovation in the data space we are going to need for tomorrow, including incorporating patient experience data.

We also need to continue to innovate on the analyses that are possible going into the future. So how do we make sure that we have confident output that can then inform not only decisions based on real world data, but decisions that can combine real world data with clinical trial data so we can get a complete 360-degree view using totality of the evidence?

Also importantly I think as seen, we can now start to think about how do we leverage solutions such as decentralized clinical trial solutions that meet patients where they are in the home, but also pair that information

with information coming in directly from, for example, clinical research sites that we are able to get a complete view of the patient and the data really through the ability to leverage technology.

*Mr. Upton. Well, that is what we hope to accomplish with our provisions regarding RWD.

Quickly, one common refrain that we have heard so often from patient groups is that CMS has taken such a long time to make payment decisions on new drugs that make it through the approval process at FDA.

Are there ways or what ways do you see where we can have FDA and CMS better communicate so that once a drug is approved by the FDA, in fact, it will make it through the payment process quicker?

*Dr. Abernethy. So certainly the ability to make sure that Medicare beneficiaries have access to interventions that work is critical.

One thing that I think that we can do is align CMS and FDA thinking, especially through data. How can the same data sets that FDA is already requesting for continued evaluation of interventions post approval now also provide the confidence to CMS that they need to understand how this intervention is going to perform across time for Medicare beneficiaries?

I think we can begin to develop solutions that leverage

the same data for both tasks, but really give capabilities to those two different agencies to do their work.

*Mr. Upton. Again, I hope that we can see that happen when we get this thing moving along.

Dr. Hood, a quick question as a follow-up to Ms. Rodgers' question about the All of Us initiative.

You talked a little bit about youngsters one to 16 and how it is so important to measure that data. Where are you in terms of not the demographics but the actual numbers of ages one to 16?

What emphasis do you have on collecting that database from those youngest, I will say?

*Dr. Hood. Well, I can tell you we have just started talking with Kaiser about the possibility of setting up a longitudinal phenome analysis of just that population group, to begin taking in some of the data that we have talked about here.

I would like to add just one comment about real-world data, and that is one should realize the longitudinal phenome analysis, for example, of 6,000 different blood elements gives you entirely new spectrum of real-world data, and what those 6,000 elements let us do, because blood base all of the organs and they secrete informational molecules into the blood, is having a global assessment of your internal as well as your external health and so forth.

So real-world data is going to expand exponentially in the future as we bring more and more people into the longitudinal phenome analysis, and the pediatric examples, I think, will just change the whole spectrum of how we view disease in the pediatric population.

*Mr. Upton. Thanks very much.

I yield back.

*Ms. Eshoo. The gentleman yields back.

It is a pleasure to recognize the gentlewoman from Florida, Ms. Castor, for your five minutes of questions.

*Ms. Castor. Well, thank you, Chair Eshoo, for holding this important hearing.

Thank you to our witnesses for sharing your expertise. It gives us an opportunity to mark the 20th anniversary of the Human Genome Project. So to Dr. Hood and all of the scientists and all of the policy makers who play an important role, thank you for your remarkable contribution.

And when you look out, think about the next 20 years. It is very important for us now to concentrate on how we combine this, one of the greatest feats in recent scientific history, the sequencing of the human genome, now with advances in bioengineering, biomedicine, to prevent and cure disease. So the opportunities are enormous here.

I am fortunate to represent a top health innovation center in the Tampa Bay area anchored by the Moffitt Cancer

Center and the University of South Florida Health.

Moffitt has been leading the charge on precision medicine and immunotherapy for cancer patients. For example, scientists at Moffitt have been working on a range of research to better understand how the immune system can successfully fight cancer.

But I have also heard from folks who think we are making a lot of advances on precision medicine when it comes to cancer but maybe not other diseases. Dr. Thomas McDonald from USF highlighted to me that while we have made great strides in personalized therapies for cancers, we are kind of lagging behind in other diseases.

His laboratory established a cardiogenic program top search for novel therapeutics for hereditary heart disease.

Do you agree that we need to focus more maybe?

Dr. Abernethy, I will start with you.

How do we ensure research tackles broadest, deepest health problems?

Where are we headed?

*Dr. Abernethy. Thank you very much.

As a fellow Floridian, I appreciate this question.

I certainly think that we need to take the lessons learned in the cancer and rare disease space and now start to apply to all therapeutic areas.

Practically speaking, it may not always be the same

science and the same methodologies, but the premise, the premise to individualize and to tailor, to blend the best of what we understand of biology on a personhood together to figure out treatments, I think we can take to all therapeutic areas.

*Ms. Castor. So NIH, they had to strike this balance before, and I guess our funding decisions will address that as well.

And maybe, Dr. Minor, you could address that a little bit, and I also want you to focus in on the advances due to CRISPR technology.

I have been fascinated by this. I have met with Dr. Jennifer Doudna at Cal-Berkeley across the bay, rap SU, about the significant or the potential advancements across biology, medicine, agriculture.

If you just take a peek at what is going on now with CRISPR, sickle cell clinical trials; mosquitoes, how we reduce the spread of malaria; impact of climate change on crops in agriculture, vision and blindness.

Dr. Minor, talk to us about this and the enormous opportunities. Is ARPA-H a way to help advance CRISPR technology and all of the advances there?

And what else do we need to keep in mind?

*Dr. Minor. Well, thank you, Congresswoman Castor.

Yes, I think ARPA-H offers tremendous promise in making

the types of advances that you have described.

And one of the reasons I think that we have seen so much progress as you outlined from the great institution in your district and other places in cancer immunotherapy is that for basic research, we have a much better understanding today than we did as recently as five years ago of the immune mechanisms and cancer, and that enabled the development of these immunotherapies in many other diseases, most notably degenerative neurological diseases.

We do not have nearly as complete an understanding of the basic mechanisms, and that underscores again the importance of basic science research in driving the advances that then fuel better therapeutics and diagnostics in the future.

I agree with you that CRISPR is truly transformative technology. We want to make sure that it is used ethically and with appropriate standards and regulation from the government and certainly with input from bioethicists.

But as you so well described, it has the potential to improve our development of crops, to extend the productivity of so many things in the agricultural space as well as to treat both genetic, clear monogenic disorders that Dr. Hood described, but also treat disorders that have a genetic basis, of which most do, such as cancer, degenerative neurological diseases, to be able to treat those much more

effectively than we have in the past.

We have only begun to scratch the surface, and I am very excited about the future of CRISPR.

*Ms. Castor. Thank you very much.

*Ms. Eshoo. The gentlewoman yields back.

She mentioned UC-Berkeley. I'm very fond of saying that I get to represent the greatest private university in the world that lives in the shadow of the greatest public university in the world.

With that I recognize one of the doctors on our subcommittee, Dr. Burgess, for his five minutes of questions.

*Mr. Burgess. I thank the chair.

Dr. Abernethy thank you for being with us today, and thank you for your time. Speaking with your colleagues at Verily and Alphabet earlier this year on a Zoom call certainly gave me some significant insight, and I appreciate the fact that you have spent some time in the belly of the beast over at the FDA, and you may have some insights that you are able to share with us.

Operation Warp Speed I think we all acknowledge was a significant win, and a vaccine was worked on and produced within what seemed to be a very short period of time, but are there lessons that you can help us with?

We have got an FDA reauthorization bill coming up I mean literally in hours where we are going to need to be working

on, and it has got to be done by the end of July. So the time frame is fairly short, but the good news is that gives us an opportunity to talk to your former colleagues over at the FDA a lot about things because the user fee agreements are what allows for probably 75 percent of their funding.

So help us with the lessons learned from Operation Warp Speed and how we might incorporate that into our better understanding of the work of the FDA.

*Dr. Abernethy. Thank you very much, sir.

The interesting thing as we look back across the pandemic, we have seen some incredible work that has really helped with managing the care of our population, but also pointed towards the future.

As I reflect on your question, I think that this story has taught us the importance of not only making sure that our discovery engines are working as quickly as possible and we are scaling the regulatory apparatus, but we have really not only revolutionized clinical trials, but really taken this fast forward so that we can make sure that clinical trials are being conducted as quickly as possible and also incorporate and meet all people where they are.

Another thing that is going to be important as we think about FDA going forward is to make sure that FDA is enabled with the tools of evaluating the totality of the evidence, clinical trials data, and real-world data, and we certainly

saw that in the context of the pandemic.

We also saw in the context of the pandemic the opportunity to make sure that when possible and when needed that we can move forward with thinking about manufacturing early so that we can be ready with solutions when people need it.

So I think there is a number of things to learn from the pandemic.

*Mr. Burgess. Thank you.

And we will certainly look forward to your continued participation as we go through that process.

Dr. Hood, I want to just ask you. You mentioned you were working on -- and thanks for bringing up the four Ps, by the way. My very first meeting when I started this committee years ago was with Dr. Zerhouni, and he articulated about the four Ps, and the participatory part of that is probably one of the most important things.

But you mentioned the ability to optimize aging, and I am just wondering do you have an app for that.

*Dr. Hood. The optimization for aging.

*Mr. Burgess. With your longitudinal look at the genome with the phenome?

*Dr. Hood. Yes. The algorithm that we have developed right now employs about 50 metabolites, and those 50 metabolites not only give us the ability to estimate your

global age, okay, but we can estimate the age of your major organs, your immune system, your heart, and your kidney, and so forth.

And from the metabolites --

*Mr. Burgess. Let me restate the question. Do you have an app for that? I may not live long enough to hear the answer to your question.

*Dr. Hood. Yes, yes. We do have an app for it, and I can send you where you can get your biological age done, but the key thing is to do it repeatedly because you want to show that you are going down rather than going up.

*Mr. Burgess. I do not need to know that. I actually know that.

Dr. Minor, I just wanted to ask you quickly. You brought up the ARPA-H and, as you know, there are a couple of proposals before our committee introduced by members, and you have heard the testimony of Dr. Abernethy. One of the things that we are going to need the most amount of help with, as I see, is just what the up-front funding and the basic research, but you mentioned translational, how you get through the guardians at the FDA, how you get past the bureaucrats at CMS for coverage determinations.

All of these things almost need to happen simultaneously, and now we have set the public expectation with Warp Speed that we can, in fact, get things done

quickly.

So could you help us with that?

*Dr. Minor. Well, Congressman, I think you described it really well. I think we did set the expectation with Warp Speed. We showed what is possible by bringing together the various branches of government, various government agencies in collaboration with public institutions and with industry.

I think that expectation will continue, and so far as the success of ARPA-H, you know, DARPA probably serves as a good model overall in that DARPA serves the Defense Department, is charged with innovating in ways that will improve the ability to defend our country and protect our troops.

Similarly, ARPA-H will likely have a similar charge for doing the same thing in health care and the health and wellbeing of America.

I am optimistic that because of what we demonstrated is possible during COVID with the development and deployment of vaccine that a similar mandate will exist for ARPA-H.

*Mr. Burgess. Thank you.

Madam Chair, I yield back.

*Ms. Eshoo. The gentleman yields back.

The chair now is pleased to recognize the gentleman from Maryland, Mr. Sarbanes, for your five minutes of questions.

*Mr. Sarbanes. Madam Chair, thanks very much, and

thanks for the hearing.

Like all of us, I hear from constituents about the need to fund and advance research on a variety of diseases, whether it is cancer, diabetes, or other diseases and what we can do to detect and treat and cure those diseases, improve the quality of life for our families and communities.

I also bring the perspective of being an original cosponsor of the critical bill, the Henrietta Lacks Enhancing Cancer Research Act, which as you know directed the GAO to complete a study and provide recommendations on how Federal agencies can address barriers to participation for underrepresented populations in federally funded cancer clinical trials. And this bill became law earlier this year.

Mr. Falcon, could you speak to the critical need, again, for increased diversity that you laid out in your testimony? And particularly the importance of the All of Us Research Program which we have heard a little bit about today already.

But this program aims to build a diverse biomedical research database on health information. How can that program, the All of Us Research Program, inform our knowledge of differences between and among certain types of cancer or other diseases?

*Mr. Falcon. Well, first of all, Congressman Sarbanes, I would be remiss if I did not thank you and the committee for the Henrietta Lacks Act because it has helped put a focus

on the importance of diversity.

Yes, All of Us is an important national resource. We are working to get to one million persons enrolled. We are a little bit over 400,000. There are over 300,000 bio samples collected, over a quarter million electronic health records collected.

But the central feature is that equity was at the organizational core of All of Us. It was part of the mission statement. It was part of the metrics that were measured. It was part of the funding for community engagement partners.

It showed that equity can be done in health research because over half of the participants are members of racial and ethnic groups that have been previously underrepresented in biomedical research.

This was a significant change, and it can happen with ARPA-H as well. I am pleased to see that the proposals for APRA-H do have equity explicitly stated as a goal of APRA-H, and that kind of organizational capacity around equity makes research happen.

I would also point out to the committee, and I think Congressman Ruiz and others, that as clinical trials are advancing, we are moving more and more to decentralized clinical trials.

But as we make that move, we are creating another barrier for underrepresented populations, and that those

decentralized trials depend in large part on technology and being able to have access to technology.

And we do need to take care and create some safe harbors for clinical trial sponsors to be able to provide funding, to provide the technology, but also funding in order to participate around transportation, childcare, and all those other issues as we move to decentralize trials.

*Mr. Sarbanes. I appreciate very much that observation because, as you know, we can suffer up here a lot from unintended consequences. So as we rally around the idea of decentralizing, it is important to have you flagging some of the new obstacles that can present, and we have to have strategies for getting over and around those obstacles.

Dr. Abernethy, as we continue to collect health information and employ these innovative technologies, can you speak a little bit about the ethical or privacy considerations that we should be thinking about in terms of future policy making?

*Dr. Abernethy. Thank you very much.

As we think about leveraging new solutions and technologies to make clinical trials easier to participate in, we need to make sure that we stick to our core principles of privacy, of security, and making sure people, real people, understand what they are participating in, and have a full understanding across the time of their participation,

including how their information is going to be used.

I think we should be leveraging the best of what software development and technology has to offer with respect to user experience and user design so we can get that right.

And we also need to reduce the burden of participation for all people in order to be able to be in clinical trials.

*Mr. Sarbanes. Thanks very much.

As I conclude, Madam Chair, I would note there is some intersection there with that last observation with Dr. Falcon's or Mr. Falcon's observation.

As we decentralize, we have got to make sure that people who are participating in these trials understand fully what that participation means and that their interests are being absolutely protected.

With that I yield back.

*Ms. Eshoo. Excellent points.

The gentleman yields back.

The chair is pleased to recognize the gentleman from Virginia, Mr. Griffith, for his five minutes of questions.

*Mr. Griffith. Thank you very much, Madam Chair.

Thanks to all of the witnesses here today.

I have been listening to the testimony, read some of the written testimony as well, and want to note that several of you who have expressed support for bills that would increase NIH funding.

I am a long-time supporter of funding for NIH because of the important work they do, and as my fellow committee members know, I am currently participating in an NIH study on those who had mild cases of coronavirus or COVID-19, and I think the NIH team is great. They have been doing great work on that. I have been very impressed.

However, in my role working on the Oversight and Investigations Subcommittee of this committee and on this committee, I have been disappointed by the agency's disregard of requests for information and documents on the origins of COVID-19.

Members of this committee have sent five oversight letters to the NIH and have not received a response to our concerns and questions.

They have been unresponsive to Congress on vital questions, and that is not acceptable. I think any discussion about additional funding and new programs has to be coupled with an understanding that while the NIH has the job to do the science, we have the job to do the oversight and to make sure that what is being done is being done in accordance with the principles and the guidelines and so forth that we have set out.

And if somebody has made a mistake, and mistakes will happen in every organization, that we have the ability to look at it and try to figure out how do we do it better going

forward.

That is our job, and so I have been very concerned about that, and here is the problem when we do not get answers. I know you all cannot answer any questions. I am going on a little diatribe. I apologize to you all for that.

But here is the problem that I have and I think lots of others have. If we cannot get somebody to get us answers to the questions that we have, then we must assume that the answer is the worst-case scenario, and then we have to act accordingly.

All right. That being said, I am done with the diatribe and I appreciate you all letting me do that.

Dr. Abernethy, you talked extensively about personalized medicine and incorporation of real-world data into medical care. So I am going to go right into what would have been high tech ten years ago but is now becoming low tech.

My watch, a friend of mine has a better one than I do or his son did, and it picked up and I do not know how; probably with AI that you talked about in your testimony as well. It picked up an afib about a year ago. Went in to see the doctors immediately. They could not find anything. Then he went for a much more extensive test. Sure enough they found a heart valve problem, and even though the family had been told it was not genetic, it is the same heart valve problem that killed his mother a couple decades ago.

They were able to go in because they identified it. They were able to go in and fix it, and he is fine. Everything is going to be great.

But that came from a wearable. What are your thoughts on wearables?

And I also would note that you indicated with our studies we have to go to the people. Is it possible we can use wearables in that regard, too, to get the data instantly, but also get feedback from wearables?

*Dr. Abernethy. Thank you for the question about wearables.

Practically speaking, being able to leverage all available data, including the sensors in our watches, is going to be a critical way of being able to not only collect information across time, but also do so in a way that helps to personalize, as you described that story being able to leverage the heart-related information to now direct care, but also do so in a way that reduces the burden of a person having to fill in a form or enter information through secondary purposes.

We have a lot of work to do though to make sure that the information coming, for example, from a wearable is reliable, does not create biases that would amplify disparities in our health care system, and that we understand where to switch out data points, such as an endpoint from a wearable in the

sensor in your watch as opposed to the way we traditionally conduct clinical trials.

I suspect we have got a lot of work to do across time, but this is a huge area of opportunity.

*Mr. Griffith. Well, I envision we may have the opportunity to cut down on the number of visits, say, to the NIH, particularly if you come from a distance and you are perhaps living in a rural area. We could combine maybe the wearables with telemedicine as well, and things I probably have not even thought of yet.

But thank you all so much for being here.

Yes? Did you have another comment? I have got a couple of seconds.

*Dr. Abernethy. I was just about to say that the combination of telemedicine, wearables, et cetera, not only help us get more information data. They meet people where they are, and they reduce the burden of participation. We see that as really important for the future.

*Mr. Griffith. Absolutely.

I yield back. Thank you, Madam Chair.

*Ms. Eshoo. The gentleman yields back.

The chair is pleased to recognize the gentleman from Oregon, Mr. Schrader, for your five minutes of questions.

*Mr. Schrader. Thank you very much, Madam Chairwoman. I really appreciate it.

I guess I will start off by making some comments on the prescription drug bill that is incorporated in the PPP plan because of the inaccuracies, inflation comments, unfortunately that I have heard here today regarding what it is all about. That prescription drug bill is an excellent, excellent balance between H.R. 3 and what others would perhaps like to do. It incorporates most of H.R. 19 that my colleagues on the other side of the aisle have referenced.

Our problem solvers group that is made up of Republicans and Democrats looked at H.R. 19, looked at H.R. 3, tried to figure out a good balance between these to not stifle innovation, but make sure that people could afford the medication that we are talking about here today that are truly lifesaving for a lot of folks that are out there.

It incorporates Part D redesign, the insulin caps, frankly, the biosimilar ASP plus eight provisions, trying to get at some of the key gaming of the system that goes on by the pharmaceutical industry, but at the same time respecting the fact it costs a lot of money to innovate these drugs.

I want that to occur in America. I want it to occur in the districts that some of these members in this room represent, and I think we found that balance.

The idea that it is going to cut off all innovation is ridiculous. H.R. 3, yes, it had a greater impact on the number of drugs that might come to market, according to the

Congressional Budget Office. I respect that, and that was our goal, to respond to that.

Our bill, according to the Congressional Budget Office, might result in maybe one drug over the next decade not coming to market. All right. That is one drug. I get that, but I will balance that any day against seniors may not be able to afford the medication.

We reduce the out-of-pocket expense for seniors to $2,000 a year that they can pay over the course of the year to make it as affordable as possible to them. They are no longer subject to the donut hole that they are on hook for thousands, tens of thousands of dollars for some of these very exciting new, innovative drugs that unfortunately cost quite a little bit.

The same thing, insulin is capped at $35. That is huge. That is huge for seniors. Diabetes is -- I am a veterinarian. It is prevalent in the animal population, prevalent in the human population, absolutely critical to prevent that sort of thing from becoming endemic in our population.

And limiting the cap on everyday drugs we have used for years to the price of inflation when they try and jack the cost up on us. I think that is a good deal. That is great deal for Americans, Oregonians, being able to afford medication.

So the idea that this is stifling innovation is completely complete you know what and inaccurate, and I resent the fact that that is being put out. We have incorporated a lot of my Republican concerns. It is a really good bill, and I hope it actually passes the Senate. It is a boon for our America's seniors to enjoy quality of life along the lines of what we are talking here.

Dr. Hood, if I could ask you a question here. I am really excited about the work you are doing. The phenome thing is critical. Medicine is a lot more than about just the disease entity itself. It is about the environment. It is about these predispositions, all those things.

So it is very, very exciting. What is the role between NIH and industry in getting involved in the phenome project itself?

*Dr. Hood. Well, we have talked with the All of Us Program at NIH, and conceded we have really complementary possibilities. We are exploring it, and we plan to work together in the future.

I think the industries that we have brought in that are going to enable us to get this program started very early on are going to push us to points where we can begin delivering the longitudinal phenome data that will push us towards wellness but will let us really transform diseases.

And, again, let me just say this agnostic view of

disease is interesting because with a million people, we will have hundreds of thousands of people with the major diseases, diabetes, cardiovascular disease, cancer, Alzheimer's. Okay?

From the data we get, we will be able to create and identify I would guess the 20 to 40 different subtype each of those major diseases have, and each of those will be targets for new drug approaches.

*Mr. Schrader. Very cool. If I may, I am sorry to interrupt, but a question, like a little bit now and, of course, later because my time is running out, but how do we adopt lifestyle and environmental concerns into what we do in the pharmaceutical domain and how we approach patients and their medications without unfortunately discriminating.

I mean, some people have genetic predispositions, certain races, certain cultures.

*Dr. Hood. Absolutely.

*Mr. Schrader. How do we improve outcomes without discrimination?

And you can answer that later or --

*Dr. Hood. Again, the key point I want to make is this Guardian Research Network. Why does it cross all of the major ethnic groups? And we will put them into the million-person project in proportion to their level in the general population.

And with a million people we will get a lot of data on

Latinos, on Blacks, and the whole thing. So you are absolutely right. We need that data because we treat different people different ways according to what their genetic predispositions are.

And the same may be true of these diseases like diabetes and Alzheimer's. We may see different tendencies in different groups, and with the million-person project, this will come out absolutely beautifully in a non-selected manner that gives you what you see in the population.

*Mr. Schrader. Very good. Thank you very much.

I yield back. Thank you, Madam Chair.

*Ms. Eshoo. The gentleman yields back.

I just want to take a moment to salute the gentleman from Oregon for the important work that he has done to lower the price of prescription drugs for the American people. The costs are simply unsustainable.

And when this becomes law, everyone's constituents, Republican constituents, Democratic constituents, those of us that, you know, who are Republican members, that are Democrats. All of our constituents are going to benefit from it.

And I doubt then that my Republican colleagues will go out and say that this is a bad thing.

So with that I would like to recognize the gentleman from Florida, Mr. Bilirakis, for his five minutes of

questions.

*Mr. Bilirakis. Thank you, Madam Chair, again, for holding this hearing on biomedical research and innovation and the future of personalized medicine, so very important.

I said before that data drives decision making. I think the panel would agree that we should focus on generating quality data in order to make advances in biomedical research and quality, evidence-based decisions. This is, indeed, a key part of the puzzle to translating basic research into breakthrough cures.

With this said, I am disappointed that the Biden administration decided to repeal the Medicare Coverage for Innovative Technology, or the MCIT rule, that provides our seniors with access to breakthrough devices.

We created the FDA Breakthrough Device Program to allow for a priority review process for groundbreaking breakthrough technologies that have no approved alternatives, offer significant advantages to the existing options or availability of which would be in the best interest of our patients.

I applaud the FDA for setting up a rigorous pathway to ensure these medical devices, the device technologies, are safe and effective upon approval, and these manufacturers must collect the necessary data to show its benefit to patients.

Unlike their drug counterparts, these devices do not received coverage by CMS upon approval until they embark on a costly and year's long clinical study process. By contrast, the MCIT rule allowed for temporary national coverage under Medicare during which FDA continues to collect data and conduct its post market surveillance requirements.

This rule had broad bipartisan support, and I even co-led a bipartisan letter with Representative DelBene and Representative Cardenas and Representative Walorski that would codify coverage for these breakthrough devices.

Despite this, CMS bureaucrats decided to repeal the MCIT rule leaving these patients with less access to care. I am grateful that the leadership on this subcommittee wrote a letter to CMS asking them to reconsider their decision.

Dr. Abernethy, you mentioned in your testimony the importance of longitudinal post-market monitoring of medical products to ensure we are establishing evidence-based decisions.

Can you tell me more about the importance of incentivizing this evaluation of evidence over time?

*Dr. Abernethy. Thank you for your important question, and another fellow Floridian.

I really think that one of our core tasks as we encounter not only breakthrough devices but other interventions that can impact health and personalize our care

is that we continue to evaluate how medical products perform across time, and we generate the data sets needed for that data-driven decision making.

Importantly, evaluating medical products across time not only allows us to continuously understand safety and effectiveness but also helps us understand what is the best way for a doctor and a patient together to make the right decision for this particular patient and within the context of that patient's needs because we develop more and more data across a more diverse and richer population, as well as more understanding of which specific scenarios and what timing.

So that is this critical issue of continuing to collect longitudinal data.

*Mr. Bilirakis. Thank you.

It makes sense.

Do you agree that Medicare coverage of innovative technologies, which would lead to more widespread access, patient access, could result in more robust evidence generation in the post-market setting?

*Dr. Abernethy. It is really important that we continue to think through how we are going to get Medicare beneficiaries and others the interventions that they need, and this is an important topic for us all.

*Mr. Bilirakis. Very good.

Thank you, Madam Chair. I yield back.

*Ms. Eshoo. The gentleman yields back.

It is a pleasure to recognize the gentleman from Missouri. Oh, I am sorry. Well, you are after the next person. How is that, Mr. Long? I am sorry. I was anxious to get to you because you were the very first one in the hearing room this morning.

The chair now has the pleasure of recognizing the gentlewoman from Michigan followed by the gentleman from Missouri, Mrs. Dingell for your five minutes.

*Mrs. Dingell. Thank you, Madam Chair, and thank you, Ranking Member Guthrie.

And I am sorry to my Missouri colleague that I came in front of you because I always love hearing from you.

But I really want to thank the chair for convening today's hearing on advances in biomedicine and personalized treatment because it really, really is so critical.

But I also think it is important to note that public funding of research through institutions by the NIH is fundamental to the development of cutting-edge therapies and personalized medicine that we have been discussing today.

In fact, NIH contributed to published research for every one of the 210 new drugs approved by FDA from 2010 to 2016.

So we have got to continue to provide the resources necessary at the Federal level to translate basic research into medical breakthrough. This starts with supporting early

career scientists and investigators who are the future of medical innovation in this country.

Dr. Minor, you mention in your testimony that since 2000 NIH applications have doubled, but the success rate of those applications declined from 32 percent to 21 percent, leaving many quality proposals unfunded.

Can you discuss the importance of NIH funding for early career scientists?

What impact has this development had on those aspiring to a career in research and how can we in Congress help support early career researchers?

*Dr. Minor. Well, thank you very much, Congresswoman Dingell, for your question, and, yes, I think that the decline in the proportion of investigators supported by the NIH, the proportion of grants that are actually funded by the NIH, the decline has had an effect on our biomedical infrastructure in the country and, in particular, on young investigators.

I applaud the NIH for doing a number of things to address that, such as early career investigator awards that are reviewed specifically for the cohort of young investigators. When I mentioned young investigator now, it is a relevant term since the average age at which an investigator received their first NIH grant is now in the early to mid-40s, and that is because of the length of

training that is required to get to the level to compete effectively, even for these early career investigator awards.

There is no more precious resource, I would say, in our country than our biomedical workforce, and we are privileged to have still an extraordinarily dedicated group of young people who want to go into biomedicine and physicians, physician scientists, basic scientists.

We need to make sure that our governmental support, particularly from the NIH, rewards their years of dedicated and minimally compensated effort in order to give them an opportunity to build their research programs.

And that is why, as you mentioned, it is so important that NIH funding be increased to enable that work force to continue to thrive.

*Mrs. Dingell. Thank you, Dr. Minor.

And I do believe that my colleagues and I in a very bipartisan way on this committee will continue to prioritize support for the NIH moving forward.

In the time I have left, I would also like to touch on the issue of data privacy. We have heard from our witnesses about the potential to leverage large data sets of health information to drive advances in care.

However, we have also seen that personal health information is an attractive target in countless research institutions and health care providers have been the victims

of data theft and cyberattacks.

In fact, I myself have signed up for NIH's data collection, but was very worried about my own privacy because it can result in the theft of medical identifiers, prescription information, treatment information and other sensitive medical data.

Dr. Abernethy, you mentioned the importance of data governance and privacy in your testimony. What policies should Congress be looking at to reduce the risk of unauthorized access to personal health information, given the proliferation of electronic health records and aggregated health data?

*Dr. Abernethy. Thank you so much for this important question.

Practically speaking, we must prioritize privacy, security, and consent if we are going to advance this critical work in personalized health care. We need to make sure that we continue to look at current rules and think about what are current implications with respect to capabilities and technologies of today and how those rules are performing for us and whether or not we need to update those rules, such as HIPAA, across time.

We also need to think about how do we incentivize innovations that continue to improve on privacy and privacy sparing solutions, such as leveraging tokenization, so we do

not have to include personal identifiers and leveraging, for example, synthetic data sets that obscure personal information but maintain the ability to analyze data sets to understand important outcomes around health care intervention.

If we do not prioritize privacy, we will not be able to maintain trust and trustworthiness, which is critical to the system going forward.

*Mrs. Dingell. Thank you.

It is very important. I am out of time. I want to pursue it more.

Madam Chair, I yield back.

*Ms. Eshoo. The gentlewoman yields back.

A very important line of questioning that we need to pursue, Mrs. Dingell.

Now, Mr. Long, the gentleman from Missouri, you are recognized for five minutes for your questions.

*Mr. Long. Thank you, Madam Chair.

And if I was going to yield to anyone in the John Dingell Room, I could not think of anyone better than Debbie Dingell to have yielded to.

So you kind of set that up for me. Thank you.

Dr. Abernethy, thank you for being here to share your perspective. Can you talk about how traditional clinical trials are conducted and what makes them resource intensive

and inconvenient and overly burdensome for their participants?

*Dr. Abernethy. Thank you for this important question, sir.

In the context of a traditional clinical trial, we prespecify in a very carefully worded study protocol, like a recipe book, all of the actions that a study coordinator and a patient and other actors in the clinical trial system must take in order to collect that data.

Often it requires an individual to go to a clinical trial site. So, for example, my father needed to go to Houston if we were going to consider him for a specific cancer trial rather than his hometown of Orlando.

It also often requires a person to get an extra or many extra tests that may be duplicative of tests that they have already had in the past, such as extra biopsies or additional scans.

And then it requires a person to be followed over time, oftentimes going back to that site way far away from their hometown.

The opportunities in the future are to leverage the ability for a person to participate in the clinical trial leveraging video means or digital health solutions to collect data. However, we need to make sure that when we leverage these new solutions in the future, we do not sacrifice

participant safety or the ability to collect high quality, credible data that can answer questions confidently.

*Mr. Long. My second question was going to be on the use of digital health tools to alleviate some of the burdens, but I think you kind of covered that in that portion.

So my next question is what are the challenges to generating adequate and acceptable evidence using these new tools and trial designs and how do we overcome them?

*Dr. Abernethy. Thank you. I appreciate the opportunity to follow on here.

Practically speaking, we have the opportunity to leverage a number of tools, some of which were made possible by this committee within the context of 21st Century Cures and opportunities going forward. These tools include, for example, leveraging all available data, including data that had been passively collected in the electronic health records, claims data assessed, and tools such as the censor in a watch to be able to collect information, for example, about movement.

We have got the opportunity to leverage these tools. However, we are going to have to do the hard work of understanding how to make sure that the data are cleaned up, are of high quality, and are representative of longitudinal reflections of care that also help us understand how a person performs across time.

We also have the responsibility to make sure we develop the scientific methods that allow us to make sure that we can responsibly and reliably analyze these data sets, including when we pair clinical trial data sets with real-world data sets.

And we also have to make sure, as I mentioned before, that we critically make sure that patients are kept safe whenever being involved in clinical research and we never sacrifice the issue of patient safety.

*Mr. Long. Okay. Thank you.

And, Madam Chair, this is my eleventh year in Congress, and I have picked up recently on the fact that when you are in Congress not everyone is a huge fan, and every time I wear my neckwear today, I get people that maybe are not real big fans say, "What is that goofy tie Long has on?''

But I always like to point out this is actually the St. Jude Children's Research Center tie, and being a father of a cancer survivor, I always wear it with pride. So before everybody starts saying, "What is that goofy tie?'' now you know.

I yield back.

*Ms. Eshoo. The gentleman yields back.

I do not know anyone in Congress that does not think the world of you, Mr. Long. So just disabuse yourself of that notion, and your necktie is beautiful because it represents

something that is magnificent in our country, St. Jude's and the care that they give to children day in and day out.

The chair is pleased to recognize Dr. Bucshon, another of our outstanding doctors on our subcommittee, for your five minutes of questions.

*Mr. Bucshon. Thank you, Madam Chairwoman.

And today's hearing comes at an opportune time. Promoting innovation and advanced research in our biomedical industry is crucial for not only America's leadership in the world, but also more importantly, it is crucial for America's patients.

That is why I am concerned about the drug pricing protocols in the Build Back Better Act, and they would have the opposite effect limiting research and hampering innovation, in my view.

In fact, a recent analysis conducted by the University of Chicago found that the drug pricing protocols found in the Build Back Better Act would lead to 135 fewer new drugs by 2039.

The study also said that it would generate a loss of 331.5 million life-years in the U.S. during that time. That is 31 times as large as the 10.7 million life-years lost from COVID to the U.S.

As a physician, I sometimes had to share bad news with patients and families, and I know all too well that

eliminating just one new drug is one drug too many. What if one of those new drugs is a cure for Alzheimer's, cancer, or ALS?

Dr. Abernethy, obviously, the Federal Government plays a role in funding research to the NIH and other programs, but it cannot stand on its own. Can you explain how important private research and development is to innovation and discovery of new cures?

*Dr. Abernethy. Thank you very much for this important question, sir.

I learned during my time at the agency the criticality of public-private partnerships. When I was at the agency, I was a co-author of a committee report for the National Academy of Medicine on digital health in COVID, and what we realized was that we had data, data everywhere, but really the inability oftentimes to put it to work the way that we were hoping in managing the pandemic.

And one of the key recommendations was to build on public-private partnerships, something that we had learned as an important element when I was at FDA.

What can public-private partnerships do for us? Well, first of all, they can help us move faster by ensuring coordination across industry, across government, across academia, to solve hard problems.

They can make sure that there is learning from each

other, but also co-investment in solving problems that matter and amplify the work done by those different sectors.

The other thing is when regulators are also part of that conversation or at the table, regulators can learn and start to think about what is possible into the futured and start to think about how that might be regulated in the future so that the regulatory actions are not falling behind of what is possible in the private sector or in academia.

*Mr. Bucshon. Well, thanks for that answer.

And I think we saw during the pandemic with the development of the vaccines this cooperation between the Federal Government and the private sector and resulting in therapeutics and vaccines available in really record time.

Madam Chairwoman, I yield back.

*Ms. Eshoo. The gentleman yields back.

The chair is pleased to recognize the gentlewoman from Texas, Mrs. Fletcher, for your five minutes of questions.

*Mrs. Fletcher. Thank you so much, Chairwoman Eshoo, and thank you for convening this hearing today.

Thank you to all of our witnesses for your testimony. It has been a very interesting and informative morning.

And it is not really surprising. Apart of the reason that I was so excited to become a member of this committee and this subcommittee and this subcommittee, in particular, is because of the committee's jurisdiction over medical

research and the hugely important and interesting issues in front of us.

So I thank you very much for doing this today.

Part of the reason that I was so interested in this is because I represent Houston, Texas, and it is the site of the Texas Medical Center, the largest medical center in the world and home to some of the country's and the world's greatest researchers, and it is such a privilege to get to represent so many of them here in Washington.

Institutions like the Texas Medical Center Institution, the research institution there, the incredible care they provide, they are ready and willing to tackle some of these biggest health research challenges.

But an important piece of turning research into treatment and advancement is academic, clinical, commercial, and public partnerships.

So, Dr. Minor, I want to thank you again for being with us today, and from your testimony, it is clear that you know what it takes to conduct research both responsibly and efficiently and also what the biggest barriers to progress can be.

So I want to direct my questions and the time I have to you. Based on your decades of experience in the academic space, as you noted in your testimony, basic research served as the foundation for innovative technology and treatments.

However, translational research is also critical to disclose what strategies or further investments should Congress consider to help advance translational research and the advancement of research from academic labs to commercialization.

*Dr. Minor. Well, thank you for your question, Congresswoman Fletcher, and congratulations on representing the district that has in it the Texas Medical Center, which is a truly amazing complex, truly amazing work in all spectrums of health care, from patient care, research, and training the next generation of leaders.

I think there are several things that can be done, and many of which we have discussed today already to accelerate the translation of basic science discoveries into new therapeutics and diagnostics and improved health.

We have talked about the importance of Federal funding, also the importance of making sure that appropriately monitored and governed collaborations between academic institutions, nonprofit institutions, and the commercial sector, making sure that there are no impediments to those interactions because really to translate and to bring developments to the benefit of patients, there is a commercialization step that absolutely is essential.

So making sure that proceeds at a facile way, and I would just look back to something that Congress did many,

many years ago with the Buy Gold Act, which really fueled the beginnings of the biomedical revolution in our country and in the world.

Similarly, looking for ways that we can incentivize public and private collaborations in the future will be really important.

And finally, one thing perhaps that we have not touched on as much in today's hearing but that is critically important is really focusing on training the next generation of leaders.

You know, the level of existing scientific knowledge today compared to when I was training is unfathomably greater than it was before, and we need to focus on how we train our scientific workforce and the opportunities we provide to them to receive outstanding training.

Training grant programs from the NIH are critically important. Recently the funding for those programs has been challenged, and that is the critical pipeline that will drive innovation and drive this translation of discoveries into new therapies and diagnostics for decades to come.

So I think focusing on our training programs enabling our academic institutions that run those programs to be successful and making sure that the Federal Government at all levels is providing the appropriate support for those training program is critically important, from training

Ph.D.'s to training medical students in medical school, and also postgraduate medical education which plays a hugely important role.

*Mrs. Fletcher. Well, thank you so much, Dr. Minor, and that is certainly something that I have heard from our institutions as well, the importance of finding and training and retaining talented folks who are doing the kind of work that has payoffs today.

I am sorry that I am almost out of time to ask more questions, but I would just like to say for anyone on the panel who would like to weigh in on that question, if you wanted to submit an answer to that for the record, I would be so grateful.

And I really am grateful for all of your time today and to you, Chairwoman Eshoo, for holding this hearing.

And I yield back.

*Ms. Eshoo. The gentlewoman yields back.

The chair is pleased to recognize another outstanding doctor on our subcommittee. We are so proud of those that are. Dr. Dunn of the State of Florida, you are recognized for your five minutes of questions.

*Mr. Dunn. Thank you very much, Madam Chair, and thank you, Ranking Member Guthrie, for hosting this hearing today to discuss biomedical research and personalized medicine.

I am very proud of America's robust tradition of

innovation in the biomedical industry. We are living in a fascinating age. The success of Operation Warp Speed is a great example of the ability of the industry to step up to that when we really needed them very much.

This committee should be considering and advancing policies that continue to incentivize innovation unimpeded by regulatory barriers and excessive red tape.

And I agree with the witnesses who spoke earlier today that said that government price fixing of pharmaceuticals does not serve that goal.

I want to shift my attention though to another important and topical area of medicine, which I think can help tailor treatment decisions for patients today, and that is T-cell testing.

Dr. Abernethy, you wrote recently an article entitled "Winning the War on COVID Requires a Complete Understanding of Immunity. So Why Aren't We Demanding It?''

Well, let me start by saying kudos to you for writing that article. There are a few of us who are in the trenches trying to fight for T-cell testing.

I introduced a bill that provides for coverage of T-cell tests and T-cell immunity. I have discussed and written about this at length with Drs. Fauci and Collins and others over at NIH and I must say to a fairly poor reception.

Can you please elaborate for the rest of them here who

may not be as familiar with T-cell testing as you and I are what kind of information we are missing out on due to our public health authorities' narrow focus on antibodies rather than T-cells as well?

*Dr. Abernethy. Thank you for this interesting and important question, sir.

Practically speaking, as we look towards the science to help us combat the pandemic and address both SARS-CoV-2 as well as think about management of pandemics in the future and other health care concerns, it is important that we leverage the complete portfolio of solutions in front of us.

Our immune system has more than just one compartment. We have the ability to not only build antibodies, but also leverage cellular immunity, T-cells, to also attack and combat.

*Mr. Dunn. And I wonder if you would also compare, you know. We knew about T-cell testing when we studied SARS-CoV-1, right? Twenty years ago, and so this is not entirely novel science we are talking about here.

*Dr. Abernethy. An interesting point. Sometimes science takes pace at not always with exactly the same pace in all areas, and so one of the things that we have seen in the immunology space is sometimes the story on the antibodies side is amplified with not as much discussion going on on the T-cell side.

But I think that we have the opportunity to really look at how the entire immune system is performing and making sure that we really are amplifying how the whole immune system works, and at least just making sure that we are figuring out how our interventions are performing across the spectrum.

*Mr. Dunn. Thank you for that.

Dr. Hood or Dr. Abernethy, are either of you familiar with any large-scale T-cell testing, screening, if you will, for SARS-CoV-2?

*Dr. Hood. Well, I have participated at the Institute for Systems Biology and Swedish Hospital in some large-scale COVID tests. One of --

*Mr. Dunn. Were they T-cell?

*Dr. Hood. Well, one of the really powerful tools for being able to separate B-cell and T-cell specificity is to take it each blood draw as you follow these patients, 5,000 white blood cells, and sequence all the information in each of those.

*Mr. Dunn. That is a lot of sequencing, is it not, Doctor?

*Dr. Hood. So it defines the T-cells. It defines the different classes of T-cells, and we found new classes of T-cells.

*Mr. Dunn. Yes, yes, I am familiar. So you are going to get lost in the details of T-cells.

*Dr. Hood. So it is heartily clear that T-cells play a critical role in COVID immunity.

*Mr. Dunn. I think it does. Let me just say we agree with each other.

In the interest of the short time we have, I want to turn to Dr. Abernethy and say how do you think vaccine manufacturers could be, maybe should be using T-cells as they develop their vaccines and work on whether or not there is a need for boosters?

*Dr. Abernethy. Interesting question. In line with the science, we have the opportunity for vaccine manufacturers to really explore the full immune system response to vaccines.

We also have the opportunity to make sure that that information becomes available not only to regulators but to the entire clinical and scientific community, and that is going to be something that I hope to see more of in the future.

*Mr. Dunn. Yes. I would very much like to see this.

And in the few seconds that remain let me say that I have been recommending to people that if they wonder if they need a booster say, "You may need a booster, you may not. Get a T-cell test. Find out if you need a booster. If you have got immunity, why are you going and getting a booster? And if you do not have immunity, why are you not getting the darn vaccine?''

So those are my thoughts when I am advising patients. I should have prefaced that, I guess. Chairwoman Eshoo has told you that I am a doctor.

Thank you very much. I yield back, Madam Chair.

*Ms. Eshoo. I thank the good doctor. He yields back.

The chair is more than pleased to recognize the gentlewoman from Massachusetts, Mrs. Trahan, for her five minutes of questions.

*Mrs. Trahan. Thank you, Madam Chair.

And thank you, all the witnesses, for being here today.

A key thing in this discussion of biomedical research has been the importance of collaboration, and this comes in many forms, including data sharing. We know that data plays a critical role in advancing biomedical research and translating into new diagnostics and treatments.

And we have seen that for viruses like COVID-19, but also for chronic diseases, mental health issues, maternal health outcomes, and substance use disorder.

Dr. Butte, you have successfully worked to combine health care data from across six University of California medical schools and health systems.

And, Dr. Abernethy, likewise you have extensive data science experience in both public and private settings. This question is for both of you.

How can we improve communication between State and

Federal public health agencies and private health systems?

And what special considerations should be made to ensure communications between public and private health systems is efficient, effective, and secure?

*Dr. Butte. Great. Maybe I will go first.

As we all know, the United States has a competitive health care system. While we all want to enable and empower patients with their own data, especially using Federal standards, our pharma, biotech, and AR providers do not often want to share data with each other for competitive reasons.

However, the Federal Government has the ability to convene data, and that could be enhanced. So, for example, the National Public COVID Collaborative or N3C Program by NIH is one great example, with nearly 10 million COVID tested patients now and across many of the Nation's health systems.

With the right governance, NIH has shown that clinical data can actually be shared with each other to drive the best treatments and practices, for example, with patients with COVID.

*Mrs. Trahan. Great. Dr. Abernethy, can you please add?

*Dr. Abernethy. Thank you. I will expand on Dr. Butte.

But first of all, we can continue to leverage technology that enables the ability to share, whether that is pooled data coming to one particular place or federated data where

data sits in different places, but we share essentially insights.

The second thing I would say is that we do need to continue to incentivize sharing. Incentivize in hoarding gets us into trouble, but incentivizing learning how to use data together is one of the things that has become progressively more important, and we have seen that in the pandemic.

But it has to be safe sharing where we are focusing on patient privacy and security as we have talked about earlier today.

The last thing I would say is we have mentioned today the value of public-private partnerships, and you have also mentioned the value of, for example, State and Federal work together, and learning how to do this work well and learning from each other is going to be important as we try and work forward with patient data sharing.

*Mrs. Trahan. Great. Thank you.

Researchers, like many of you, are also pioneering in answers for AI in health care. Ensuring that minority groups are properly represented in data sets is one possible way to mitigate the risk of a predictive health care tool performing poorly for minority populations.

And so, Dr. Abernethy, can you explain the importance of training these AI models on diverse data sets and comment on

how feasible it is for manufacturers to diversify their data sets?

*Dr. Abernethy. Thank you very much for this important question.

As you critically point out, the building of algorithms is based, first, on the data that informed the development of those algorithms, and therefore, first of all, algorithms can become biased just by virtue of bias in the underlying data sets.

Therefore, it is incumbent on our future if we are going to leverage artificial intelligence for us to build data sets that do not systematically exclude specific populations.

We need to be leveraging those data sets that are as complete as possible and also documenting or essentially measuring the bias in data sets so that we can continue to improve.

The other thing that we need to do is document the performance of artificial intelligence solutions, including the output as well as how they perform in terms of their intended task and document that performance across time if we want to make sure that artificial intelligence continues to work for us.

*Mrs. Trahan. Great. Those are great points.

Well, let me just close by thanking the witnesses for their time today and for your contributions to biomedical

research, especially through this pandemic.

Thank you, Madam Chair. I yield back with time to spare.

*Ms. Eshoo. The gentlewoman yields back.

A pleasure to recognize the gentleman from Oklahoma, Mr. Mullin, for his five minutes of questions.

*Mr. Mullin. Thank you, Madam Chair.

Dr. Abernethy, am I saying that right?

*Dr. Abernethy. Yes, sir.

*Mr. Mullin. Okay. We all know that the precision medicines are typically incredibly expensive. Can you speak to why these drugs have such a high price tag?

*Dr. Abernethy. Thank you for your question, sir.

While I am not an expert in drug pricing or cost, I think that one of the critical issues as we think about personalized health care and precision medicine is that there is important adjustment in biology, in clinical development, in moving through the regulatory expectations, and importance of ultimately development interventions that are oftentimes for smaller and smaller populations, which may ultimately get reflected in the context of price.

But practically speaking, I hope towards a landscape where innovation across this space is going to increase the availability of precision medicine for all.

*Mr. Mullin. Thank you.

I agree with that, too.

In your testimony, you had highlighted that your company has payment structures that were based on patient outcomes.

Can you speak to the importance of this innovative payment structure that you guys have?

*Dr. Abernethy. I am not specifically sure exactly which payment structures to which you are referring. I think one of the things that I have thought a lot about and been considering in the future is the availability of data and the same data sets and processes that we use for clinical trials and clinical studies, the ability to interrelate patient treatment and outcome to help inform, for example, outcomes-based pricing in the future, where we can interrelate the performance of intervention to then the ability to think about how to pay for value across time.

*Mr. Mullin. Right. I believe in your written testimony you said that you have only been able to use these payment structures in limited circumstances. Am I correct on that?

*Dr. Abernethy. I think this was more generally from a policy perspective, the ability to use these kinds of interventions rather than specifically for our company.

*Mr. Mullin. Well, you know, I am part of the Innovation Caucus and co-chair that, and we are always looking for ways to incentivize the medical industry to look

at new directions, new ways. Instead of us just trying to fit everything into the same doughnut that we have always done business in, let's figure out a way to go around that.

And the payment structure is one of them. You know, in anything we do in life, be it get an attorney or call a service company, the payment is based upon the outcome. In most cases it is based upon did you complete the job or did you not. Did it work? Did the product you installed, did it work? The same thing with our mechanics that work on our vehicles.

And if we can look at a new payment model and tie it to the patient's outcome, that is something for us to look into, and as we are looking through innovation, I think we have got to look at the payment structure, too.

And so as we move forward, I hope we can have more of these conversations.

Madam Chair, with that I yield back and thank you for having this hearing.

*Ms. Eshoo. The chair thanks the gentleman for your interesting line of questions.

The chair is now pleased to recognize another one of our fine doctors, a member of our committee, Dr. Schrier of Washington State, for your five minutes of questions.

*Ms. Schrier. Thank you, Madam Chair.

And thank you to the witnesses who testified today. I

was thrilled to hear about your research and work, and I am very proud to see a Washingtonian here in the room today. Washington State is the hub for innovation, and, Dr. Hood, I am so glad that you are with us today.

Dr. Hood, I want to talk to you first and the work that you have done through phenyl health and the Beyond Human Genome Project. Briefly, can you tell me how this project, the Beyond Human Genome Project, is different from the Human Genome Project?

*Dr. Hood. Yes. The Beyond the Human Genome Project differs in that it employs for all the million people longitudinal phenome analyses, and the phenome, as I said before, is the reflection of the interaction of your genome, your lifestyle, and your entire environment.

So it gives us deep insights into what is happening at different points in your life to lead to actionable possibilities that we will be able to use on patients.

So it differs fundamentally with a whole new technology that is opening up probably thousands of new actionable possibilities we will be able to use to treat patients.

*Ms. Schrier. I love the idea of finding risk and disease early and being able to treat more appropriately. Research is paramount to finding treatments and cures, and that is why I was so proud to co-lead the Pediatricians Accelerate Childhood Therapies Act, the PACT Act, with Dr.

Joyce.

I think it is imperative that we invest specifically in pediatric research if we are going to address the greatest public health threats facing children in the 21st century like obesity and malnutrition and cancer, diabetes, asthma and, frankly, COVID-19. We have got to incentivize pediatric research.

In the meantime, you talked about prevention, identifying people at risk, finding the interaction of the genome and the phenome, and then addressing early detection, and we detected diseases as well as we can.

Can you briefly give me just like a sense of what sorts of diseases you can screen for in the Beyond Genome Project so that people have an understanding of what this means in their specific lives?

*Dr. Hood. Well, I think the first answer is we can really screen for approximately virtually any disease that we know about. As I said earlier, there are about 7,000 rare diseases, each for the most part caused by a single gene defect, and the complete genome sequence analysis at birth would instantaneously let us diagnose those diseases.

And, again, with single gene defects and the information we get with longitudinal phenome, we have the possibility of being able to generate early therapies and not let a person go through life with that gene defect.

There are many recessive mandilion diseases like cystic fibrosis and hemochromatosis and some of the Tay-Sachs-like diseases and so forth. And, again, these can be detected, and the individuals followed to be able to treat them at the earliest instance of moving toward a disease process.

We will be able to take the --

*Ms. Schrier. Sir, if I can --

*Dr. Hood. -- and stratify them into their different subtypes and go after therapies that can attack each of these subtypes.

So those are the kind of things we could do in a precision population approach to pediatrics.

*Ms. Schrier. That is great.

Dr. Hood, you know, I will tell you this as a pediatrician. I think about Type 1 diabetes. We now know how to identify those genes. Screening for them at birth would be incredibly important.

And we know that while we cannot cure it yet, that we can delay its onset, and every year that onset is delayed is a year less with the potential risk for complications.

Cystic fibrosis we now have gene therapies for. See, you could do that before any lung scarring develops.

And hemochromatosis is simply treated with blood draws, and if you could do that, you can protect the liver and other organs.

This is very exciting research. Thank you very much for leading the way, and I think that the last thing I would say is now we have to get doctors and teach them onboard because sometimes it is carrying through on those preventive lifestyle teams that can be the toughest part of all.

Thank you very much. I yield back.

*Dr. Hood. Thank you.

*Ms. Eshoo. The doctor yields back.

And now finally, the patient and deliberative gentleman from Utah, Mr. Curtis. You have five minutes for your questions.

And really, thank you for your patience. Mr. Curtis was the second person in the chamber this morning before we began. So thank you, and you are recognized.

*Mr. Curtis. Madam Chair, you are too kind. Thank you, and thank you to our ranking member. This is obviously a very important hearing.

I have to say I am very impressed with all of our witnesses today and the expertise that you bring.

Madam Chair, you have rightly so bragged about your district and Stanford, but I must take this occasion to brag about mine and to share some information about what is happening in Utah with life sciences. It is the fastest growing life sciences community in the United States in Utah, and the Beehive State is home to a vibrant health care

innovation ecosystem that is a key driver in both life changing interventions and tools.

Utah has referred to their State life sciences industry as "BioHive,'' a reference to the Beehive State.

This community consists of approximately 1,400 companies across the State working as a collective in order to have real impact.

There are a few interesting statistics I would love to share with everybody today. These individuals specialize in research, medical device manufacturing, biotech, and pharmaceuticals and diagnostics. They are second in the Nation for medical device employment concentration.

They provide $13 billion in [audio malfunction] GDP from industry. They account for eight percent of Utah's total GDP, and they filed for 538 bioscience related patents in 2019.

The BioHive includes partnerships with our top-notch universities, start-up accelerations, health systems, and State and local government, which collectively drive health care research and innovation in the State.

I believe it is particularly important to promote public-private partnerships and to prevent policies from being enacted that impede private sector investments. One of those 1,400 companies is Queen. It is a clinical stage biopharmaceutical company that was founded in 2013 working on

-- get this -- the reverse cellular energetic failure and to enhance repair of nerve cells.

Boy, if we could strike that, that would be an amazing thing.

But they are concerned. Sweeping government drug price controls, such as those proposed in H.R. 3, worry them and worry their investors. The Queen team is passionate about their work, but ultimately advanced potential solutions that will make a difference in patients' lives need investment, need predictability from the community.

And I would be remiss if I did not address that in our comments today.

Dr. Hood, I have been fascinated as you talked about your research, and I am curious. This committee and myself personally have a deep tie with ALS and the suffering that we see from them, MS, Parkinson's, some of these diseases that attack the nervous system.

Are you seeing anything in your research that are flags for these diseases that would help us help these good people?

*Dr. Hood. Well, one of my major areas of research now is Alzheimer's disease, and I will make a couple of general statements.

One is that there have up until very recently not been more than 500 clinical trials on drugs for Alzheimer's. All have failed, and it is because they have entirely the wrong

hypothesis about what it is as --

*Mr. Curtis. And I am going to push you just because we have all got tight time constraints.

*Dr. Hood. I am just saying what we are learning about Alzheimer's is almost certainly going to apply to the other neurological diseases.

*Mr. Curtis. We are all cheering for you.

The frustration has come before this committee and those who have addressed this committee, is the inability of these patients to access treatments. Can you give us any advice on how to speed that up?

*Dr. Hood. I think the key to access to the patient really lies with better treatment and a focus on wellness and prevention rather than always attempting to attack --

*Mr. Curtis. I just wish we had so much more time, but I am just going to keep moving on and just press the fact that some of these cures that are potential but undefined, they do not have access to them, despite the fact that they have been given a life sentence.

So I would just like to lay down a marker in this hearing as well for these good people.

Dr. Minor, let me switch to you quickly. You have also been part of an ecosystem that is very healthy as I think I have described in Utah. Tell us what we have to worry about with government destroying these ecosystems.

We have talked a little bit about what government can do to help. What are your worries about what government might do that would be detrimental to these ecosystems?

*Dr. Minor. Well, thank you for your question.

I think that harmonizing and developing greater clarity on regulatory requirements coming from the Federal Government between the various agencies is one proactive step that government can take.

Certainly all of us as Americans should be grateful for the FDA and the CDC for the critically important role that they play, long before COVID, but during COVID as well. But we have learned from COVID ways that perhaps the FDA and CDC could look at better coordination, particularly when things are moving quickly.

So I think there are a number of areas, Congressman, that could be explored.

*Mr. Curtis. Doctor, I am over my time, and the chair has been very patient with me. I would like to just end by pointing out that we have to be as careful not to hurt the industry as we are in our efforts to try to help the industry.

Thank you, Madam Chair. I yield my time.

*Ms. Eshoo. Did you get the answer to your question? We should give time for the answer.

Who did you direct it to?

*Mr. Curtis. So to Dr. Minor, but I would be curious if any of our other witnesses want to weigh in on that.

*Ms. Eshoo. Dr. Minor, would you like to weigh in on that?

*Dr. Minor. Well, I think there are opportunities for the government to look at impediments that may exist and get feedback from industry.

And, yes, I do think that there are concerns with regard to the speed at which things can be developed and commercialized, and regulatory issues may play a role in that.

*Mr. Curtis. Thank you.

Thank you, Madam Chair.

*Ms. Eshoo. Certainly. The gentlewoman from New Hampshire, Ms. Kuster, is recognized for your five minutes of questions.

*Ms. Kuster. Great. Thank you so much, Madam Chair.

And, Mr. Curtis, I will pick up where you left off.

I want to thank the witnesses for being with us to discuss advances in biomedical research and how Congress, the research community, and industry can work together to advance innovative technologies and treatments.

Well, basic research plays critical roles in achieving these goals. We also must ensure the knowledge gained from the basic research can translate into the development of new

diagnostic and therapeutic tools that can be used in clinical practice.

And it is this translational research that gives many of us hope one day it will provide the foundation for new diagnostic devices and promising therapies for patients with diseases like Alzheimer's or cancer.

Dr. Minor, as a scientist, surgeon, and academic leader, you are a strong proponent of translational research. As such, you are well aware of the significant challenges associated with crossing the Valley of Death, the phase between research and innovation that can be so difficult.

As technology advances and leads to breakthroughs in fields like defense and energy, breakthroughs in disease treatment and cures seem to lag behind.

So, Dr. Minor, this committee has supported efforts to address the Valley of Death by supporting the advancement of novel clinical trial designs and streamlining the regulatory process when we passed the 21st Century CURES Act.

Have these efforts been effective?

And could you explain why or why not?

*Dr. Minor. Thank you very much for your question, Congresswoman.

I do think that the efforts have been effective, but there is so much more that we can do and that we should be doing.

Yes, I think the best evidence of the efficacy is what was accomplished with Operation Warp Speed and the development and deployment of messenger RNA vaccines, a completely new class of vaccines, in about 11 months from the sequencing of the genome to the emergency use authorization.

That is an example of what can be accomplished. A lot more needs to be done. ARPA-H, I think, is a great step in that direction of accelerating translation initiatives such as the innovative medicine's accelerator at Stanford that I described in my testimony earlier. I think it is also an important step there.

Today is the difference between how we train basic scientists and the way basic science is done and how we do translation, and so assisting basic scientists in the process of getting their discoveries in a translational pipeline is a big responsibility for those of us in academia, and I think it is something that the government through programs such as ARPA-H, Operation Warp Speed, and the things that were done during that period can be very beneficial.

*Ms. Kuster. Well, I would certainly agree with you, and it was quite extraordinary. I wish we could be as successful in convincing the American people to take the vaccine.

What policies have been useful to you?

And could you recommend any changes in the statutes or

changes in directions that this committee could take to increase advances in contemporary science and increase diagnostics and therapeutics?

*Dr. Minor. Thank you.

Encouraging innovation is critically important. NIH does a fantastic job of funding research. Most of NIH funding is directed at projects that are already up and going.

Preliminary data plays a major role in NIH applications for understandable reasons, but oftentimes the most innovative discoveries and advances come from a spark of an idea. Many of those ideas may fail to yield results. Yet we still need to give those ideas an opportunity to see the light of day or not.

And right now we do not have a grouping of governmentally sponsored programs that fund the most innovative research. Again, I think the notion behind ARPA-H will advance that goal, and what we do at universities is important.

But we have heard the word "innovation'' and several members of Congress today on this committee have talked about its importance, and I think there are ways that we can pursue legislation that encourages innovation and entrepreneurship in our biomedical communities.

*Ms. Kuster. Well, I thank you all for being with us,

and I thank the chairwoman for her leadership in this area.

And with that I am going to yield back and hopefully you will save the ten seconds that Mr. Curtis went over.

[Laughter.]

*Ms. Eshoo. Thank you very much, Congresswoman Kuster.

It is a pleasure to recognize the gentleman from Georgia, the pharmacist on our committee, Mr. Carter, your five minutes for questions.

*Mr. Carter. Thank you, Madam Chair.

And thank all of you for being here. This is extremely important. This is the wave of the future.

I started practicing pharmacy back in 1980, and what I have seen in health care and the progress that we have made has been nothing short of miraculous. I can only imagine what we are going to see in the next few years, to be quite honest with you, with this type of discoveries that will result from this innovation, and that is exactly what it is.

Dr. Hood, I want to ask you. Biomarker testing, what does it mean in the context of precision medicine?

I heard you say earlier today, and I found it fascinating, being a pharmacist, and I am thinking of it in terms of from the way that the insurance companies now, you know, you have a formulary, and you have to take that medication on that formulary.

But what you are talking about, you would be able to

identify specific drugs that work for specific patients, and I am trying to get my arms around how the insurance companies are going to adjust to that and how we in CMS and Medicare and Medicaid, how we are going to adjust to all of that because it is no longer going to be a competitive bidding system to get your drug on the formulary if it is, indeed, as you say, patient-specific in precision medicine like this.

*Dr. Hood. Well, my feeling is it will not be quite patient specific, but there will be different categories of patients and smaller categories than they are used to.

So blood biomarkers are the most common. The things they can do are, one, I can identify biomarkers that for statins can tell me who can take that successfully and who can avoid the complications of statins, diabetes, muscle pain, and all of the other things.

Another use is to be able to see the wellness to disease transition at its earliest stage, and that marker lets us think about therapy at that point in time, at a reversal of disease before it ever manifests.

So blood biomarkers can look at any state change or they can look at populations that respond to external stimuli like drugs.

*Mr. Carter. You know, I believe you are going to run into some barriers here because we run into it now. You know, let's stick with statins. You know, there are certain

statins that work better for patients than others because of the side effect profile or because of the patient's biology, if you will, whatever.

But, you know, again, I think this is going to be a barrier with coverage. I mean, let's face it. Some patients, they have to take whatever the insurance company is going to pay for. They cannot afford to pay out of pocket.

What other barriers are --

*Dr. Hood. Well, if, in fact, the drugs that the insurance companies pay for work every time, then that is going to be an enormous improvement in the long run for better response.

*Mr. Carter. Absolutely.

*Dr. Hood. Okay? And, again, on the ten most common drugs, about ten percent of the people responded effectively. Ninety did not. So if we can clear those 90 away, we are going to save hundreds of billions of dollars in cost for drugs.

*Mr. Carter. That is good news.

*Dr. Hood. And that is not saying we have to reprice or anything. That is just making sure if we match the right drug to the right patient.

*Mr. Carter. Thank you, Dr. Hood.

Dr. Abernethy, I want to get to you really quick and I only have a minute left.

We all know what is happening in China, and we all know that the United States has been on the forefront of genomic testing, and it remains the world leader. However, China recently has begun a significant push into genomics and through state-backed entities.

And how these companies and the Chinese Communist Party use the data that will become available through genomics research is a major concern with the Intelligence Committee. I have been in hearings here at the Capitol where they have expressed concerns to us about what they are going to do with that.

How concerned are you about the threat of China and what we can do to counter China's advances and potential data theft?

*Dr. Abernethy. Thank you for that very interesting question.

Practically speaking, I think that we have the opportunity and responsibility to do the critical work, especially in genomics and other areas here in the United States.

Practically speaking, the more of that that gets done outside of our country takes away from our ability to innovate here and also increases risk of, for example, specific information about our population to be in other places where the people can do things that --

*Mr. Carter. But do you think it can be used adversely?

*Dr. Abernethy. I do not have specific knowledge about how it can or cannot be used adversely, but I certainly can imagine ways that genomic information can be used by bad actors when they want to.

*Mr. Carter. Dr. Hood, do you think it could be used adversely?

*Ms. Eshoo. Doctor, you need to turn your microphone on please.

*Mr. Carter. Microphone, microphone.

*Dr. Hood. The major threat to us in longitudinal phenome analysis is China. They are the only one, apart from us, that is doing this in a major way, and I think the key is going to be to fund this in an aggressive way so we will remain a world leader in this particular area.

*Mr. Carter. If they stay with it, and they will stay with it. They are no friend of ours.

*Ms. Eshoo. You have --

*Mr. Carter. And I know I have gone over, and I appreciate your indulgence, Madam Chair.

Thank you all, both.

*Ms. Eshoo. Certainly. The gentleman yields back.

The chair now recognizes the gentleman from California, Mr. Cardenas, for his five minutes of questions, followed by Mr. Crenshaw, followed by Ms. Kelly, and then finally

followed by Dr. Joyce.

*Mr. Cardenas. Thank you, Madam Chairwoman Eshoo and also Ranking Member Guthrie, for holding this important hearing.

And I want to thank all of you who are witnesses today and experts helping to educate us policy makers in the Energy and Commerce Committee here in Congress.

Biomedical research is the foundation for the development of future medical treatments and cures, and this hearing will help inform us, the legislators, on how to improve on these processes.

Today I would like to focus on the importance of diversity and inclusion in biomedical research. We know that there are many groups that are underrepresented in clinical trials and biomedical research, including racial and ethnic minority groups, sexual and gender minority, people living with disabilities, people who have low income or low educational attainment, and rural residents.

Mr. Falcon, I want to also thank you for your decades of work in this area. Based on your testimony, you have extensive experience in community engagement efforts and providing traditionally underrepresented groups the opportunity to be heard on issues related to their health. So thank you for that.

In your testimony, you state that the biomedical

research enterprise has not met the standards for diversity and inclusion set forth by the NIH, the Revitalization Act of 1993.

It has been offered that one reason for this is that much of the Food and Drug Administration and the National Institutes of Health guidance on inclusion is nonbinding. I will repeat. It is nonbinding.

That means that it is recommended and not required. And as you mentioned, this unfortunately does not seem to be enough. I want to provide a very recent example of this.

Aduhelm, which costs about $56,000 a year, was approved just a few months ago by the FDA for the treatment of Alzheimer's disease, despite older Black adults being estimated to have Alzheimer's at double the rate of White adults. Only .6 percent of the study participants were Black.

Additionally, only three percent were Hispanic, and .03 percent -- that is one person in the study -- was Native American. Clearly, the nonbinding approach is not working.

From your vantage point, Mr. Falcon, what further actions could Congress take to improve representation in clinical trials?

And what are the consequences of inadequate inclusion in biomedical research?

*Mr. Falcon. Well, thank you, Representative Cardenas,

for the question and for your leadership on this issue.

Very clearly recommendations have not worked. We have had decades of recommendations. It is time to make the recommendations binding, and that can be done in some very clear and, frankly, very straightforward ways.

At NIH, there is a review process. That review process now should include a score tied to funding on whether or not the study meets standards of community-based participatory research, which NIH itself has said is the gold standard of research.

That score should include whether or not there is adequate inclusion of underrepresented groups, whether or not the study is designed to power to report out findings specific for those underrepresented groups, and whether or not the study design includes all the principles of community-based participatory research, and finally, all study research should be reported to report out data by race, ethnicity, sex and gender, as recently required now by the New England Journal of Medicine.

Those changes would dramatically change the landscape of research being approved and research being reported out of NIH.

With regard to the FDA, in a very straightforward way, again, the FDA review standards of clinical trial proposals should include a review of inclusion and should set metrics

for clinical trials as they progress for meeting those standards of inclusion.

And if those standards are not being met, there should be enhancement required during the clinical trial process so that we do not get to the end of a clinical trial without adequate inclusion.

The effect on health care has been dramatic of there being a lack of diversity. Right now one in five cancer clinical trials fail because of lack of enrollment. It is very expensive to get to the point of starting a clinical trial, and if we are failing simply because we cannot achieve enrollment, we are failing in terms of innovation, and we are failing in terms of delivering on the promise of health care for all.

*Mr. Cardenas. Thank you, and that is why I am working with my UC colleagues that are Representatives Kelly, Butterfield, and Clark on a bill to improve inclusion in clinical trials, and I am so grateful for the impact that you have been able to give us today.

With that, I apologize for going over my time, Madam Chairwoman. I yield back.

*Ms. Eshoo. I would just add that is the beauty of a hearing in the Congress of the United States.

It is a pleasure to recognize the gentleman from Texas, Mr. Crenshaw for your five minutes of questions.

*Mr. Crenshaw. Thank you, Madam Chair. Thank you and the ranking member for holding this hearing.

I know that this is of particular importance to the chair of the subcommittee because she represents many of the firms that make these drugs and is also very passionate about finding new cures.

In Houston, we also have a very strong biomedical innovation sector, and we are very proud of that. I am going to talk about one of my constituents. This committee paved the way for her innovation with the CURES Act working on adult stem cells.

So Donna Chang works on this future of medicine, curing a person's disease with their own stem cells, and she is doing clinical trials with stem cells on Parkinson's, long COVID, and other neurodegenerative diseases.

And these are not unsafe treatments. They are not fringe doctors. They are FDA approved trials and procedures that show promise, but they do get stuck in the regulatory framework set up for stem cell therapies.

So, Dr. Abernethy, if you would indulge me for these questions, stem cell therapies are supposed to be regulated under the RMAT pathway established in CURES. For these more advanced treatments, specifically what I am talking about, they sometimes do not meet those strict requirements set by the RMAT pathway and, therefore, are regulated as a drug

through that pathway.

Stem cells are part of a person's body. Could they be regulated in the same way as we regulate other autonomous bodily tissue?

*Dr. Abernethy. Thank you very much, Mr. Crenshaw. An interesting question, and certainly we are all looking for better ways to take care of patients and to personalize.

I do not really have an opinion as to whether or not they can be regulated outside of their current pathway, and practically speaking, I do think regulatory innovation is going to be important across this space.

*Mr. Crenshaw. Do you think the RMAT pathway could be amended to broaden the number of stem cell therapies that can be safely approved?

*Dr. Abernethy. I honestly have not explored this specific question. So I do not know the answer to that question.

*Mr. Crenshaw. Yes or no?

Do you think we need to act on that as a Congress to design new drug pathways for stem cell therapies?

*Dr. Abernethy. It seems like this is an important question to this committee. So this is an important time to look in detail.

*Mr. Crenshaw. Oh, boy, here we go. Mesenchymal stem cells are more and more commonly extracted from adipose

tissue. Okay? So we are talking about just taking stem cells from fat. All right? Let's have a normal conversation.

Unfortunately, the FDA has ruled that the stem cells taken from fat tissue just do not pass the muster of the definition of minimally manipulated, and in my conversations with the FDA, I learned that they are hesitant on adipose tissue stem cells.

It does not come from a safety concern but a lack of knowledge on this tissue. They do not know how the process of removing the adipose tissue changes the function of the stem cells.

And maybe you still cannot answer it, but maybe you can shed some light since you spent some time at the FDA. I mean, short of writing a bill that tells the FDA how to interpret what minimally manipulated means, what can Congress do to help close their knowledge gap on stem cells derived from adipose tissue?

*Dr. Abernethy. So again this is not an area where I can specifically comment with discrete knowledge. I think your question about what can Congress do to help FDA in these areas is an important one.

Critically, there are often areas where there is evolving science and FDA needs the opportunity to have the personnel, so essentially the scientists at FDA have the

scientific dialogue, for example, together with the National Academy of Medicine and others and also the opportunity to update a regulatory pathway that is needed.

And so as Congress there is the opportunity to make sure that the FDA has the right resourcing and also the right sense of urgency to solve these problems.

*Mr. Crenshaw. And based on your answers, it seems like we do need to tell the FDA what to do. I think maybe that is the point I want make here.

And I think there could be some great bipartisan work to get really cutting edge, effective treatment to people that right now are just tied up in a web of paralysis by analysis at the FDA. And ironically some of these treatments are potential treatments for paralysis. We have actually seen some real interesting case studies from this particular biomedical research firm that I was talking about.

So I hope that is something this committee can work on.

And I yield back.

*Ms. Eshoo. The gentleman yields back.

I would suggest to the gentleman perhaps a briefing, you know, to meet with the FDA. I would be happy to work with you on that if you so choose to do it.

The gentlewoman from Illinois, Ms. Kelly, a wonderful member of this committee, the subcommittee, the full committee. You are recognized for five minutes.

*Ms. Kelly. Thank you, Madam Chair and Ranking Member Guthrie, for holding this hearing on the future of biomedicine.

COVID-19, as we all know has had --

*Ms. Eshoo. Is your microphone on?

*Ms. Kelly. Yes.

*Ms. Eshoo. Okay.

*Ms. Kelly. COVID-19 has had a devastating impact on our country's physical and mental health. According to a U.S. Census Bureau survey, Black and Latinas individuals were disproportionately affected by mental health issues during COVID-19.

However, according to the National Institute of Health, clinical trials for depression treatments funded in 2018 had a median participation of 67 percent white participants but only 11 percent Black and seven percent Latinx participants.

These numbers are not reflective of racial and ethnic diversity in the United States and future clinical trials need to reflect the disproportionate impact these conditions have on communities of color.

Unfortunately, this example is not an outlier and similar disparities can be found in many clinical trials, from anxiety and prostate cancer to heart disease. The lack of progress highlights the need for increased racial and ethnic diversity in clinical trials.

Mr. Falcon, as you mentioned in your testimony, the NIH Revitalization Act established many of NIH's current guidelines around inclusion of women and members of minority groups.

Are there any gaps in current NIH policies to increase clinical trial diversity specifically around accountability for clinical trial sponsors?

*Mr. Falcon. Yes. Again, the fact that the guidance is not mandatory is the most significant action that does need to be taken by NIH, and unfortunately the government is following rather than leading as I have mentioned before.

Just last month, the New England Journal of Medicine is requiring all of its publications to include specific data for underrepresented populations. The government should also be doing that as well, and NIH should mandate that.

The FDA should follow a similar path, and with regard to the FDA, I would recommend that it is time for this committee to receive an update on the 907 plan to increase diversity in clinical trials.

*Ms. Kelly. Thank you.

Would there be any benefit to empowering NIH with greater authority to work with clinical trial sponsors to establish clear and measurable goals for diverse recruitment and retention in the funding applications.

*Mr. Falcon. Yes, absolutely, and in fact, the Diverse

Trials Act, one of the three components, actually asks the Secretary to set standards around decentralized trials that could be implemented.

I think if you talked to some of the major trial sponsors, what they are most looking for is clarity on how to deal with the issue of inclusion, and I do think there will be recent activity, in fact, to a greater partnership with NIH in terms of this issue.

*Ms. Kelly. Thank you.

We need to make sure we increase accountability to ensure that clinical trials represent the racial and ethnic communities impacted by the disease or condition being studied. That is why I am working on a clinical trial diversity bill focused on the NIH with my E&C colleagues, Representatives Cardenas, Butterfield, and Clark.

We look forward to working with our colleagues on both sides of the aisle to advance this important bill.

Dr. Abernethy, can you provide any examples of how developing drugs without racially and ethnically diverse trials can lead to drugs that might not be effective or even cause adverse effects for certain populations?

*Dr. Abernethy. Thank you for this important question.

Practically speaking, if we think about developing drugs and we leave parts of our population, segments of patients out of the story, it can have an adverse effect because we do

not understand, for example, whether or not there might be specific consequences, whether those are as it relates to personal health, like renal function, symptom experience or also other issues, such as the issues around managing comorbidities and co-administration of medications.

If we are going to make progress in this space, we have three critical things that we need to do. First is we are going to need to update our approach to design the clinical trials themselves. So, for example, make sure that generalizability, the eligibility criteria in clinical trials do not exclude patients unnecessarily.

We see, for example, that some clinical trials exclude people with HIV when there is no real reason that people with HIV should be excluded in a particular population within that clinical trial context.

The second is we need to make sure clinical trials meet patients where they are whenever possible. So, for example, decentralized clinical trial initiatives are very important.

And the third is that when specific parts of our population cannot be involved in clinical trials for some reason, for example, people with advanced hepatic failure, we need to make sure that we leverage real world data sources to fill in that knowledge gap so we understand the performance for all patients, whether that is due to medical comorbidities, due to racial and ethnic backgrounds, or due

to inability to participate for some other personal reason.

*Ms. Kelly. Thank you so much.

And I yield back.

*Ms. Eshoo. The gentlewoman yields back.

It is a pleasure to recognize the gentleman from Pennsylvania, yet another one of the doctors on our subcommittee that we benefit so much from, Dr. Joyce. You are recognized for five minutes.

*Mr. Joyce. Thank you, Chair Eshoo and Ranking Member Guthrie, for convening this critically important hearing.

Biomedical research and the advancement of cutting-edge therapies and their cures, they save lives. It is critical in our role as policy makers that we acknowledge this and work together to facilitate innovation.

That is why it is so concerning that we are still discussing the Build Back Better Act, which would cripple this innovation, especially with regard to rare diseases.

We also must realize that we cannot limit private research and only fund the NIH and expect the same advancements and new cures. Almost 90 percent of new drugs originate in their entirety from industry, and pursuing policies that discourage this type of lifesaving investment is counterproductive and will ultimately harm the patients.

As a physician and as a legislator, simply this is unacceptable.

With that said, I would like to thank the witnesses for testifying regarding advancements in biomedical research, and I truly appreciate your expertise in these efforts

Dr. Abernethy, like you, I am trained in internal medicine, and I am also trained in dermatology and have spent more than 25 years treating patients with melanoma, similar to you.

I have seen what advancements in therapies have done for patients with metastatic melanoma. That has ultimately led to cures and lives being maintained.

Dr. Abernethy, as we move toward more individualized medicine, such as cell-based therapies, do you believe that regulators are prepared to review and to advance these types of products to approval?

*Dr. Abernethy. Thank you very much for your question, sir.

And you are absolutely right. In the space of melanoma, we have watched how the landscape has changed, and with patients sitting in front of us who are 20 years old, who are dying, now have treatments that improve their lives.

Practically speaking, I do believe that the regulatory framework is ready to continue to allow for the continued approval of therapies as they become available. However, I think that the FDA is going to need several things.

The FDA is going to continue to need to make sure that

there are enough scientists and experts at FDA to review those applications as the science gets progressively more complex.

I think the FDA is going to need to be ready to scale from a regulatory perspective. There are a thousand cell and gene therapies in front of the FDA right now, and we are going to need, for example, data and technology tools to be able to do that work faster.

And we are also going to need to make sure that the FDA has the tools in place to allow the evaluation of therapies across time because as these new innovations come forward, we are going to need to study them not for one or two years, but five, ten, 15 years, which is going to require new ways of thinking.

*Mr. Joyce. Dr. Abernethy, I agree. These new innovations, they save lives, and given that it may not make sense to adopt the same regulatory requirements for patients with specific therapies as we have for more traditional treatments, what actions should we here in Congress and regulators take to ensure that the government keeps pace with science so that patient access is no longer delayed?

*Dr. Abernethy. So I sincerely believe that we need to make sure that we are developing the solutions that allow us to evaluate individual therapies across time and hone our understanding around which patients work or for which

patients any particular intervention works.

That means that we do need the continued tools that allow for appropriate earlier approval when it is appropriate for both our understanding of safety and effectiveness of a potential therapy and at least adequate understanding of that, but also continue to evaluate that across time.

This way we are able to leverage the opportunity of earlier approval with the balanced expectation of understanding when we should pull back that approval or adjust it if we find out an intervention is not working as expected.

*Mr. Joyce. Thank you, Dr. Abernethy.

Dr. Schrier, who is here present today, and I have worked and introduced bipartisan legislation to deal with the issue of creating pathways for pediatric research.

With the time that I have remaining, can you please speak to why it is important to make the distinction between adults and pediatric research and the importance of this research towards developing future cures to childhood cancers?

*Dr. Abernethy. Thank you.

Three critical things. One, children are not small adults. We have to understand how interventions work within the context of real people, including our children.

So that is the first reason this is critical, is that we

have to make sure that we are actually doing the work with the people for whom it matters, not just translating what we think from adults into children.

The second is that the afflictions of children are different than adults, and so we have to make sure that we have done the science to address the problems that are affecting our children, which is often different diseases.

And the third thing is that we have to make sure we incentivize essentially research and regulatory work that needs to happen for populations that are oftentimes not the focus of investment for essentially the communities that capitalize the clinical development of the future.

*Mr. Joyce. Madam Chair, my time has expired. I thank you for allowing me to extend.

And, Dr. Abernethy, thank you for being here for your important insights.

And I yield.

*Ms. Eshoo. The good doctor yields back.

We do not have any more members that wish to question. I want members to know that they have ten business days to submit additional questions for the record.

And, witnesses, we ask you to respond as promptly as you can to the questions, the written questions that are submitted to you.

Also, I would like to request unanimous consent to enter

the documents that I shared with the minority into the record.

*Mr. Guthrie. No objection.

*Ms. Eshoo. No objection. So moved.

[The information follows:]

**********COMMITTEE INSERT**********

*Ms. Eshoo. Let me thank the witnesses, Dr. Abernethy, Dr. Butte, Mr. Falcon, Dr. Hood, and to my constituent, Dr. Minor. You have been with us since 10:30 this morning. So that is what, almost three and a half hours?

But these three and a half hours have been highly instructive because you have been excellent witnesses. We certainly are going to put our heads together to identify the key areas that you have brought forward. They all deserve legislation that addresses how best to advance for the betterment of the American people.

And when we advance in terms of all of this, it is a gift to the world because America leads.

So thank you so much for the time and effort that you have put into this hearing. I thank all of the members.

And at this time the subcommittee is adjourned.

[Whereupon, at 1:54 p.m., the subcommittee was adjourned.]