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Research!America Holds National Health Research Forum on the Future of Medical And Health Research

LIST OF SPEAKERS

WOOLLEY:
Good morning. Good morning, everybody. And it's still morning, I think, technically. And welcome to Research America's National Health Research forum. I'm Mary Woolley, president of Research America. And on behalf of our board, and several of our board members are here, if you would, either stand, wave, you know, we want to acknowledge you.

Thank you for your long service.

(APPLAUSE)

Thank you. And on behalf of the board, then, and on behalf of my talented colleagues on staff, those of you should also wave, take a bow. Thank you, for all you do.

(APPLAUSE)

Welcome again. Our annual Straight Talk program is intended to really get your juices flowing. We want them to hear us down there in the Capitol, OK? We want to have a conversation really flowing with ideas, comments and questions about the future of medical and health research.

We'll be probing on many fronts. For example, how are we going to answer the questions that patients are asking right now, that the public broadly is asking about discovery, delivery, development of better healthcare, better preventions? Whether they're asking directly or via the media or via policymakers, we need the answers. And how can medical research itself truly progress at a time like this when we have a constrained environment in terms of both resources and policies, many of which haven't changed for decades.

We know that our distinguished panelists will shed light on these questions and more, and we look to all of you to weigh in, as well. Decisions made just a few blocks from here this fall are going to be consequential. In its few legislative days this month, Congress has to deal with the Iran nuclear deal, respond to the refugee crisis, talk about budgets and appropriations for the new fiscal year and much, much more and meanwhile, take some time out for the Pope's visit next week. I do hope Pope Francis is coming prepared because it may take a miracle or two to get to a budget for the next fiscal year.

And speaking of things coming right up, will the Senate take action anytime soon on its version of the 21st century cure bill that's so many people in this room have worked so hard to get across the finish line first in the House, and now we're working in the Senate? Well, there is lots of good news in that there are champions, long-standing champions and new ones on the Hill, who are doing their best to advance policies to drive private-sector innovation and to ensure that our federal health agencies get the resources they need to further their mission.

Our panelists will talk about this and more, and we encourage you to join the conversation during the Q&A, and on social media using the hashtag #raforum15.

Already, though, less visibly than the presidential candidates, members of Congress are running for re-election. Today, Research America is excited to announce the launch of our national voter education initiative for 2016 Campaign for the Cures, vote for medical progress -- this button right here.

Every election cycle, we work closely with our partners to engage candidates so that we can elevate public and private sector research in the national conversation. So as candidates engage with voters at rallies, at town hall meetings and through social media, we will be urging them and our partners will be joining us in urging them to tell us what they would do, if elected, to maintain this nation's preeminence in science and technology and put research to work to improve the health of our loved ones.

I hope you will join our campaign. Our staff is prepared to tell you all about it or you can learn more on campaignforcures.org.

Now, I'd like to take a moment, though, to thank our national forum sponsors. Our lead sponsor, Astellas Pharmaceuticals; our panel sponsors, Shire; AdvaMed, and BD; our marketing sponsor, Celgene; our topic sponsors, Elsevier and Gilead; and our innovation sponsor, Janssen Pharmaceuticals Company of Johnson & Johnson. Thank you, sincerely. Our ability to deliver a quality program does depend on your support. We have other supporters today and they're shown in your program. I encourage you to say thanks to both sponsors and supporters.

We're very pleased to announce that WebMD is again this year livestreaming today's program to viewers across the country, and indeed, all over the world. We know there's a contingent in Japan tuning in for sure. So thank you, WebMD and thanks to The Hill and the Washington Examiner, our media sponsors this year.

Now, I'm very pleased to introduce Dr. Jeffrey Bloss, senior vice president of scientific and medical affairs at Astellas U.S., our lead sponsor, and will deliver introductory remarks. And please don't hesitate to start eating. Thank you.

(APPLAUSE)

BLOSS:
Well, thank you, Mary, for that kind introduction. And it's a thrill to be joining you all today. You know, I've had the pleasure of working with Research America, America for the past several years, and I can honestly say very few match her vigor and enthusiasm for advancing medical research.

And Mary asked me to give a few remarks today, which I normally, as folks who know me, do quite off the cuff, but I realized that I've never spoken for less than about an hour and 10 minutes. More importantly, our staff realized that as well. So they've prepared written remarks for me today so I'm actually off the stage somewhere in less than 50 -- no, 10 minutes.

So Astellas is indeed proud to be the lead sponsor of this year's forum, which comes at a particularly intriguing and promising time for all of us engaged in healthcare research. For those of you unfamiliar with Astellas, we are a global company with about 18,000 employees across 55 countries, 3,000 of which are here in the United States. This is actually our 10th anniversary year. Astellas was formed about 10 years ago as a result of the merger between the third and fifth largest pharmaceutical companies in Japan.

And while we're involved in a multitude of therapeutic areas, we remain industry leaders in both urology and transplantation, but we have clearly expanded our portfolio and are moving very rapidly into the area of oncology. However, regardless of the therapeutic area in which we're involved, we're focused intently on one key priority, and that is turning novel science into value treatments for our patients.

As Mary eloquently captured, we are all in the midst of a new era in medical science, really in healthcare overall. Is there a segment of our system that isn't undergoing significant change from surging understanding of the molecular underpinnings of disease to the emergence of personalized medicine, from evolving Affordable Care Act exchange marketplace to the empowered patient, from hospital consolidation to an enhanced focus on value in healthcare delivery and formulary decisions?

The changes are clearly fast and furious, catching up -- catching up or even staying ahead of the curve is clearly our challenge. It was only a year ago that I participated in a thought assembly that followed this very event one year previously. During that session, we noted with regard to two diseases, both cancer and AIDS, it essentially required a U.S. president to declare war on those diseases to mobilize the government in our country, to step up to fight for our patients through well-funded research.

Based on that observation, we concluded that day that if we want America to remain the epicenter of molecular innovation -- medical innovation, we need to learn from these lessons and engage in passionate advocacy from across the ecosystem to create a groundswell around medical research.

Arguably, that groundswell may have already begun in the firm -- in the form of the 21st Cures Act. Over the past year, this massive effort dominated much of the discussions around the discovery development and delivery of healthcare.

While not everyone agrees on the details, I am sure that no one would argue that as notable as the legislation itself, it is the collective spirit forged by Chairman Upton and Congresswoman DeGette that has really been remarkable.

I bet there isn't a person in this room who hasn't participated in 21st Century Cures in some way. This galvanizing process has been a powerful contagious force coupled with the parallel efforts being shepherded in the Senate by the HELP Committee Chairman Alexander. It is admirable, impactful and a collaborative rallying cry that I hope will extend well beyond this singular initiative.

At Astellas, we're also strong believers in partnership, collaboration, and the collaborative spirit is woven into everything we do, including one area of increasing importance to us, which Mary has asked me to talk about today briefly. And that is the collection, analysis and dissemination of the real-world evidence.

I'd like to share a few thoughts about real-world evidence before we embark on today's discussions. You don't have to be a student of pharmaceutical science to know that randomized controlled clinical trials have long been the primary source of data and clearly the gold standard for establishing the safety and efficacy of investigational drugs.

Randomized controlled clinical trials are designed to target specific patient populations and to address the important questions of risk and benefit in a very controlled clinical setting. However, once a medicine is approved by the FDA and prescribed by physicians, many more patients receive the drug and almost always outside that controlled clinical environment. These real-world patients offer hugely valuable safety and efficacy information that goes well beyond the information generated in a controlled clinical trial.

Let me give you a hypothetical example. Say you have two medicines in the class, both with proven safety and efficacy in a particular disease. But one is more tolerable to patients than the other leading to better patient compliance.

In a randomized controlled clinical trial, it would be difficult to differentiate these two agents as patient adherent is largely assured through the controlled nature of the trial. However, in a real-world evidence study design, the more tolerable agent may, in fact, prove superior to the less tolerable agent-based only on patient adherence.

Real-world evidence allows us to examine broader information on the use of these medicines. We can learn new details about how a patient responds that can be incredibly meaningful over time. Such information has certainly been used by the FDA over many years to inform safety-related decisions. But is typically not been used to evaluate the benefits of a drug.

In comments on 21st Century Cures, our trade institution, Pharma, support an expansion of FDA's ability to make decisions on therapeutic benefits of a medicine based on real-world evidence. They suggested, and we agree, such a policy shift is long overdue. It would have a profound impact on the efficiency of drug development. It would appropriately reflect our technological capabilities, and most importantly, it would benefit patients seeking new options to battle disease.

Of course, real-world evidence has applications beyond the review of new medicines and new indications. If there's one word that's quickly catching up to innovation within the healthcare nomenclature, it's the word value. The shift is on to value-based decision-making, payers, physician groups, engaged patients, bias -- biopharmaceutical companies. We are all focused on value and related frameworks. But as these frameworks are evaluated and put into place we need appropriate guardrails that preserve evidence-based decision-making by patients and physicians. Judgments about the value of a treatment are highly individualized, and they change over time as new research and treatment options emerge. We must use our available tools to reach such judgments, including real-world evidence. Understanding how treatments work in the real world is vital for health plans, providers and biopharmaceutical companies.

Payers want to know more about real-world patient experiences and they seek pharmacoeconomic data, but lingering ambiguities in related regulations serve as a deterrent not only for sharing such information, but for collecting it in the first place.

I understand that 21st Century Cures take steps towards addressing FDA's current regulations which prevent companies from sharing certain accurate data-driven information with healthcare providers and payers to inform patient care. That's encouraging, and I hope the Senate continues to focus on improving health outcomes through more informed clinical practice. In the meantime, at Astellas, we're looking at new ways to bring better value to our customers and patients, and real-world evidence is a key component.

For example, we are working with our partner Medivation on a prospective patient registry to better understand the unique needs of patients with castrate resistant prostate cancer. This disease-based registry called Trumpet, will roll and evaluate 2,000 patients diagnosed with castrate resistant prostate cancer from urology and oncology sites across the country.

We will follow patients for up to six years and gather information about the management of castrate resistant prostate cancer including patterns of care and the settings in which they occur. The registry does not evaluate any specific medicine but focuses on treatment patterns and their effect on the quality of life.

So why do we do it? We do it to learn. The insights from Trumpet can directly impact our research approach and increase our understanding of important -- important organ treatment considerations. And we do it as part of our commitment to provide effective evidence-based care for cancer patients and their caregivers.

The digitalization of healthcare information is changing the way we do business. It's enabling us to better identify what works and for whom, what doesn't and why. Tapping into this real-world, digitalized data cannot only help establish the value of an individual product but it also provides us with much more.

It helps inform our research and development, it provides better insights into disease states and it demonstrates how well the current standards of care work. While we all stand together to embrace the importance of robust funding for basic research, we can't lose sight of the role that the biopharmaceutical companies play in evidence development.

In 2014 alone, biopharmaceutical companies invested more than $51 billion on applied research. By comparison, NIH's total budget for 2014 was $30 billion. Now is the time to create a modernized regulatory framework that encourages the development and dissemination of accurate data-driven information which includes real-world data to improve patient care.

And we need to count on your impassioned advocacy to achieve these changes. Again, I'd like to thank Mary and her team for bringing us all together today. And I am very excited to hear the panel discussions which will follow. Have a great meeting.

(APPLAUSE)

WOOLLEY:
Thank you, Jeffrey. I think that's an important perspective for us to consider, and I'm sure that we will be hearing more about it today. So I would like to introduce now our first panel. Our moderator -- and I think they're coming up -- oh, here they are, OK. Our moderator is Richard Harris, a multiple award- winning science correspondent with NPR, which he joined in 1986.

He's been reporting over the years on a wide range of topics in science, medicine and the environment, and has recently shifted focus to emphasize biomedical research. He's on leave right now, which is really the reason I think he had time to come. And he's writing a book about reproducibility issues in biomedical science. Thank you so much, Richard, for being with us.

HARRIS:
Thank you.

(APPLAUSE)

WOOLLEY:
Now, the bios of all the panelists are included in your program book but I want to introduce them just by title. So Vincent Forlenza is the president and CEO of BD. Vincent. Dr. Gary Gibbons is the director of the National Heart Lung and Blood Institute. Dr. Anil Jina is head of global medical affairs at Shire. Ron Mobed is the chief executive officer of Elsevier. And Sudip Parikh is vice president and general manager, health and analytics at Battelle; and a member of the Research America Board of Directors. Thank you all for joining us today.

(APPLAUSE)

HARRIS:
Thanks very much. I think I hear the microphone; that's good. We have 50 minutes -- we have 40 minutes to talk among ourselves and then 10 minutes to field questions from you. So actually, you'd think that that's not very much time to talk about modern medicine, where is the science taking us, but as a radio reporter, 50 minutes, that's a pretty good chunk of time. So we'll see what kind of -- we'll see what kind of progress we can make on a whirlwind tour through this. And the first question I'd like to ask is actually to all of you, each of you, which is, just sort of stepping back and reflecting that we're at a really truly remarkable time in biomedical research right now. The technology has come along so amazingly. We had the genome sequence more than a decade ago. We have deep sequencing now. You've got all of these "knockout mice" and so on. You got just the big data. You've got personalized medicine becoming precision medicine. I mean, it's just amazing all of this foment, and people are turning and saying, OK, this -- you know, on the technological side,

the progress is fabulous, but it doesn't seem like the actual results in clinical medicine are keeping pace with the technology, that it's a -- and I'm wondering if each of you would sort of reflect a little bit on what it would take to accelerate that the transition from having these fabulous tools to actually making them -- putting them to use more rapidly.

And I guess we could start -- just -- we'll just down the line. I guess, Ron, would you like to start?

MOBED:
Sure. Thank you very much. I have to say it's a great pleasure to be here in Washington. And actually, I was thinking about your question. Many of you know this very well, but I recently happened across an NSF paper from 2004 talking about the confluence of four particular areas. And they named them as Nano-Bio-Info-Cognitive. And some of you have probably read that paper long time ago.

And when I think about what we see in our company around the world of research in general and medical research in particular, you see that confluence actually taking place. And I reflected on two things.

First of all, it's a testament to the forward thinking nature of research in the United States. That 10 years ago, this trend was identified and now, today, you are seeing the impact of those trends flourishing.

My general answer to your question follows on from that observation which is it seems to us at Elsevier that we are at the cusp of making that promise a reality. When you see the type of work that's being done, you're moving from the theoretical, the conceptual to real, practical implementation.

Some of the comments from the speaker earlier on supported that evidence-based medicine going into the patient, talking to the patient while they are being treated outside of the trials. And so, from our perspective at Elsevier we see the research, we see the clinical impact. And when I talk to customers and when I talk to our own employees who are involved in understanding what's happening with research in clinical medicine, we all are coming to this conclusion that we are right at the cusp right now.

HARRIS:
Yes. And Dr. Gibbons, obviously, as part of the NIH, it's your institute's job to bridge that gap, right? How are you are you thinking about that?

GIBBONS:
Absolutely. I think it is an exciting time. I reflect for the National Heart Lung and Blood Institute, and I think one of the major success stories in reducing coronary heart disease just by over 70 percent over the last 50 years, in large part, driven by investments in biomedical research.

And we have classic studies like the Framingham Heart Study to identify mediators of disease that then led to basic science research, the Nobel prize-winning work of Brown and Goldstein to identify targets and then pathways that are druggable and public-private partnerships to develop the cornerstone therapies like statins. And that took a period of time. What's remarkable as we've basically repeated that cycle just very recently, very quickly, with the new PCSK9 inhibitors in which, again, genomic science now sequencing people with the details of distribution of cholesterol to identify a new druggable target. And we already had those that are FDA approved within a rapid period of time. That's the nature of this technology. We're also excited about the big data that's emerging. So in Framingham, people would come in to a clinic visit every two years. Now when you think about what we can capture about their blood pressure, their heart rate, their sleeping habits that is now captured by mobile technologies and sensors.

We have the capacity now to create data sources that we can then layer on genomic information about those same participants. So at NIH, we now can create datasets that are incredibly rich that not only involve those classic clinic visits, but actually data that's person- centric that is derived from the new technologies. And I think the challenge for us is to promote a culture of data sharing where all of that will be available for everyone to mine, whether in the public sphere or in the private sphere, so that those new targets that new understanding of mediators continues to advance. And so that's --

HARRIS:
Yes. Obviously the genome sort of set the standard for data sharing for sure. Anil Jina will tell about this from the perspective of a pharmaceutical company. How come you aren't giving us wonder drugs every other week?

JINA:
Sure. Thank you very much for the opportunity to comment and the opportunity to be here. And I certainly would agree with your -- with the point which is, you know, we're at the cusp of something which is an evolution in the way we manage healthcare from research right through to a delivery perspective.

I think the one thing that I would say, you know, not even looking at Shire and pharmaceutical or biopharmaceutical companies in general, one of the key things that I think that we would need to do to progress this is to break down silos, is to enable a lot more partnership.

I think whether you're talking about, you know, working clinically in a hospital setting or working in an academic setting or working in a research setting or working in the pharma setting or working on the government or the payer or the delivery setting, we've gotten better over the years partnering, you know, within a certain elastic state with various partners that you must partner with. But broad partnership across the board and a coordinated approach to partnership, I think is lacking.

And I think that's one area if we really want to truly embrace this next level of precision medicine or next level of more personalized medicine, I think we're going to have to break down those silos. I notice that a lot -- obviously at Shire, we do a lot of work with rare diseases so we know that we don't develop all those ideas in-house. We collaborate a lot with external partners who have great ideas and, you know, our area of specialties by taking those great ideas and hopefully bringing them to a patient ultimately. But you see -- or I've certainly seen in the last few years going back to that breaking the silo and working with other partners, working with regulators in rare disease, working with payors in rare disease, working with other researches in other companies in rare disease is totally different than my experiences in working in other large pharma companies because, you know, that silo just has to be broken down in order to have that next level of innovation.

So for me, that's definitely one area to think about is how do we just have policy or, you know, ideas from all the different stakeholders involved at this particular cusp to break down those silos and enable us to work better together.

HARRIS:
Yeah. We should circle back and talk a little bit in a few minutes about the role of the federal government, what it could do to help on that. But let's -- let me keep the question down the row here to Vince Forlenza. What is -- what is your take on this?

FORLENZA:

Sure. So from the perspective of someone who's been in the medical device and diagnostics industry, you know, I can remember doing my first -- funding my first biomarker discovery program back in, like, 1984. I think fundamentally, we understand the complexity of the science and thinking about, you know, what's going on in genomics, it's totally different than the approach we were thinking about 20 years ago with identifying the single oncogene.

We have a toolset now that is so much more powerful. Those toolsets, though, are still very complicated when one thinks about the translation from the research to actually a clinical diagnostic, for example, and it's every step of the process. I think the exciting thing is that there is a lot of funding going into improving that toolset, but how you take that so that it's a repeatable process with the right sensitivity, number one, so that laboratories can employ it, and then, two, get it through the regulatory pathway and get paid for is really important. And that's where I think the 21st Century Cures work is going on.

AdvaMed has been also working with the FDA on this issue as a separate breakthrough pathway. So I think we have to solve those problems first. And then on the back end, when you have the result, the understanding of what that result means in the physician community -- because these are going to be complex, as I said earlier -- I think you have to solve that from the information and understanding standpoint.

PARIKH:
I may be building on a theme here, and at the risk of -- at the risk of saying that I'm -- everything's been said except not are going to set it yet, I'll be brief. I think the key point...

HARRIS:
I can throw you a different question if you like.

PARIKH:
For me the -- I think first of all, the fact that my primary care physician is talking in these same -- in these same words means that we've gone past the cusp. We're actually at the point where the dam is starting to break. And what's amazing to me is that this is by and organic nature through the silos. The silos have all made progress on their own.

And to me, the biggest thing we can do to build on the theme of breaking down the silos is to create something that's beyond the organic progress to something that actually has a strategy behind it, something that actually builds toward something, the final vision, and we need that. And that to me is my breakdown.

HARRIS:
Well, a lot of biomedicine in this country is focused certainly, and academia is focused on individual investigator, the RO- 1 Grant, et cetera. et cetera, and people pursuing really, you know, interesting ideas. But is that the right model if we're trying -- I mean, obviously, then it shifts into pharmaceuticals. But is there something to rethink that model do you think, or do you think or do think that it needs to be -- that that's still the right way of thinking about it. And well --

(CROSSTALK)

GIBBONS:

I certainly think that we -- I think we want to be innovative and adaptive. As we mentioned, we have the capacity now to create datasets that are accessible to more people with more analytical tools than ever before. And so I would agree with an element of that premise that funding sort of 'mom and pop' little elements alone is probably not sufficient.

But I would dare say that we -- one of the strengths of that model is to empower individuals to basically follow their nose and into to try to solve difficult problems. So for example, if we're thinking about something like gene editing technology that really potentially could transform how we think about curing certain diseases like in our portfolio of human globanopathy, sickle cell disease.

You then have to step back as to how did that tool come about. Well, because someone was studying the immune system of E. coli, and there are not a lot of advocacy groups for E. coli, and yet, those tools, those technologies, that understanding was fundamental to creating this tool that then could translate. So I think we need to be careful that we maintain that balance of allowing brilliant scientists to follow their nose and ask how systems work, as well as this translation that had that as part of a broad portfolio.

HARRIS:
Ron, did you want to --

MOBED:
Yes, let me build on that. I completely agree with the point about the balance. And from our perspective, when we talk to government funders around the world we hear that question about balance, but it's calibrated a little bit by something which I think is little bit new and a little bit recent around the need for demonstrating value, demonstrating return.

There is a little bit more of a pressure on what benefit does the taxpayer get for this investment and being able to demonstrate some kind of metric back to the taxpayer that there is a measurable return. So that balance between allowing the individual researchers to follow their noses, which I completely agree with, with this, how do I measure? And we've been doing quite a little work around that sphere.

Because without the -- if you can't convince the taxpayer of the value, then you don't get the funding to follow your nose. You have to balance these two. And so we spent a lot of time on that. The second point I'd make is around the role of government aside from funding, around creating frameworks. And that actually was mentioned in the earlier introduction. There are some times when governments need to provide a framework from which industry, commerce, other organizations can work.

In the absence of frameworks, it's very difficult to coordinate the different stakeholders effectively to get to the results, to get to the promise that you were describing earlier.

HARRIS:
Yeah. And Sudip, I know you have spent time as -- on Capitol Hill and thinking about science policy issues. Is there some -- is there some need to change the incentives a little bit and to scientists and say, maybe we need you to be in a team and working on a team on a particular project and not worried so much about proving something that will get your -- you know, get you your next RO-1 Grant.

And, I mean, the whole structure is an interesting place right now because -- yes.

PARIKH:
It is. You know, the framework is one that has really remained the same from Vannevar Bush forward, right? It's the same framework, and it's been wildly successful. Could be very careful when tinkering with something like that.

HARRIS:
Indeed. Yes.

PARIKH:
But, you know, are there -- I think NIH has already been experimenting with some ways of saying where are the incentives for a young investigator, do I want my name on a publication with a hundred authors or do I want my name on a publication with three? That -- the...

HARRIS:
Physicists don't mind being on a paper with 100.

PARIKH:
They don't.

HARRIS:
But the biomedical scientists, not so much.

PARIKH:
They do. They do And I think that that is something that the culture is slowly changing. The other piece of that is that you have to have -- you have to have both. And so how do you build those cultures both separately and together at the same time. And that's a very challenging problem for NIH and for NSF and for any other funding agency.

But I think that the key to that is seeing the problems that can be tackled. So when you think of something like CRISPR or something -- some other technique that's profound that comes from basic research -- it's wonderful to have those folks follow their nose, but then it's also wonderful when they can frame it in a way that policymakers can say that's valuable.

So I'll give you an example. I used to work for Senator Arlen Specter, and I was very excited. He asked me, what's something great that's happened in biomedical science. And the structure of the ribosome had just come out. So for the science geeks in the room the ribosome's a really important piece of the -- piece of protein material, protein and RNA material.

And I said -- and he said, OK, why is that important. And I said, well, you know, we were seeing that the RNA world was the-- predated the DNA world. And he said, I don't care. And he said tell me something I can care about. So while it turns out that, you know, you can actually use this information to create drugs, suddenly, that's where the value of NIH from a $7 billion agency versus a $31 billion agency became apparent to a

policymaker who's a lawyer who has to walk into the next room talking about -- you know, worries about defense.

You have to make it very clear, and that's something that the young folks, the new scientists, the younger scientists can start to talk about. And that culture is changing.

HARRIS:
Yes.

FORLENZA:
So maybe this is from a totally different point of view, but as a manufacturer, we are seeing -- so this is not directly in terms of NIH or what not, but if we look at NGOs and other organizations looking at, for example, the developing world, we're starting to see public-private partnerships being formed in the research area and in the commercialization area from a shared risk perspective and figuring out both how can they get paid back and under which circumstances, which is a pretty interesting model. And so it's enabling you to take more risk.

And in a -- in addition to that, they're looking at shared value. So where are they going to put the money and how they are going to create these frameworks that we were talking about before. They're starting to build in the societal impacts. You know, I wouldn't say this is standardized, you know, I would say it's all initial work that's happening right now. So maybe there are some analogies here in terms of the pure research side of things.

HARRIS:
Yes. So -- and speaking of value, I think it's a word that I expect will be said often today and is an interesting issue. I think one of -- value that people are interested in is not only having more medical advances but having them remain affordable. And I wonder is -- if you guys would like to talk a little bit about whether technology inevitably makes things more expensive or are there opportunities with using these technologies actually to make -- you know, to get more bang for the buck out of these things and to have -- you know, whether it's diagnostics or treatments or whatever -- actually be more affordable. So you do...

(CROSSTALK)

JINA:
I can certainly start with that.

HARRIS:
Yes.

JINA:
And it picks up on some of the teams that were highlighted by my colleagues just in the last few minutes. I mean, I think the amounts of research and what is discoverable is phenomenal. And I think allowing people to keep pursuing those ideas with all the different platforms that are available is very important.

But it goes back to a point that you were raising earlier is, you know, from a society perspective, how valuable are they. So I think it's very important to keep those incremental pieces of research going and going off down different rabbit holes in whatever direction they need to go to enable that innovation moving forward.

But what are the important things to think about from a society perspective, and then it gets back to that question, the value, all right? If it's of value to society, if it's value to a certain group, then it should be valuable to, you know, a number of groups who come together to think about how we actually can achieve this.

And I think that's one of the problems we see at, you know, every other day. And in the industry, you come forward great ideas, with great targets that you've been up to fulfill to hit a small number of patients or maybe larger number of patients, you get down to that point. The cost to society, the cost to payers and the incremental change, is it actually value --

It's certainly valuable to those patients, but in the wider sphere of society is it valuable. And I think if we're kind of left to our own devices, we will figure out -- and if just think about the rare disease environment; 7,000 plus rare diseases -- if you enable lots of different researchers to go looking for those targets, I'm sure over the next few decades we'll find solutions for a number of them.

But is that where we want to go from a society perspective, and I think that's the, you know, hard part.

HARRIS:
And the -- and the -- yeah. If each program costs $300,000 a year, that's a -- that's a solution that is not practical solution.

JINA:
And the development cost is now up to about $2 billion per...

HARRIS:
Two billion.

JINA:
... compound. That's the latest research you see coming out. It's not a cheap endeavor, so.

HARRIS:
Yes.

JINA:
So, I think it goes back to that original question. What do we actually want to try and answer?

HARRIS: Gary Gibbons, do you -- how do you grapple with that one?

GIBBONS:
Well, I guess I would cycle back to the example I gave. There is an importance and even in cholesterol. Part of the work of Brown and Goldstein was actually related to kids with a familial hypercholesterolemia, a rare disorder that I helped to identify one of the molecular pathways for cholesterol metabolism.

So, I think of it not as an either/or, but both end balance that we have to maintain that we gain insights into a broad target group of statins based on understanding that rare disorder. And now their generic and...

(CROSSTALK)

GIBBONS:
Yes. They're with the cost-effectiveness; I think is a quite compelling.

HARRIS:
Right. Pennies a day for the sense of Brown and Goldstein sort of laid the groundwork work for these new drugs at $14,000 a year, and all of a sudden -- then that is part of the value proposition, right? So, I mean, that the difficult to mention. Ron, did you want to...

MOBED:
So, let's now for the slightly different perspective because I'm not a researcher and I haven't been -- but I'm an engineer. And so, from an engineering perspective, when I hear the question, do you think that things will continue to be very, very expensive or will it get cheaper. There's of course only one answer, it's going to get cheaper.

And because there is an observation I think that a lot of the techniques that we've been describing are technology based and data based, information based. We hear people talk about personalized medicine about big data. These are capabilities that at the beginning are extremely expensive to develop.

The unit cost of production very quickly drops. The ability to search through vast quantities of information, it makes the unit cost very, very low. And then the promise is that it becomes the elimination of the reduction of failures. That instead of 1 and 30 successes or 1 in 10 successes, you get to 1 and 3 successes. That changes the dynamics of the environments.

Now, speaking deliberately as an engineer not as a researcher as you can tell me that it doesn't work in this industry.

(CROSSTALK)

HARRIS:
It certainly has not work in biomedicine. Yes.

MOBED:

But in the most, in many industries the combination of the technology -- and I'm pointing at this particularly because it seems to me when I talk to researchers, that research is getting so much more and more information centric technology centric.

HARRIS:
So, Vince...

MOBED:
And that's where potentially the promised lies. This could be something that changes those success dynamics in this industry.

HARRIS:
Yes. Vince, what about this, I mean, one way you can imagine and people talk about sometimes is, if you have better diagnostics that really pinpoint a disease, then you, and say, OK, this is the mutation, this is the treatment. On one hand, that's good because you don't have to give a drug to a huge number of people for whom it won't be effective.

On the other hand, you'll have a very smaller number of patients available to repair your $2 billion development cost. So, I mean, where do you see the technology leverage here?

FORLENZA:
Well, I think the good news is that the cost on creating a diagnostic is a lot less costly than creating a drug. And, but you're going to -- you have to tie those two costs together in terms of the system because you have to do that position medicine together.

And so, I do think in going back to what Richard is saying that you do drive the cost down. But there's a timing issue here because the costs are front end loaded. So, how you get those two things in alignment I think is one of the tricky parts of this. And so, because the benefits secure to the patient over time, and you have the cost upfront whether it's a surgery or whether it's a drug.

So I think we're going to have to get very inventive in terms of how we do those things.

HARRIS:
Sudip, did you want to add something to that?

PARIKH:
Yes. I think of it as just from the slightly different perspective of, you know, in the 1950's, President Eisenhower had a heart attack while he's office and he get the best standard care, bed rest. In 2005, my father a textile worker of North Carolina had a heart attack. He got $300,000 for the care. On the other hand, his life has been extended by 20 years. And multiply that by, you know, 10 million people.

And so, there is this other piece of it, which is that you get gained the value of the economic productivity of the people that they're going to spend a -- they're going to have a longer life. And so, you have to balance that I agree, that the fundamental question; how do you pay for it upfront to get that value over time?

HARRIS:
Yes. It's obviously, well, in industry I guess, who, I hope your investors will you give you money in exchange for return in the long run. I guess government is a little bit different because that's not as clearly couple together. You can, you know, you certainly have to rely on Congress to see the value of looking in the long term.

FORLENZA:
But there is second piece of this which is, you know, how do you coordinate your care. And this comes back to you now have the opportunity with information systems going in the direction that they are to tackle some of these cost issues and reinvest in some of the technologies that we all know about.

But when you think of 5 percent of the patients, you know, costing, you know, up to 50 percent of the healthcare system. There are rooms -- there's a lot of room to redesign the system to create more room for innovation while you pay.

HARRIS:
So, what role should the government play in that? And that anyone wanted to take on that question?

GIBBONS:
One role I think from the standpoint of the NIH agent and certainly NHLBI is related to that public-private partnership and breaking down the silos even terms of how we think about collecting data. We tend to go off and have a pristine clinical trial that, as you said, maybe difficult to walk back into clinical practice.

What if we started to engage in more embedded research within systems that are actually functioning in the real world and collect that data. Now, that a lot of that is digitized. And so, I think it is going to challenge us to think about different models of how we learn new things about drugs or their additional benefits and make that part of our population laboratory. That's one opportunity we have with a digital age.

HARRIS:
Yes. And I think that's a huge public education issue. I mean, I remember when your colleagues announce the, you know, the latest iteration of precision medicine. One thing that they mentioned was, oh, when, you know, maybe your Fitbits or other electronics will be gathering data that they think can be shared with researchers.

And I thought, what I would -- what I want that to happen without knowing, and then, a little while later I look at my iPhone and I realized it has been collecting my -- it's been counting my footsteps. I didn't ask it to.

You know, but, there's a huge data there, maybe, you know, under what circumstances would it be appropriate for industry researcher somebody to tap into that. You know, these are -- I guess that -- I guess it didn't charge to be a question. But, if someone knows, somebody I went -- if anyone who would like to have a thought about that I would be interested.

GIBBONS:

Well, certainly, it challenges what we mean by the informed consent for research. And being sure that, indeed, we maintain those principles of beneficence and privacy protection. But, as you pointed out, there is data being collected on you right now that -- and sometimes being sold, that you really probably weren't aware of.

HARRIS:
Who knows, this conversations being streamed across the world.

FORLENZA:
That could happen.

HARRIS:
Well, Ron, actually, let me turn back to your earlier comment about and thinking about how to make these things more efficient. And one thing is -- I was mentioned I am writing a book right now about biomedical research and the reproducibility issues involved in that. And part of it is, I mean, science is going to always have is never to be perfect. You're going to go down by now we're seeing one.

But one thing that you could do if you can figure out a way to do it is you could reduce some of these easily preventable errors of people, you know, using someone's that are inappropriate not authenticated or whatever. And so, in the journals could play a very important role in that. And I wonder if you would say a few words about whether, you know, whether how -- are the journals in general stepping up to this.

I mean, everyone wants to be published and sell, right? And so, you have tremendous power to make a difference here.

MOBED:
So, earlier on I was hearing from Mary that you're writing a book on reproducing ability of it here. And now you've asked me a question about reproducing. But it seems to me that maybe we should be switching chairs. But let me take your point. So, reproduced ability is something actually which we, in Elsevier, spent a lot of time on internally but also collaboratively with others.

A couple of remarks but I recognize that these are things that you will know very, very well. First of all, when we think about reproducibility in two ways. First of all, is there enough information about the experiments to reproduce it in the first place? And then secondly, was the way in which the data presented that ethically correct? Or was it somehow doctor said it does not become reproducible.

And there are two separate issues that both important and we welcome both of them. Within the individual environments of journal publications there are some things that we can do. One of things that we can do is make access to the underlying data easier for the reader.

And so, we've now connected papers to 200 separate institutional data repositories. That if you're reading a paper you can click on hypothetic test. It can go straight to the unlighted data. These are very much pairwise links that we negotiate with each repositories to make sure they're happy for this to happen, or the researchers happy for this to happen.

Such one area that you can work on getting the data more connected to the underlying research paper in the first place. Another thing that we do is there is a lot of work going on around methods. Because if you -- one of

the issues around reproducibility it came up about nine months ago in the press, is that sometimes it's difficult to reproduce because the methods are quite complex.

If you don't take all the steps the same way as the original researcher, you may not get the same results. It doesn't mean the original research was wrong. And maybe you made a mistake, maybe you didn't follow the steps.

HARRIS:
Right.

MOBED:
Maybe you weren't presented with the steps correctly. So, we've been working on that within Elsevier and we've actually created across Science Journal called MethodsX, specifically to allow people to get into great detail about method. That's the second that we're doing.

Now, we're actually working with the NIH. Elsevier published at Cell in the family of Cell Journals. And so, press is working directly with NIH on issues around reproducibility because we have a program going on over there. And we're working with an institution in Germany as well, around mostly from the second language, the data integrity piece of the reproducibility.

So, we see the -- because the journal is meant to be the document of record it should be the place where it has the integrity that you're looking for in reproducibility. And I think there is a lot that we can do, especially now that we can move away from paper and we can go digital, we can help that environment move much more aggressively towards an environment where you have everything you need to know to reproduce the experiment.

GIBBONS:
It's just that this is an opportunity as a community to respond to this challenge and there has been a nice partnership between NIH and the journals as switch or the safeguards of the public with the data. And I guess I would say that it only add that we're instituting some of these things on the grant application process as well, such that, we ensure that we're funding research that is based on rigorously done on science both in preliminary data as well as its execution.

HARRIS:
Are you worried that this might undercut public support for science at some point?

GIBBONS:
Well, again, this is a public good. And it's critical for to in general, public trust, and have integrity. And so, the storage that's why we think is important thing to ensure that what we fund has that integrity and we're setting a bar, if you will, of expectation of those who receive taxpayer funds. That this is the expectation of rigor and coupled with the general reinforcement of that, I think that something that community will step up into.

HARRIS:

Yes. And I must say that in my reporting so far in the book, I think that people are very upfront about this. They recognize it's an issue and they're not trying to suit it under the rug. And I think that's a laudable approach to it. But it's also, you know, what has to be where -- here we are talking about thinking about potential, you know, trying to convince Congress that there are this entire enterprises is heavily we're supporting, and this is an issue that can raise questions. Did you want to add something to that?

PARIKH:
Sure. The example that we're saying here is actually you ask the question what can government be doing to foster what's going on here. And that example is a singular example of NIH and an industry working together. That framework can be provided by government. Part of that is 21st Century Cures. But there are other things that are provided that framework to allow that cross collaboration, again, with the goal in mind.

And I think that the goal isn't just about a target for a cure, it's about that that is one way of looking at reproducibility, it's about how we get the promising candidates into the pipeline for orphan diseases, what kind of collaborations are needed for that, how does engineering workforce seem to be provided for by NIH in the same way at the molecular biology workforce that are being provided for by NIH.

These are things that require a framework and require funding. And there are two things are coupled like they are in the 21st Century Cures Act or when there are thoughtful preparations as opposed to a continuing resolution for a full year. We can make a lot of progress. When those things are not done in a thoughtful way we have that same organic on a one-off basis of these collaborations and that's a real problem.

HARRIS:
Yes. Vince, you want to say...

(CROSSTALK)

JINA:
I completely agree with those points and just, you know, just have one of the questions you asked at the start about government and changing mindset and paradigm. I mean, I go back to the example you said about you're collecting steps and it's going into some database somewhere. I think the paradigm of research the way we've been doing it, you know, the single investigators, the multi- investigators, the randomized double-blind, you know, that all approach.

And the methodology piece which we are mentioning I think a lot of that needs to evolve. We have massive data sets that don't really collect data in the same way but are equally very powerful as long as the reds appropriately and as long as, you know, the reproducible. But that's definitely a paradigm change which we could embrace a lot of the technology pieces, and actually, you know, use the power of that to do something different.

So, if you look at the development pathway for, you know, biotech or pharmaceuticals it still very much follow that same paradigm which has been around for a long time. It's long, it's expensive, it's costly, it leads to a lot of them expensive the other end.

But, I mean, there are ways to think about the requirements in different way more real world, more real time and think about that paradigm differently. I think that's an opportunity you need to follow in there.

FORLENZA:
Yes. I guess the question would be if this is a problem are you going to fund -- are we going to fund the program and divert some resources to getting at reproducibility? Because I don't think it happens on its own. And so, when you're thinking about grants what kind of grants are going to get approved? Does this get into the queue and the appropriate way?

I think it's been much more driven by, OK, this is really interesting science as opposed to the methods that we need to be usable. You know, we have the largest blood collection business in the world. And I can tell you when we look at our research customer base, by and large, they are not using the state-of-the-art methodologies in blood collection to preserve proteins and what not.

There is a fraction of them that are. So, I think it's a really interesting question that you start to investigate.

HARRIS:
Yes. And it seems as though, there is a role for industry to come up with some solutions. I mean, this is partly, I think we spent the rest of my time talking about my book but which book. But with questions and answers are coming up momentarily. So, let me just turn -- let me just take one step back and ask Dr. Gibbons of a broader question.

We've been talking about technology here and these sorts of solutions to healthcare. But there are -- in moving things forward. But there are also we're reminded that there are disparities in this country, around the world course, in how -- in people's health and it's not purely a matter of diagnostics and drugs and treatments and all the rest of the stuff. There are other broader issues here.

And I wonder if, I know it's something you've been thinking about a lot and I wonder if you just want to say a couple of words just sort of, you know, just to broaden the conversation before Q&A here.

GIBBONS:
Yes. No, I think that's another dimension of what we're talking about in terms of systems and data. It's appreciating not only that we may have your genome sequenced, but in part related to digital age, we can get much more information about behavior and social contacts.

And so, if we're trying to address an asthma care in the NHLBI, yes, we'll have genome sequence. But we also may have a sense of the neighborhood where you live and work or play and the air pollution and ozone levels in particulate matter that may exist there that may also have an impact in your health outcomes. And have a way now integrating some of that social context of information.

So, similarly, in fact, some of the individuals at the Lawrence socioeconomic status actually use this mobile technologies even more because that's their real source to the internet. There may be opportunities then to put in reminders, to promote greater adherence to their therapy by, again, leveraging some of these technologies.

We want to be sure that we don't create a digital divine, in fact, we try to leverage it and wait to maybe actually shrink help disparity. So, I hope we take that inclusive approach.

WOOLLEY:
All right. That's what we're saying.

HARRIS:
All right. I think it is time for question-and- answer and I am guessing that there are going to be microphone circulating around in the room. Is that correct? So, if you have question, please, raise your hand. Someone with a microphone will come to you. Please, introduce yourself and ask a succinct question.

HENDRIX:
OK. Thank you my name is Mary Hendrix. And I have the privilege of serving on the board of Research America and also run a research institute for Northwestern University. I think I have a new chapter for your book, or maybe something we could think about.

As a working scientist, I think the public is unaware of the fact of the tremendous variability that exists in commercially purchased reagents that we all use in the laboratory. Many of these reagents come to us they can be totally inactive, antibodies not working. It would be great if the government hosted a site for maybe preferred vendors or reagents just a site where researchers could feed into it.

And so, you know, this one really doesn't work. Don't bother purchasing it. It's just a thought, but it's something that we usually don't talk about.

HARRIS:
That, do you think that would be popular, Vince?

FORLENZA:
As a manufacturer of very high quality. We'd be happy to be on the web site as a -- so. I think in the research community that might be possible.

HARRIS:
Yes. All right. Thank you for that.

HARRIS:
You'd support that for the...

FORLENZA:
So, we're very happy to get our quality out there in any way that we want and you can, you know, and plenty methodologies. I think that I don't know how broad that problem is quite -- you're saying it's quite broad. But, I know that you need the government to get involved with that one and to do that.

HARRIS:
It is a conundrum, yes, for sure.

FORLENZA:
I think you can create a web site and start -- there are a lot of web sites and talk about these various things now.

HARRIS:
There are. Although there are --- there are some web site that lists the hundreds and hundreds of cell lines that are not authentic.

FORLENZA:
Yes.

HARRIS
And the journals unfortunately, do not even go to them to see before, maybe sell those but many other journals do not go to actually see. And you can still see papers published that are based on faulty cell lines.

FORLENZA:
That's part of your reproducibility that we've been talking about before.

HARRIS:
Yes. Anyway, more -- who's -- I don't see where the next microphone is. Something where is the hand? Who has a question? Here we go.

WOOLLEY:
Thanks. Mary Woolley. So, all the conversation about data I thought it was really important and I wonder if we can hear a little bit more. Now we, maybe from the perspective of are we going to fulfill the promise to the patient of the highest and best use of data.

We know from the public opinion polls that we commission at Research America that very large majorities of the public say they would be willing to have the science community and the medical community use data to help assure better healthcare delivery, better healthcare for themselves and for other people.

So, your possible comments on how we get there and what are the barriers that we might have to overcome.

HARRIS:
Yes. You want to.

GIBBONS:
Yes. Just one thing really to Mary's point, we also made a deal with the sort of challenge in history in which, as a researcher, we talk about research subjects. In which it was about my RO1 in which you contributed to get my data that I'm then going to publish in Cell.

What if we actually thought about it in terms of it being your data and that you still have control over your data. And if you do want it shared you might be able to change that equation about how and who shares it and under what circumstances. And to the degree to which a lot of the data and information we're talking about may actually be generated by you from your sensors or where have you. Actually it is your data.

And so, I think this is one of the challenges for us. It's not as I'm going to be the PI who's going to be the sole determinant. It may, in fact, again, if you all the different kind of partnership where the participants in research have a say about where their data go.

(CROSSTALK)

MOBED:
I think it's a really interesting point. In the U.K., a similar survey was done where people were much happier to release that data if they were told the data belong to them as opposed to belonging to somebody else.

And interesting there is a small company in the U.K. at the moment which is building of that and trying to create a mobile app where the individual on their cell phone own the data on the phone they're taking the cell phone to the clinician and saying here's my data, as oppose to you got my data. I don't know what you're doing with it. And I show up and you tell me that, you know, I'm 35 years old and I'm actually 43 years old or whatever happens to be.

So, to switching that over, it seems to be one of the ways, commonly now listening to you that the discussion with the public moves away from what are you going to do and what it gets loss, what if they get stolen, what happens to my health insurance to -- it's your data.

JINA:
And I think you also see it in areas if you look at something, an example, like Cassian's like me, the amount of people who really want to be part of that whole process and on the last time I spoke to them as tens of thousands of people who want their information to be used in a research-type. They want to share their information so that it can be used once pulled together to enable further research in their specific diseases.

So, I think it's a really important points that, you know, given the choice people actually want to be part of these things. And if you look at, I mean, so I studied medicine and the one piece that I've learned a lot about in terms of cardiovascular disease with the Framingham site. I recently moved to Boston, I drove through Framingham's and it's a famous thing for me and I know you feel like rolling down the window of my car and asking people how do they feel like to be part of this massive data set that really, you know, enables the cardiovascular research to move forward abounds.

If we think about the power of Framingham or something like that on a global scale for people who are willingly sharing lots of things in real-time and it is absolutely phenomenal what we potentially could do and this day and age with the technology we have.

HARRIS:
Yes. Sudip, do you think that would actually encourage people to think more about, you know, more positively about research and general and feeling like if they're part and parcel of it that this would be a sort of a good -- basically to have them a participant.

PARIKH:
Absolutely. We have, I mean, we can -- there's anecdotes about they'll all around. So, if you look in Framingham say they're people who are proud to be part of this stuff.

HARRIS:
Absolutely.

PARIKH:
If you look at studies like the black women study, another study were people are proud to be 20 years in and there is a small certificate on the wall is worth a fortune to them. So, those engagements are important. The conundrum is that everyone feels this way. But if you pretended any of the sort of sessions about the precision medicine issue, you run to all these questions about what can you -- how do you do this. How do you actually make this practical because everyone wants to share the data.

There is a million veterans study, there's a, you know, there's a studies all around with lots of cohorts to how do you actually blend that data together and there's not an answer to that yet. And that is where this dichotomy between what the people in our research we actually want and what we're able to provide as taking place.

And so, the way around that is to have this personal discussion which is when NIH started. But then after that is the action to say what it's going to take. Does it take changes the privacy long, does it take -- does is take during the 21st Century look at how we handle our data versus the 1990s HIPPA laws that we're looking at.

HARRIS:
Right. And we've done so well with the electronic medical records out of that way. So, it's the model for this. How not to do it.

I think that we have another question over here.

HAIT:
Hi. Bill Hait from Janssen. As reformed academic now and in terms of the industry act, I want to make a comment and ask the question. I really appreciate Dr. Gibbons comment about curiosity driven research and then the discussion about value.

And I think one of the things that strike me is to talk about the value of the generation of new knowledge and what that means to ultimately to society. But the question is how do you frame that discussion of the value of generating knowledge as opposed to say, well, we discovered a new targeting, therefore, we're making a drug.

GIBBONS:
Am I supposed to answer?

HARRIS:
I think he was looking at you.

GIBBONS:
Well, that's what I think Research America does brilliantly and the collective in this room. I think that's our challenge. That's our value proposition that has to be made to enable the public to have a grounded understanding of the importance of discovery.

And some of them has been as you call yourself a reform academic, this notion of actually how scientific discovery works that they didn't know there was a statin at the end of starting that first question. And so some of that, as I said, by the nose, has to happen as part of the process.

So, that's why I have difficulty with the either or. We all want to target is just what's the best way to do it. And for, from the government standpoint, it makes more sense for us to do the research investment on that front end. Actually, quite frankly, the risk is and because there are going to some rabbit holes.

And it's helping the taxpayer understand that in the end with the public-private partnership there's going to be value. But the public part has to create this larger pool of public good where new knowledge is going to pay off at some point.

HARRIS:
Now we have 40 seconds left. Maybe one super quick question on all of this, a wonderful point to end on actually. I'm always reluctant to take another question right from you, particularly from this guy.

DZAU:
This is Victor Dzau from National Academy of Medicine, formerly the IOM. So, the question which been raised 21st Century Cures certainly saying that's remove all the photos let's move to discovery faster to cures. But there is in fact, a couple of issues that we have to discuss.

Gary mentioned human gene editing. As you know, in all academies we're working very hard in addressing this issue. The question is what's a -- where should be responsible for the sponsor of research conduct research lie among the scientists being to know how fast to move, moving genetic to human embryos? Where are the regulatory framework and who -- where are the ethics around this?

So, how do you create the framework around this as we're struggling to think through this at the academies? But since you've been talking about really this issue about advancement in science to faster cures. When technologies are actually moving faster, then in fact, regulatory frameworks and others and, yet, we say, they're holding us behind.

When yet, this, in fact, if you think of gene editing there is a real reason to have that kind of oversight and framework. So, can you say a word about this?

HARRIS:
Again, it's a fabulous question. I think we're already overtime, and I wonder if maybe you could take on over coffee unless somebody has a 30-second answer. I mean, it's a really deep question and I don't really want to give a short shrift. But unless somebody wants to take 30 seconds, I think we should -- I think we should leave that hanging in the room and something really interesting to talk about.

So, thank you all very much for your attention. Thank you.

WOOLLEY:
Thank you to this panel. And we'll be back in just a few minutes with the second panel.

(BREAK)

WOOLLEY:
OK. We are going to begin the second panel. If you'd please, take your seats. Please take your seats for the second panel. Hell, everybody. So, welcome back. The moderator of our second panel this afternoon is Dr. Seema Yasmin, an award-winning journalist at the Dallas Morning News. Dr. Yasmin is also a professor of public health at the University of Texas at Dallas and the medical community -- contributor for CNN and NBC Channel 5.

She served as a disease detective in the epidemic intelligence service at the CDC. Her work has appeared in peer-reviewed medical journals as well as scientific American Thompson Reuters and The Huffington Post. You can read more about her and all of the panelists in your program.

Our panelists this afternoon are Dr. Richard Kronick, the director of the Agency for Healthcare Research and Quality or AHRQ. Dr. Lucinda Maine, executive vice president and CEO of the American Association of Colleges of Pharmacy and a member of the Research America Board of Directors.

And please feel free to come up on the stage. And I call your name.

Barbara Newhouse, the president and CEO of the ALS Association. Dr. Anne Schuchat, the director of the National Center for Immunization and Respiratory Diseases at the Centers for Disease Control and Prevention. And Dr. Reed Tuckson, managing director of Tuckson Health Connections.

Thank you all for being with us.

YASMIN:
Thank you so much. So, real privilege for me and for all of us to be able to engage in discussion with you because you really are global thought leaders when it comes to many of the public health challenges that we are facing.

But before we talk about those challenges, I want to put you guys on the spot and I want to challenge each of you, find with you, listen in and moving this way to tell us from your perspective and the perspective of your agency what you believe to be the single most pressing public health challenge that we're facing right now.

MAINE:
I would have to say that our inability to prevent and adequately control chronic illness is becoming the greatest public health challenge. And it's one of course that we're very interested in, in producing the medication used experts that have to try and help patients manage those chronic illnesses that we don't prevent.

SCHUCHAT:
I'd actually like to say our interdependence as a species is our probably our greatest public health challenge. And I think you can just say, what does an earthquake in the Philippines have to do with an Amish community in Ohio? Well, that's how we got a big -- the largest measles outbreak that the U.S. has had in 20 some years.

Why does a family going to Disneyland or a nurse in Dallas have to worry about the rest of the world? Well we're completely interdependent. And so for the CDC the idea that to keep Americans safe and healthy requires strong health protection in every country. Because the weakest link in the global chain can mean problems at home, as well as devastating problems abroad.

TUCKSON:
I'd like to build on both of those points. Number one is there's no question that the tsunami of preventable chronic illness is absolutely unaffordable for this nation. You cannot medicalize your way out of the magnitude of preventable illness that is pouring into a delivery system that we already can't afford.

So, I think that's very important. I think related to the interconnectivity around the world is the interconnectivity of the causal factors that either produce health or lead to preventable misery and suffering.

And so, there I think the number one issue ultimately becomes doing the research that allows us to better understand and at much greater level of detail - the intersection between genetics, behavior, social determinants of illness, the physical environment, and medical care.

If if we don't have a better sense of how those things fall together, then the interventions will continue to be suboptimal. We will not be as efficient in targeting and we will not do as very good job in monitoring the progress that we make.

So, I think it's the research agenda that looks at the interconnectivity of these issues that produce the preventable chronic illness, which we, as a nation, will not be able to afford.

KRONICK:
That's a really good question and I just building on the, you know, previous answers. There is a very long list of challenges that we could choose from.

YASMIN:
Just pick one.

KRONICK:
You now, obesity, tobacco, opioid depend -- use and drug use, you know, way too high infant mortality particularly among women who are disadvantaged, antimicrobial, you know, bacteria. So, we have a lot of problems. I would focus on a more systemic problem, which is the challenge of figuring out how to deliver the care that we know that works safely and with high quality.

You know, the previous session was focused primarily on what, kind of figuring out what works, that a very big public health challenges figuring out how to deliver that care safely. And, you know, we've made progress and a report that AHRQ put up at the end of last year showed that hospital care is a lot safer now than it was three years ago.

We've reduced from 145 adverse events per thousand hospitalizations to 121 adverse events. So, adverse events being central line infections, urinary tract infections, catho-associated infections, falls, pressure ulcers. And that was, you know, that's tremendous progress, 1.3 million fewer bad things happening with 50,000 lives saves as a result, which is more than one year of deaths from breast cancer.

So, you know, pretty amazing progress made possible in part by the evidence that was produced at AHRQ by research that we have funded create a, you know, created by the very hard work done by people in hospitals and supported by work that CMS didn't in the prohibition for patients much more need to be done.

And that count to the safety side, you know, parallel and even larger problems in quality of care. We know that people with hypertension and high cholesterol barely more than half have it under control. We know how to do that. We know what needs to be done to fix it. But we don't know how. And we at AHRQ have been investing in research and making good progress that clearly much more needs to be made to solve that basic public health.

YASMIN:
I'm so surprise that you didn't up a lot of political will or funding issues as a public health, kind of we will get to that.

KRONICK:
I'm assuming it will have a chance to get to that.

YASMIN:
We definitely will. Barbara.

NEWHOUSE:
I don't think you're going to let me get or saying all of the above. So, let me add -- I think that access to specialty diagnosis is a big issue. So, in a disease like ALS, for example, they oftentimes on are diagnosed or misdiagnosed with so many other things. So, by the time they are given a diagnosis of ALS they are already progressing down the way.

And I think that happens with all of, well, -- in the neurodegenerative space. I'm going to tell you, I believe that happens a lot because there are so many intersections. And I also -- I would say that that's another big issue and I know I'm up here with all these really smart people.

But I think that the interconnectedness of looking at the various diseases is another big issue that we face in public health. Because sometimes we are so caught up in 'this is my disease, this is my disease, this is my disease,' that we, sometimes I believe miss the obvious.

YASMIN:
Wow. That's really interesting and I love that you brought chronic diseases so much. I teach public health and for many of my students they equate public health with infectious diseases and outbreaks and that's it.

So, we'll come back to some of the challenges that you've raised. But, Barbara, I do want to take a step back and hone in on your expertise as someone who knows about public engagement. We think about ALS now. We think about the viral phenomenon while some of the ALS ice bucket challenge and you raised over $115 million. And what really sticks in my mind is that there was one day in August last year where you raised about $11.5 million in one day.

So, I want to think about the public component of public health. Because we think about the challenges, we often don't talk about the public's world. So, what can you share with us about how to nurture public interest in public health, and then also how you harness that passion to advance the field.

NEWHOUSE:
So, first of all, let me be very clear. The -- I don't think there is any ALS organization in the ALS space that takes credit for the ice bucket challenge. Now is really a very organic happening. I started with three young men who all have ALS. And they went to what they know and that's the sports analogy or the sports thing that people don't buy from themselves at the -- on the couch at the end of the game.

So, they use that and it move forward. What we did and I think, though, I could speak safely on behalf of the whole ALS community in terms of the other organizations, is what we did is we simply join their journey and began to use that public forum as a way of educating people about what ALS is, and what it means to have ALS.

And so, I think that what we learned out of that is that we've got a great forum, a great platform for educating folks and engaging them in whatever that cause is. And engaging them in a way that makes it come alive to them. Because when you saw those three young men I know my son he's 30 years old looked in the mirror and said, hey, mom, that could just as easily be me.

And so it made it personal. It made it personal. And it's given this platform for new patient engagement and for ways for them to have a voice in all of the work that we do every day.

YASMIN:
OK. So, that's harnessing publish passion. Richard, what about harnessing political interest and support. So just in case some of you are unaware, a few months ago, there was a draw congressional standing bill that next year which proposed eliminating all funding which organization, all of it.

And we're talking about an agency that as you mentioned works to improve patient safety to make healthcare more effective and also to save taxpayer's money. So, Richard, how do we get to a point and this wasn't even the first time I don't think. How do we get to a point where politicians will consider just swapping funding for an agency that does what it does?

KRONICK:
I take responsibility, I and my colleagues, for maybe to do a better job of explaining what the agency does. And I would ask all of you here who are supporters of the agency. And I know that many of you are to help in amplifying that message.

You know, the budget for ARHQ is about one, 100 of a percent of national health spending. And the mission of the agency is to produce evidence that will make healthcare safer, higher quality, more affordable, and accessible into working to try to make sure that evidence is actually understood and used.

And we do have, I think, you know, some pretty remarkable stories of success. The one that I started with, you know, 50,000 fewer death in hospitals, 1.3 million fewer bad things happening. And part as a result of work that we have done at the agency.

We're working with seven grantees around the country and the evidence now initiative. The recruiting 5000 primary care clinician and working at trying to figure out what sorts of supports these clinicians need to

improve cardiovascular outcomes for their patients. Aspirin, blood pressure, and cholesterol, smoking part of the departments million heart's campaign.

Many things that we are doing I am the colleague need to tell the story better and that story needs to be amplified more effectively.

TUCKSON:
Well, I think you do need to, you know, as you say, take -- tell the story about it. But also, I think the people who are the stakeholders for your products need to do a better job. There are lot of people sitting on the sidelines here. You have a great opportunity today.

The last panel talked so much about value. So, if we look at value the way in which the delivery system is reimbursed. If you want to make any money as a provider of healthcare, you have to deliver value. And that value has to be transparent, both in quality and cost-effectiveness and patient satisfaction, which is key.

If you're going to be a patient you will inevitably be spending much more money out of pocket in having a much greater financial stake in every decision you make around how you interact with the healthcare delivery system. And if you are a device manufacturer or pharmaceutical company you must deliver value. Therefore, wouldn't you think everyone will have an interest in knowing what works and what the cost-effectiveness is of what I do. Since it's my financial stake at -- that's on the line now.

So, this is -- yes, one that we can appeal to the violin playing which we should do, it's beautiful it's important. At the end of the day, enlightened self-interest are to drive a huge amount of support for AHRQ. And we are hoping that we can get other stakeholders to decide now that they need to act on their own best interest.

YASMIN:
OK. Well, what you make a good point about demonstrating value and one place that that's happening is in healthcare delivery and the use of telemedicine, right?

So, I wrote recently on how telemedicine's being used in Texas prisons to deliver really standard of care to prisoners and it’s improve health outcomes that patients with HIV that are living longer. It improved blood sugar control for diabetic patients and, you know, is also save a millions and millions of dollars.

And yet, still, we look across the country and we're seeing a patchwork of state regulations around telemedicine. And I'm thinking also like Idaho and Arkansas, they're quite prohibited. So, if we are demonstrating this effectiveness, its cost-saving why aren’t we promoting something that would actually save money?

TUCKSON:
Well, I appreciate the President of the Telemedicine Association; I thank you for those comments. But to drill into the purposes of this meeting with Research!America in this forum, there is no interest on my part for supporting innovation for innovation sake.

I could care less that you got a new thing with 14 bells and whistles and lots of excitement. Does it do what is supposed to do, does it drive up quality, does it take out cost, and does it make patients more engaged in making personally appropriate healthcare choices and decisions?

This field of telehealth, the beauty of it is, it is moving so fast. It is so -- it is now exploding in all areas of how healthcare is delivered. That means we must have evidence to guide how it's used, when it's used for home is it appropriate, under what circumstances.

So, I could make any appeal it comes back to Research!America's advocacy for AHRQ and Research!America's advocacy for CDC, in addition to the vaccines, but for the guide to community preventive services. These are the places where we've develop the evidence that allows regulate doors and other people at the state level to make intelligent decisions.

The last I'll say is, if I'm a member of the State Medical Board I've got a hard decision. I have an ethical and moral responsibility to protect the health of my citizens, to make sure things are done well. I also have a problem with the learning curve being really, really fast in trying to keep up with the developments.

I would want to turn to the literature to be able to look at evidence to tell them what I should do. The problem is we don't give you enough money to help develop the literature-base and the researchers to turn out this work so that we can answer those questions. So, I am mad at the State Medical Board. I'm mad at the fact we don't have enough research. And thank God for Research America.

YASMIN:
I think Anne wanted to comment first.

SCHUCHAT:
Yes. I just wanted to add comment. I think the first time I actually saw telemedicine in action was in 2003 in Beijing during the SARS epidemic, where my thought here is that things like telemedicine have to solve the problem. It's not about the groovy technology or the cost really. It just needs to solve the problem.

And the Chinese had a problem that a lot of healthcare workers were getting sick and dying. There was an outbreak in many different hospitals, they needed to get the x-rays of the sickest people in an efficient way you'd quickly by the best experts they had. And the differential between, you know, tuberculosis reactivating because people are steroids for their SARS.

They essentially use this. It was one of the things they did well early when they did many things poorly. The other thing that they did well though, in the long run was learn from their problems with SARS, with being lead footed with transparency issues, and so forth.

And when they really had that horrible experience with the human loss as well as the embarrassment of really not being up to snuff, they really invested in public health and they are so much stronger than almost any other country right now.

So, when the 8, 7, and 9 influenza or bird flu statin emerge a few years ago, they were quicker than ever affecting it doing whole genome sequencing making the sequence available to the world pretty much instantly, and really got ahead of their complex live bird market problem.

So, I think they were solving a problem with telemedicine at the beginning, but then they have to solve their bigger problem of, you know, a really bad emerging infectious disease knowing they will be hosting the Olympics. You know, it was a huge thing for them to get their act together.

And I think the example for all of us right now when we've been through the Ebola problem when we know that we all need to do better as a global community to really channel our problems into improved performance.

YASMIN:
Richard.

KRONICK:
I would certainly not argue with Reed's comments that the agency could make very good use of substantially more resources. I would point out that in telemedicine we did fund a few years ago, a Greco Project Echo out in New Mexico that tested the safety and efficacy and cost implications of telemedicine to connect specialists to patients in rural areas.

A very successful project, VA and DOD have been using as well as in the variety of other places picture questions Seema, they are currently as resistance but there needs to be better evidence about the safety and effectiveness. And we've developed some of it. There are still a place -- some places where we're more evidence would be useful.

YASMIN:
Yes, Lucinda.

MAINE:
Just very quickly. I think the regulatory framework for healthcare is going to be the key impediment to the movement of telemedicine. And I think in our space I saw players come into the distribution of pharmaceuticals, especially the importation of drugs from out of the country.

Even you mention the drug abuse epidemic and technology has been a facilitator of some of that, which has made the regulatory community, I can tell you with pharmacy very reluctant to think about expanding it without better evidence that it can be done safely and effectively.

TUCKSON:
You know, what's exciting though, and I want to elude back to the last panel, although this is the prevention panel but on this point which I think it still remains for the research for preventative research agenda; is that telemedicine is going to be a very important player in decreasing the costs of conducting clinical trials.

It will also be able to -- now with increasing enrolment through social media and other mechanisms you would decrease costs in terms of the need for folks to come back every two weeks for visit. You can do a lot of things online, the wearable technologies are going to bring forth a huge amount of data, and then of course, patient reported outcomes.

Issues will be of certainly streamlined through it. So, I think that one of the exciting things in the modern research momentum is the application of telehealth technologies. And we're starting to see conferences now that are focusing just on that principle.

YASMIN:
But you also made a really good point about agencies telling the story better. And so, listen, I want to ask you to talk about the role of pharmacist. And I teach 65 students twice a weekend, most of them are pre-med, and one of them wants to be a pharmacist.

So, I want to ask you about the role of pharmacist in public health. I want to put it in the context of the specific public health challenge, and that would antimicrobial resistance. Mary, it is off with the session earlier today talking about putting public health in the context of the national security.

And the superbugs ask something that could affect national security. There was a cystic reasoning that within the next 30 or so years, superbug infections could supersede cancer as a major cause of death in the U.S.

So what's the role of the pharmacist in that challenge?

MAINE:
Great, quite a few thoughts. First of all, to clarify that public health content is a required part of the doctor pharmacy curriculum and it has been for quite a number of years. How that manifests itself is probably not as standardized, but I can promise you that in the context of infectious disease everyone who graduates the 14,000 young people who graduate annually.

And I would love to have you send a few more of those right people of your teaching, (Inaudible) would include the expectation, especially those people who are going, but not exclusively to hospital and health system space. I think maybe one of the problems that we confront is that we think about it a little bit more proactively in that space if we have stronger drugs et cetera.

But the broadest use and misuse of antibiotics is probably in the outpatient setting where, you know, 'I cough, I'm sorry,' and I expect to get an antibiotic to take care that cough when it's not real. It is -- in its presence in our continuing education we need to do more. And we need to replicate the experience that we have seen with the help of CDC and others, of bringing pharmacists into the immunization space.

Because that is a real public health success story. Twenty years ago, there were probably a couple dozen pharmacists who actually were actively involved in the administration of if of immunizations. Today, that number is over 200,000, and every student who graduates is prepared to administer vaccines.

And with the access that is expressed with the number of times Americans enter an environment where there is a pharmacy. I heard last week, five or six times a week going into a grocery store, and you don't see many grocery stores today that don't have a pharmacy in them with overhead pages reminding people that they need to have their flu shot or whatever.

So, I think there are lessons we can learn in that that would really be applicable to other public health challenges.

YASMIN:
Yes, I also want to ask Anne about immunizations and vaccine-preventable diseases. Before I do that though, Lucinda, you want me to send more students your way but it was reported recently that we have too many pharmacists. Is that true?

MAINE:
It's not true. I reported that.

(CROSSTALK)

YASMIN:

You heard it here first.

MAINE:
No, we actually and we actually monitor that on a monthly basis using data from employers. And while the recession did bring the national shortage which was extraordinarily well-documented and very detrimental to public health about 10 years ago, 10 or 15 years ago. We're now a little bit closer to parity in terms of having a sufficient number of pharmacists to keep pharmacies open. Because they can't stay open if they don't have one of those licensed professionals ready to go.

And so, and I predict that there will be another shortage as soon as we line up the incentives and the value-based healthcare system that is emerging so that we can, for instance, facilitate access in physician's offices and clinics to a pharmacists who could help with drug selection.

And some of the issues that stand in the way of addressing antimicrobial resistance. So, it's a complicated picture.

SCHUCHAT:
Yes. You know, I just want to get into the antimicrobial resistance before we lose that theme. And when we talk about these superbugs they are the nightmare scenario, there are more of them, there the, you know, the end of antibiotics is a pretty terrifying prospect.

But I think that we've thought about this issue, although the bugs are not respecting any of the silos. We've thought about antimicrobial resistance with a whole lot of silos. So, sometimes we're focusing on the consumers and reducing demands, sometimes we're focusing on the providers and try to improve stewardship. Or in a good case, we have an institution that has really good stewardship and monitoring and practices.

But with our advanced molecular detection and our tools we're actually realizing that these bugs are spreading all over the place. And the impact of having a strong institutional program and policy is nothing compared to having a community-wide one. Because the, you know, the Carbapenem-resistant enterobacteriaceae, CRE bug that's so scary is going to outpatient/inpatient homes. You really need to get your network working together towards a goal.

And so, it really gets to the power of coalitions and the power of focusing on an issue and taking the strategic approach. So, I think this, you know, in this community people have been worrying about superbugs for decades and the end of the drug pipeline is at focus. But we actually need community engagement and stewardship across these institutions to tackle this.

YASMIN:
Richard, did you have and comments about that?

KRONICK:
We have been working with CDC and throughout the federal government with private sector on this problem. We have worked with the CDC particularly on MRSA and made, you know, progress. But, to Anne's point, progress in hospitals. And we are working now with other -- with nursing homes and laboratory facilities and working on stewardship programs.

You know, to Reed's earlier point, we, in 2015, double the investment at AHRQ in antimicrobial resistant bacteria to $6 million. And, you know, we're making some progress. But the challenges that have been discussed are growing, you know, really large.

YASMIN:
So, it sounds like within public health we're operating within this really artificial silos. And yet, you mentioned, Anne, that we, as humans are really interconnected. And so, that relates directly to the huge measles outbreak that started earlier this year. It was interesting to me because now I live in Texas of people often talk about immigrants bringing diseases into the U.S., and yet, this was an example of an outbreak that the U.S. spread to Mexico and to Canada.

And when I was at the CDC whooping cough was the bane of my life. It was one outbreak off to the other and then, a child died of whooping cough in Arizona. It was absolutely tragic.

How do we or can we ever undo the damage done by the Andrew Wakefields and the Jahi McMaths?

SCHUCHAT:
This was an...

(CROSSTALK)

YASMIN:
I'll point fingers. I'm happy.

SCHUCHAT:
2015 was an extraordinary year because the public really rose -- raise their voice about vaccines and vaccine-preventable diseases and the value of immunizing children. Because we have heard voices that are not necessarily that representative being covered by the media and getting a whole lot of play and being very well-organized.

But I think this year, the average parent spoke out and some people whose kids couldn't be vaccinated because of leukemia or because of, you know, tragic conditions. Or, because they were under 12 months and routine and measles vaccination starts then where innocent bystanders in the outbreak.

So, I think it was an extraordinary year, and we've seen many polls and national surveys that have suggested the American public is moving in a different direction now. But vaccines are just an extraordinary success story. And in terms of value, the past 20 years of vaccinating children in America has prevented over 300 million illnesses and saved three quarters of a million lives that would have been lost to vaccine-preventable diseases while saving $1.4 trillion.

Vaccines today cost a whole lot more than they cost 20 years ago. There's a stronger vaccine market than there was 20 years ago. Companies are staying in the business because they are able to get a profit out of this. But we can't take it for granted because public demand for vaccines needs to stay out there.

So, I think this was a really exciting year in terms of the public dialogue. About the immigrant it's really embarrassing that the Disneyland outbreak spread to Mexico and Canada. But what's even more embarrassing

that their outbreak was bigger than what we had because the virus had a community that was under immunized.

So, I'm really pleased that our outbreak wasn't bigger than it was because even though the virus did spread to six other states we have very high immunization coverage. Nobody wouldn't it based on how you read -- you hear about things in the media you think nobody's vaccinating their kids. But less than 1 percent of children have had no vaccines at all, almost everybody. I mean, is there anything else that every single family in the country except for 1 percent is part of.

So I think it is a success to build on for other health and prevention issues.

TUCKSON:
I think this opens up the door to actually a bigger issue. And that is how well, or in this case, I think how poorly do we understand how American adults think about health. There is, you know, I've always -- I come out of the traditional respect and theology of the sanctity of the patient-physician relationship. It's very important. It's a cornerstone of Western civilization.

I used to run that division for the AMA, so I really do I'm grounded in it. I also know the dirty little secret, which is docs are not really that great at talking to patients about very complex things especially those things that relate to their behavior. And so, we are in a new era where there is a pressing need for knowledge around the pedagogy of adult learning theories.

And I think we're starting to see as healthcare is invaded by consumer companies who are digital companies and other kinds of consumer-oriented companies who are bringing this consumer mentality into healthcare. We, I think are going to benefit very greatly because -- but I think it comes back to, again, the research protocols. What do we know about how people think and how you influence them, how do you turn them on, and how do you activate.

Little things like health belief models. How important -- how much do you think it is that you will get an illness? How competent do you feel that you can do something about it. If you do -- if you do these things will affect the -- I mean, there's a whole lot of learning there.

The clinical and the public health disciplines do not understand that adult learning theory very well. And they're going to have to learn more. And I think again, this is a very further area of research, and then very much needed to disseminate it well into the delivery system in the prevention system.

NEWHOUSE:
Well, if I could just comment I would say that that's, I mean, that is in a nutshell what patient engagement is all about. That's what they're begging for is to be listened to and to be a part of the decision-making that goes on. And with great respect to the physician community and I appreciate my relationship with the physician, what I don't appreciate is the physician talking to me and I can tell you people with ALS don't appreciate that they want to be talked with and about, so that they can make decisions together.

And I think that -- I think that it's going to take some more continued education on the part of the students who are going -- who want to become doctors to understand that they get up in the morning, they put their pants on just like everybody else does and there needs to be a conversation not a sermon.

KRONICK:

I think this highlights a need throughout the healthcare system. But particularly, highlighted by this conversation around engagement. So, you know, the problem is clear, the solution over or less so. And I think as in many areas a solution probably needs to involve at some level incentives for both clinicians and patients.

The role of what kind of incentives, how much with financial not so clear, but somehow, there needs to make sense for people to do it. Probably the biggest need is how much you need. You know, if you are a clinician or a patient, how do you actually engage in ways that work.

And again, that is the kind of work that at AHRQ we spend a lot of energy funding in trying to develop that the how-to. And then the third piece of this information and feedback and measurement and how would you actually know if this is working well or not.

And you know, 20 years ago, we developed CAPS, the Consumer Assessment of Health Plans and Providers that has a little bit of some information about patient engagement and shared decision-making not nearly sufficient to get where we want. But again, you know, more needed in all three of these areas.

TUCKSON:
Can I just quickly feedback on this because I had in my mind just for this panel these three areas of priority research elements now get a couple of them. But this idea of measurement is I think essential, especially measurement in a value-based reimbursement world.

We are pushing the delivery system now towards ACO's patient centered medical home responsibility for the total outcomes of care for an enrolled population of people. And when you make your money of course in that world, especially if we move more towards capitated models being inside of that is to not have people be sick. Or to turn the spigot off as soon as you discover that there is a drip.

This inevitably means that the clinical delivery system understanding at some point that much of the pathology or the accelerators for illness lie outside of the therapeutic arena. They're in the community. They're in the population. They are these other forces social determinants disease physical environment, and so forth.

Therefore, it would seem that the delivery system at this moment which is heavily evaluated on measures is screaming out to the public health people, hey, where are my deputies, where my partners, whose going to go into the public housing development and clean the air conditioning docs and the heating docs and get the rugs with the mold and mildew so that asthmatic child does not come running through my office that needs to go to emergency room and being that being on my reimbursement formula.

So, I need the public health department. Public health has very few real measures and certainly few measures that align specifically with the needs of the patient and the delivery system to turn the spigot off. And so, when another, so I think a key area for our research is going to need to be supporting the needs of public health measures that lined up sweetly with resistive measures. Asked though, is doing a good job of trying to push this agenda and I think we have a little momentum through ask though to start to punch this. But the research community has not grab this nearly as intensely as it should.

SCHUCHAT:
I just like to say that to second the idea that health indicators are metrics that are crucial for our progress. And the public health community has done this well on some areas and we definitely need to up our game. (Inaudible) The organization the worst resurgence of measles in the U.S. happened when we had stopped measuring coverage because of budget issues.

We had a couple years where we didn't measure immunization coverage, and then we have 55,000 cases of measles over a hundred children in America died in 1989 to '91, not that long ago because we let -- we didn't

our metrics. We have very good metrics now for immunization where we do have to measure the prevention. We can't just measure the diseases because we will be surprised in bad ways.

But I think the efforts to figure out what's a healthy community, what are the simple ways to reproducibly measure that. We're doing the same thing globally with the International Housing Relation then the global security agenda trying to figure out how do we know that our countries are prepared. That they're not going to have a horrible problem that affects them and also affects other countries.

So, I think the measurement is critical and then it drives improvement.

TUCKSON:
By the way, the IOM just put out a very important work on this topic which I think forms a very fertile basis for research grants to take that to the next level, So we don't have to start from scratch just fill of the IOM report.

YASMIN:
Thank you all so much. I think it's time for our Q&A now. So, there will be microphone circulating I believe.

(UNKNOWN) – Q&A
Thank you. Great discussion. And help us -- I work at ETNO. There were several references to determinants of health and social determinants of health in particular. So, I'm curious, you know, we've known now since at least 1993, that there five determinants of health and I say this is a physician that slug is to practice a little bit.

But healthcare has actually the smallest impact on overall health status. Social determinants -- if you believe the data, has the largest and there are few other very important issues such as socioeconomic status and all of you the list as well as I do. Why have we spend so little funding research in this area?

Because I think if we can't really get our own around that question how do we really make the investments we need to make as a scientific community and at medical community to really -- and a public health community, excuse me, to have the impact that we need to have to get to a much more holistic approach, to improving health and health status, and to create more healthy days for people in this country and around the world.

TUCKSON:
Part of it is and it's a very important question and it's hard to have this conversation among multiple stakeholders in the room. Because you can sound very much like you're pitting one side against the other. But, you know, the public health stuff and all the social determinants, that's soft science, you know.

That doesn't have the credibility inside of the academy as the super specialists with the stethoscope dripping out of his or her pocket, you know, with the tyranny over the way in which the academy works. So this stuff is always been marginalized, it's always important to decide and the juice and the energy has always been around the technology and the machines that beep the night and that's the American culture.

And it's extremely frustrating. I think that the only ray of light that I see on the horizon is they don't have no more money.

KRONICK:

Sure. And I would add that I'm not sure how the problem so much not having the research as to it seems as earlier question not having the political will to make changes. I mean, as you point out, we have very good information about the effects of poverty on health.

But, you know, the research that we funded at AHRQ, I mean, the eye on everything is how is it going to be useful and actionable. And so, and we have funded research on social determinants. But the, you know, question of how does that -- how is that results of that going to be used in declaring action is I think the tough question.

KRONICK:
We're spending, you know, $3.2 trillion in the medical care system through Medicare or Medicaid, private insurance, you know, arguably, help could be overall -- health could be improve with some different distribution of how that spent but how do you get there, and what sort of research if any it would be needed to get there.

(UNKNOWN) – Q&A
Yes. First of all, can you hear me? First of all, thank you for mentioning chronic diseases as being the critical issue especially multiple chronic diseases. The thing that I find interesting is that the World Health Organization is coming out first ever chronic disease older adult recommendations for the world.

And they talked about the factors associated with immutable risk factors around the world and tailoring to those countries. Similarly, Pan-American Health organization has done the same thing and it's amazing how public health oriented both those documents are. The Apollo 1, the WHO will come out in October.

It's that what are interesting that in the context of this CDC has cut quite a bit of funding in the field of aging (Inaudible) and it's wondering why are we going backwards in many respects in some of this. But I also want to make one more important comment. There is a considerable amount of literature on the behavioral, motivational, and psychosocial factors associated with older adults in chronic disease management. That hasn't been adapted to many of the kinds of things that are being done here.

YASMIN:
Anne, did you want to comment?

SCHUCHAT:
You know, I think we have difficult choices in what is funded within the CDC's budget and try to use, you know, the scarce resources as effectively as possible we're obviously, working at a population level and working very much with state and local health departments and what they can do in their communities.

So, I think the issue of chronic diseases is very high on our radar. And the issue of it globally I think with the limited resources we have to address it globally, we do what we can. One of our priorities is actually to help develop national public health institutes in other countries similarly to a CDC kind of event where they can help prioritize what their issues are.

More and more of our international colleagues who we know from the infectious disease world say, what our population dying from now or the chronic diseases. And we need to get our arms around them. So, we don't have very much to do in terms of resources to work on the non-communicable disease agenda internationally.

But one of the areas that we try to make progress that needs to help countries have that ability to prioritize and analyze and invest in their biggest problems.

MAINE:
One of the things that I don't think has come into this conversation today, but I've heard frequently especially in advocating for increased appending on prevention and prevention research. Is this -- and I would like to call in this, I'm not sure that everyone would agree with me that prevention doesn't save money.

And that the research, I think is a byproduct of a research model that's broken because prevention does save money in the longer-term. But that's not typically as easy to research one and it doesn't go into some of the production models of the health enterprise that has all those resources and has invested some of them in curative questions versus preventative questions.

TUCKSON:
I would also hope and I mentioned earlier that goes back to your question as well. The CDC's guide to community preventative services wouldn't it be fun for everyone in this room to know where did the funding run out this year on answering critical questions.

What was the bolus of an addressed issues that tell you whether or not a particular community population prevention efforts works or doesn't work in the cost-effectiveness of it. That agenda of what didn't get funded is huge and they -- nobody pissed off.

YASMIN:
Anne, you want to comment. Now, we'll take another question.

SCHUCHAT:
Yes. I think that, you know, in the vaccine world we're one of those interventions that actually does save money. But, you know, we don't expect treatments to save money. We want to get value. We want to, you know, get treated if we're ill. But I think the issue of advocacy for prevention is challenging. Because before you get sick you're not really that pissed off.

TUCKSON:
And so, what happens is America the news media -- the news media loves to report Americans are living longer. End of story. No. What they don't say we are living longer sicker. So, it's like, wait a minute, we're going -- we're getting sicker upstream and we're carrying all this bolus of medical disease with us that get to manage every month, every other week with a specialist visit. And some of us -- so there is this bolus.

But if they, hey, we've won the war, heart diseases down. Yes. But the incidence of cardiovascular disease is up.

YASMIN:
And now we're swearing is a very respectable event. You have a question?

TUCKSON:
Yes, also.

(CROSSTALK)

YASMIN
No, it doesn't...

(UNKNOWN)
I don't think it's fun.

(UNKNOWN) – Q&A
It's not working. Oh, OK. It's a very interesting conversation, but the fundamental question in public health is where do the dollars actually come from. And if you look at -- I met former health officer running the Medicaid Program. The ability at a state level to actually redesign and put money into prevention is thwarted at the federal level.

CMS will not allow 1115 waivers very easily to actually do the things that actually could create prevention. So, the fundamental question is we saw the rate of diabetes go up in children from the first case diagnosed in 1991 in San Antonio. Now, 70 percent of the kids in San Antonio are type II diabetes.

We've seen the rate of asthma go up and inner cities among African-Americans of the last 10 years has doubled. So, lots of people are making a great amount of money on all these increasing rates of disease. The fundamental question then is, why are we not bringing in not just CDC in our health? Why not CMS? Why is CMS not part of -- because they're financing all of this and we need -- what is -- what can we do to get CMS more engaged in this discussion? Because ultimately, they are spending the most of the money, and they have the greatest vested interest in creating a shift in this regard.

KRONICK:
CMS is involved. The Innovation Center has demonstrations around community health -- you know, their new, whether they're going to be successful or not, I don't know, enough to -- I've been involved in the team and I'm not going to speak to that. But, you know, Reed mentioned earlier I think the best hope here. I mean, the decline in public health funding is the share of national health spending that has been going on for centuries. You know, 1900 much more of what we spent was in public health. And that's been a 100- year, 110- , 115- year decline now.

I think that the emphasis on accountable care organizations on you know same to groups that they're responsible for the health of a defined population is the best hope. And we're seeing some movement, but still certainly early on towards that waiting to the holy grail of how do you make public health much more important for what we're doing, rather than focusing, as the House said 3.2 trillion only on medical care.

YASMIN
Seventy percent in San Antonio have type 2 diabetes -- wow, shocking. On that note, that very uncheerful note, I'm sorry, we have to end. We're out of time. But thank you so much for sharing your time and your expertise.

(APPLAUSE)

WOOLLEY:
OK. We are about to start. If you would please take your seats, one more terrific, terrific panel discussion here.

OK. We're going to begin our third panel now and as our moderator today, we have Frank Sesno. Frank is the Director of the School of Media and Public Affairs at the George Washington University. He is an Emmy Award-winning journalist and the creator of planetforward.org, a user-driven web and television project that highlights innovations in sustainability. Frank's career includes 21 years at CNN where he served as White House correspondent anchor and Washington Bureau Chief. And you can read more about Frank and the bios of all of our panelists in your program.

Our panelists today are Marc Boutin, the Chief Executive Officer of the National Health Council. Dr. William Hait, Global Head of Janssen Research and Development and a Research America Board Member. Dr. Stephen Ostroff, the Acting Commissioner of the Food and Drug Administration. Amy Comstock Rick, President and CEO of the Food and Drug Law Institute and a Research!America Board Member. And Dr. Larry Shapiro, the Executive Vice Chancellor for Medical Affairs and the Dean of the School of Medicine at Washington University in St. Louis, and another Research!America Board Member. Welcome, everybody.

Frank.

SESNO:
Thank you, (Mary). It is great to be here and great to be with Research!America. I have always enjoyed our work together. I'm looking forward to this conversation very much because this is sort of one of the hot buttons of the 21st century, right? How quickly can we make technology data, science, and approvals work to suit our attention spans? Back in July, periodically I sit in for Diane Rehm on national radio, and we did a program on this very topic, the 21st Century Cures Act. And as you can imagine, the phone calls from the audience were animated because people feel very strongly about this. That either their health is being utterly compromised by Big Pharma or it is being held up by big government. And finding that happy space in between isn't easy.

So let me start by asking you and Larry, I will start with you. At a time when science is moving faster than ever, when data is bigger than ever, when social media are more social than ever, do we have a 21st Century FDA or 20th Century FDA? What are the priorities...

(LAUGHTER)

SESNO:
You'll notice where I started by the way, I did get approval for this before -- I got FDA approval before I got this.

(LAUGHTER)

SHAPIRO:
Well, I would say that first off, the FDA is in many ways one of the most poorly understood public agencies and maligned. And I think we can't overlook the role that they have played in keeping us safe. If you look back over the last many years that new device and new drug introductions, the number of adverse and outcomes

have really been relatively minimal. So I think as we discussed what we need to do going forward, we shouldn't neglect acknowledging what has taken place.

SESNO:
What are the priorities as far as you're concerned going forward?

SHAPIRO:
Well, I think modernizing the way clinical trials are undertaken, so that we have smart clinical trials that make use of genomic and other information in stratifying patient, selecting patients, so that we can have surrogate endpoints that are meaningful. And we can reach decisions faster, and I think there is a lot in regulatory science now that will allow us to move forward. But it's a partnership issue. It's not just the FDA that has to be responsive. I think industry, academia, and so forth need to be responsive as well.

SESNO:
Amy, what's your answer to that?

RICK:
I want to start by picking up on your FDA approval, I think regulatory science right now is often viewed in the context of 'the pivotal moment is approval.' That's where FDA's powers are coming from. But to look at it from the perspective of the outside pressures FDA is under right now, more and more were looking at approvals going through expedited review, which one could say may therefore be based on less data. And I come with this from a legal perspective. We're also in a place right now where there are some recent court decisions that are allowing seemingly going (this still) early going in the direction where industry can actually share communications if it's truthful about research it has done for (unauthorized) use. So we're in a situation right now if that continues -- that trend continues, industry may be able to communicate about off-label use. Does that diminish the value of FDA approval in fact because if you can get a drug approved on a very narrow indication that then communicate more on areas of potential off-label use, if it's truthful and this is all very early, does this make FDA approval the moments that we're looking at or are we actually looking at a place where the FDA needs to shift to an agency -- to a legal issue, Congress will have to be involved where safety and efficacy for real-world use is very much a part of the regulatory science conversation. And just to add one more that, with big data and all these data sets we're looking at, patients and clinicians are looking for real-world data on how drugs are actually being used, not just with what they're approved for. So should the role of the FDA be shifting for much more about continuum in the public health conversation? (CROSSTALK)

SESNO:
I'm very positive that anybody -- everybody in the room understands what you're talking about, with real-world data. But just to be sure, would you... (CROSSTALK)

RICK:
Sure. And actually, we may have many definitions in the room about what I'm talking about...

SESNO:

Since our real-world is all different.

RICK:
Exactly.

(LAUGHTER)

RICK:
What I'm talking about is where we can look where clinician can access data, if we ever can get datasets talk, to be able to talk to each other to find out what the actual experience of taking the therapy is with comorbidities, and more than one drug -- that kind of thing, of who actually succeeds who has unfortunate events and that kind of thing.

SESNO:
Since that you're acting...

SESNO:
Commissioner, I will give you the option of going next or going last...

(CROSSTALK)

OSTROFF:
Again, the alternative is to see what everybody else is saying, but you know, I think your question is really a good on and I'll answer that by saying that I tend to be a data-driven person. For those who don't know before I stepped into the acting commissioner's role, I was in the roll of the chief scientist for the agency. So I like data. And what I would say is that you know, today's FDA is a considerably different organization than it was in 2000 or even back in the 1990s. And I think most of the industry knows that and if you look at the more recent track record of the agency, the number of new drug approvals that we had in 2014 was sort of at a record level, not only in terms of the absolute numbers. I think the number we used is 51, between the drugs and biologics, but also the speed of those approvals is considerably faster than it was previously. And so I think the numbers speak for themselves.

You know, there was an article recently in Forbes that indicated that you know when you look at the more recent data, our approval rating on applications that come into the agency is somewhere in the range of I think they quoted 89 percent, that's their data, and not my data. And they were raising the question, have we gone too far in one direction? I think that there are a lot of reasons that the number is as high as it is and it's because of some of the pathways that Amy was talking about where we have a lot of dialogue. We have a lot of discussion about the design of not only the studies, but also the design of the application before it even gets into the agency, which I think accounts for some of the increased speed, as well as the effectiveness of the approval process. And so, I do think that we have a pretty good story to tell here.

We use a lot of adaptive designs and industry uses now a lot of the adaptive designs that come into the agency, and play a very critical role in terms of getting to yes. And so, I do think that that the agency has definitely adapted and...

(CROSSTALK)

SESNO:
Presumably, that's right. Presumably you wouldn’t say the job is done.

(CROSSTALK)

OSTROFF:
No, the job isn’t done, because you know, we live in an era where that piece of innovation is really astronomical.

SESNO:
So what are the priorities as far as you're concerned for the FDA of the future moving forward?

OSTROFF:
Well, I think that one of the things that's critical and again, looking at my role from the regulatory science perspective, is that is really important for us to keep up with the pace of change, the pace of innovation, and make sure that we have the skill-sets that we need to be able to properly evaluate what comes before the agency. That is not an easy task.

SESNO:
We will come back and drive into that some more, but I want to let Marc jump in here. Marc.

BOUTIN:
So I represent 133 million people with chronic disease, and disability. Most of those people have more than one chronic condition. If you think about this question from their perspective, the answer is absolutely no. There's no question about it. For somebody living and dying with one or more chronic conditions, we cannot do this fast enough. I also want to be very clear that we've worked very closely with the FDA over the last 10, 12 years. And we have seen tremendous improvement. We have seen tremendous work accomplished there. I think there's still a lot of opportunity to move forward. But from a patient perspective, we're not there yet.

We may not get there for quite some time, but we're in an environment where we built the system that is constrained by all the stakeholders. And this is an area of intense gray. We keep thinking of it as black and white. Are we getting to the approval stages fast enough? Are we getting products into the hands of people who are dying as a result of their condition willing to take substantial risk? And are we getting products into the hands of people that may be misused and caused harm? It is not black and white. It's intensely gray. And the answer is not the same for every part that those through the FDA.

So we have to look at how we become much more nuanced, how we include technology at the front end and backend at FDA, in very different, very innovative ways. But I'm pleased to say that in our work with the FDA, we have seen a great deal of movement in the last few years.

SESNO:
You have the opportunity -- you are sitting next to the acting commissioner now. So feel free to go ahead and suggest to him or three things that would make this happen.

OSTROFF:
I am always open to suggestions.

BOUTIN:
So I have a fantastic board member who said to me that everybody can walk up to you as the CEO of the organization, they are going to say I don't want to tell you how to do your job, but everything before but was irrelevant. And not to listen to what he says after ‘but.’ Let me say this...

SESNO:
But.

(LAUGHTER)

BOUTIN:
From the patient perspective, we have looked at the FDA and everybody talks about what is called the gold standard. And that is the clinical trial process. Gold standard. You know what? When we say something is the best, it makes it really hard to innovate. And there is actually a really good research on that. When you determine something is the best, well you don’t want to change it because you're already the best. Well, in this day and age, with the way technology has evolved, the way information evolved, the way healthcare systems are evolving, we need to be able to include information at the front-end. And that's very different. I'm pleased to say that FDA has been very responsive in patient-focused drug development. There's a huge opportunity to ensure that the patient perspective is included in drug development at the front-end. And we have the opportunity to answer questions that are important to patients.

With the 20 meetings that FDA has been conducting at every single meeting, folks from the FDA have walked away with a thought they understood what was important to the patient, and that information had been given to them through surrogates, doctors, researchers, others. And in every instance, they were wrong. And that has created this cultural (aha) moment that we have to figure out how to get that information at the front-end, and use that to develop programs to make much higher value products to make decisions about benefit-risk.

SESNO:
So are you saying not the gold standard -- another standard?

BOUTIN:
I'm saying let's not say it is the best. Let's keep evolving and improving this.

SESNO:
I will come to you in a minute, but I do want to hear Stephen's response to that.

OSTROFF:

Well, I think Marc is absolutely right. You know, we have been listening quite intently to patients and in both the PDUFA, I hate to use sort of the anachronisms, but the user fee for prescription drugs for PDUFA-5, patient-focused drug development was a very important component. And that's where those 20 listening sessions arose from. And I can assure you that it is also going to be critical to the discussions that are ongoing with the number six, which were currently in the process of now. Marc was at our original meeting in and so, we want to be able to continuously change and adapt to environments, where we can receive information from all of the various stakeholders, in order to be able to come to the appropriate decisions. And were certainly also very appreciative of the fact that the benefit-risk ratio is not a static figure. In fact, you know one of the challenges is that right now it's very largely a qualitative concept in terms of trying to balance benefit versus risk, but we recognize that it can be a very sliding scale, depending on the seriousness of the disease itself that you're approaching. And that patients are very often willing to take additional risk in order to have things that may be lifesaving to them or that may be extremely beneficial to them. And we are working quite hard in terms of being able to conduct a lot of the formulative work, to be able to make that more quantitative in terms of how we assess and how we utilize...

SESNO:
You said earlier at the onset...

OSTROFF:
The patient input into the decisions.

SESNO:
You said earlier though that you're a data guy.

OSTROFF:
Yes, I am a data guy.

(CROSSTALK)

SESNO:
So is this changing the way you consider data or the type of data that you're going to use because it's coming from...

OSTROFF:
Oh, absolutely. I don't think there's any question about.

SESNO:
Let me come down -- sorry, that...

(CROSSTALK)

SESNO:
You can have the ultimate overview here.

HAIT:
Right. And of course, I have the perspective of the pharmaceutical industry of interacting with the FDA in a regular basis, with our products, and also as a member in the FDA science board. And following up with what you said, what's absolutely critical to keep in front of our minds is the responsibility of getting the balance between safety and efficacy right. And what we need to do as a community is to provide the FDA with the data and the tools, no matter what format or what form that may take as that evolves with time. So they can make these very, very -- sometimes very, very difficult decisions. Sometimes when a drug is incredibly active, and incredibly safe, no-brainer. When a drug is not so active and it has all sort of toxins, no-brainer. Many of them fall into the gray zone, and it takes enormous judgment in my experience now working with inside the FDA, both -- both roles, particularly with the FDA science board and getting to know the people, these are remarkably dedicated people who take their responsibility, the public good, as public servants, incredibly seriously. We should be very proud of these people because I'm telling you they're working hard and they are not making a fortune doing it, believe me.

SESNO:
We're in a town where Congress gets to weigh in on these things periodically, known for innovation and expediency.

(LAUGHTER)

SESNO:
They have been working on something called the Faster Cures Act, by the time they get to it, might have been the Slower Passage Act, but I want to ask you all about that. And specifically, what is at stake here, there was an interesting piece written -- co-written in the New York Times not long ago, back in June, you may have seen a David Kessler put his name to this, and he wrote the following: In the wake of the innovations of the early (AIDS), there are new drug approvals by the FDA, become the fastest in the world. The agency introduced mechanisms, such as fast track and breakthrough, drug designations that are increasingly being used to speed lifesaving treatments to market.

But he writes, the 21st Century Cures Act could substantially lower the standards for approval of many medical products, potentially placing patients of unnecessary risk of injury or death, while the legislation does not mandate this approach, it opens the door to it. Does it?

OSTROFF:
You know, the original versions of this bill had a number of parts of it that made us somewhat uncomfortable. We were able -- and I think that there was a great deal of opportunity for technical assistance and input as this bill went along on the House side where at the end of the day, I think we were very comfortable with the content of that bill and I will continue to say that we are comfortable with the content of that bill that it does not do things that would significantly cause concerns for us for our ability to do our job effectively, which is to be able to evaluate products for their effectiveness and for their safety.

Having said that, you know our concern continues to be the costs that are associated with doing the things that we are being asked to do in that bill. I've had this conversation with Mary [Woolley] and others over the last

few months that we were certainly happy with the fact that there were funds dedicated for FDA. Our estimation and I think the estimation of the CBO is that the funds that are available are not going to necessarily cover everything that we are being expected to do in that bill. And so, then the question is if we end up having to do that of course we don't know what's going to come out on the Senate side and what will ultimately come out in the final version of this, then you know those resources have to come from somewhere.

And as I pointed out earlier, there are a number of things that I think that we can point to that we're doing extremely well. And we don't want to do anything to jeopardize the successes that we had. And so our focus right now isn't so much on what the content is, but whether or not we are going to be able to accomplish everything that we are being asked to do in that bill.

SESNO:
Let me ask Larry. You saw the content, how many things (inaudible) raises for example, he said the current version of the bill, a lot of consideration of drug approvals based on clinical experience. He is concerned that provisions could mandate use of biomarkers to approve a wider variety of drugs. Are you concerned about the speed versus safety equation here?

SHAPIRO:
Well, I think we have to be concerned about it, but speed is really something when we are dealing with chronic diseases, when we're dealing with the expectations of the public, and so forth. And we just have to take advantage of all the technology that we have available to us. If you're trying to develop a new therapeutic for Alzheimer's disease, you know, waiting 5 to 10 years to see whether there is efficacy associated with the new drug, it just doesn't cut it. And so, we have to make use of biomarkers and other tools to really design trials that will get us there faster. Speed is important. Lives are at stake, several people have said that. So I think it is a balance, but there are things that we could do to make things go faster.

RICK:
For 21st Century Cures -- there are certainly a lot in there. I understand exactly what Steve was saying that its resources question, but I would as I did before broaden the question a little bit. Resources is not just an issue for what would be statutorily mandated, but with the emphasis there are some of it in 21st Century Cures, but with some of the expedited review the emphasis we have on post-market data gathering.

I don't know that we have enough resources every time there's a signal or a concern coming out of post-market data. We have the resources to do the follow-up research that's needed, and if we don't have enough resources when you do you have to prioritize in terms of what you look and what you want really just withdrawn or you actually do follow-up research. How we're going to make those decisions about what do follow-up on, what we devote resources to, and what we don't.

And I don't know that that legislation has addressed that question. I don't really think it has, and I don't really know as a society who knows how we want to follow-up on that prioritizing in a time of limited resources.

OSTROFF:
Let me just make -- again, getting back to data because we've looked at some data and you look at -- you know it seems like ancient history already but if you look at the period 2010 to 2012, there were a total of 94 drug approvals during that period. And when you actually look at the information that went into those approvals, almost half of them use surrogate endpoints, and in many if not most of those instances of surrogate endpoints used biomarkers.

And so this isn't something new and different for us. The challenge is that the evidence of the clinical validity of the biomarker needs to be relatively solid, it needs to be demonstrated in part of the challenge, and I know from the standpoint of an organization like Research America a big part of the challenge is to be able to do a lot of the basic research that needs to be done to be able to say what are effective biomarkers for things like Alzheimer's.

And for many diseases, we just know the information isn't there yet to be able to say that this is an adequate biomarker for that particular disease. And that's one of the big challenges, and so you know that has to be as much of a focus of this issue of being able to qualify biomarkers once this has been established.

HAIT:
I want to paraphrase Albert Einstein in this particular issue. He said something like we should do it as fast as possible, no, faster. And I think the reason why this is important to keep in mind because when you do an experiment whether it's a new drug, new device whatever, there are actually three possible outcomes. It could be better, it could be no better, or it could be a hell of a lot worse.

Although we think we know where we're going in especially to these very early trials where phase I extensions can lead to an approval, we think we know until we actually have the data, and all three possibilities are very real. So I think some caution is always going to be necessary.

(CROSSTALK)

RICK:
I want to ask if I could ask Steve a question.

SESNO:
Please.

RICK:
Do you think with the provisions in 21st Century Cures that put a strong emphasis on biomarkers that will actually change their behavior if it is approved?

OSTROFF:
Well, again you know from our perspective, we you look at things through the lens of effectiveness, and we look at things through the lens of safety. And I don't think that we want to veer away from that because it has served us incredibly well for a very long period of time. And so to the extent that biomarkers can assist in rendering the appropriate decision regarding the efficacy and the safety of the product, they will be valuable.

SESNO:
We're going to have a few minutes for audience questions. Before we do that I want to touch on a couple of things, but before we move away from the hill right, now it's the Senate’s turn, you're saying before that's going to start next month sometime.

OSTROFF:
It's been going on now -- some months, we're likely to see a version of -- output sometime next month at least that's...

(CROSSTALK)

SESNO:
To all of you, anyone who wants -- as this conversation moves forward up on the Hill, it's going to affect your life and all of our lives as a result. What are the three things -- two or three things that you are most concerned about that your hearing, and that you think and feel most strongly need to be got right in this process? They're all listening to you now.

SHAPIRO:
I'm sure they are, just like they always do. I should say that we shouldn't overlook some of the really important things that probably everybody in this room agrees upon, more funding for the NIH, it is really critical that we keep the stream of basic research going, also more focus on the groups that are not currently well represented in clinical trials, minorities women, children etc.

And then decreasing some of the regulatory burden that occurs for scientists as well as on the FDA side and there are provisions in the bill for the house -- the house version of the bill that deal with those things. I would hope they would stay there.

BOUTIN:
So two things I want to put on the table. One is -- to your earlier question, patient preference data is one of the items that have been called into controversy saying somehow that would lower the standard. It's not lowering the standard, it's adding more information, and that is critical because right now when treatments come out they're approved for being safe and efficacious, but it doesn't describe it in the context of the burden of disease, or the burden of treatments that may exist.

And that information if it makes it into label and gets into the healthcare system where you can have a conversation with a provider, allows you to make an informed decision about the trade-offs that meet not only a clinical outcome needs, which are social, economic, personal needs, and the goals that you have, we don't have that ability now. And there are multiple treatments that exist in certain conditions, some of which have trade-offs.

It's very hard to get that information to the patient. That is something that is critical and that helps with compliance, adherence, improves health outcomes, huge opportunities moving forward, so that's number one. That needs to stay in there. It's in the house version, I think we're saying an improved version in the Senate. The second thing that was something was dropped in the house version, and that was a set of incentives to develop treatments for unmet medical needs.

We have not had the conversation as a society about how we incentivize treatments. The patent system works sometimes, but it creates perverse disincentives in many other instances. Dormant Therapies Act which has been introduced in both houses of Congress was meant to be part of 21st Century Cures, again got dropped. We're hopeful it will be part of the Senate.

I'll just give you one example. If you're looking at Alzheimer's, that's a condition if you want to show that you can change the progression of that disease, you need to do a 15-year clinical trial. And that runs concurrently with your 20 years of patent life. We ironically incentivize the development of treatments for conditions that you don't have to show that level of therapies.

HAIT:
To this point this is why -- in the bills it's important to focus on certain points, because this whole issue to me is called 21st Century Cures, but in the bill it is also referring to prevention, but without surrogate endpoints to say we can be reasonably sure that if you lower your LDL cholesterol and you're going to have good cardiovascular outcomes, and other things perhaps an -- Alzheimer's, tau and amyloid-beta peptide -- these things have to have some degree of assurance that these surrogate endpoints are going to be meaningful so you don't have to wait 15 years to see the drug develop.

That's very, very important in encouraging research in that area -- could be tremendously helpful.

RICK:
I couldn't agree more about the need for surrogate endpoints, diseases like Alzheimer's and Parkinson's where -- but I also personally am really excited about the aspects that would focus on the FDA -- using as a phrase I used earlier, more real-world data in its decision-making, how drugs are actually used by people and how they prescribed by clinicians. I think that's really important.

I understand the value and the importance of the scientific rigor in a gold standard clinical trial, but that isn't how the patients in this real world are taking drugs, and I think it would be the extent we can push the FDA to perhaps simultaneously -- I don't know if what I'm saying is possible-- but to be able to focus on real-world use and rigorous science in making its decision, I think that'll be a step forward.

OSTROFF:

Let me just make a couple points. First of all, you know I think the only federal employee up here on the panel, it so difficult for me to talk about pending legislation itself, but what I will say is that there is one provision in the house bill that was passed which for us -- and I think for many that are from industry that are out in the audience they know is very critical for us and that is the hiring provisions that were contained in 21st Century Cures, particularly as it relates to direct hiring authority, which is a little bit of an esoteric issue, but particularly the salaries.

Because we know that in certain very critical areas where we need really highly skilled individuals to be able to appropriately evaluate some of the things that are coming into us, we need really, really good people. And it has been a major challenge for us, to be able to accomplish that. I think the other provision which crosses a very beneficial provision -- and again it's sort of the esoteric of federal government has to do with sequestration as it relates to user fees.

Another thing that's really, really important to us because we know that some of the reasons that we did not meet our goals in the last user fee program is because of the results of sequestration, so these will make a big difference to us and certainly anything that's going on in the Senate's side we would like to see that preserved.

SESNO:
What more do you have to say about the real-world data?

OSTROFF:
Well, real-world data is a very interesting question, and it is something that we increasingly are looking at. But I think that you have to sort of separate the use of real-world data after approval from the use of real-world data for evaluating effectiveness of a particular therapy. We have been doing a lot of work trying to see how we can take better advantage of you know what's going on these days with big data to be able to use it in their pre-market...

SESNO:
What are you doing differently as a result?

OSTROFF:
One of the major things that we're doing with real- world data these days is in the post-market setting that's something called the Sentinel Initiative. And you know that uses amazingly large amounts of data to be able to look for signals, of both safety signals, but as well as whether or not to confirm some of the information that became available to us in the clinical trial setting. And so it's been a remarkable game changer for us.

I can also say in the pre-market circumstances, one of the areas that we see really significant benefit from real-world data is when you have a circumstance where a drug is already been approved. And you know I give an example -- some drugs that's been approved for use in lung cancer, and we know that in real-world settings it is being used to treat some other type of cancer.

There may be a lot of available information to suggest to us that it actually is quite effective in these other circumstances, and that data can be then used to be able to change the labeling for these other indications. I think that's a great example of how we can use type of data.

SESNO:
Let me turn the heat up on you for just a second because so much of the focus here has been on the FDA and how can the FDA move quicker. But there are plenty of people who say that the pharmaceutical industry is all about getting everybody to move quicker so it can make more money and it can bring its products to market, and that the public's interests could be compromised in that process. How hard are you pushing, and what you say to that?

HAIT:
I'll share with you a very interesting experience that we had recently with the FDA with the new breakthrough therapy designations, and we were very fortunate to have some of the earliest breakthrough therapy indications for drugs we were developing. So we didn't completely understand what it meant so we went down and we met with the FDA, and we said to them really, you're going to help us move the process along and you think we should start filing.

We don't think we can manufacture them -- we don't think we have the drugs ready by the timelines you're giving us. It was a big smile on their face, and said what do you mean you can't keep up with the FDA.

(LAUGHTER)

HAIT:
So you know I think the pharmaceutical industry is developing drugs -- developing drugs and being held to a very high standard in terms of data. In academics we used to say the data has to be publication grade, or drug development in the industry it has to be industrial grade because we're held to a standard much higher than almost any journal which is the FDA because the reviews are very rigorous.

So I believe we're moving at a reasonable pace, and we're always trying to do what we believe would be best for the patients.

SESNO:
Would you weigh in on this as the Dean of the School of Medicine here, and thinking also about real-world data, observational data, all the different kinds of data information we're talking about.

SHAPIRO:
Well, I think one of the real challenges we're all struggling with is the rate at which new information is coming online in affecting what and how we might do it. You know the issue from cancer therapy was mentioned a minute ago and so forth. I think the data is going from genomic studies has indicated that cancers are much better classified by the nature of mutations that they are within the tissue in which they originate, and yet you know the trials we do and the patient recruitment is not caught up with that just as yet.

We have the opportunity to use other new information in developing adaptive trials that the FDA has started to move on, but one can now monitor the course of treatment of a cancer patient where there are individual clones of that patient's cancer and some are responding to the therapy and other clones are not, and if you don't know that in real time you can't really develop a trial that will show what drugs are effective and what are efficacious.

So I see the big challenge as how do we keep industry, academia, the FDA all moving all at a pace that keeps up with the pace of the science.

SESNO:
Are we on the right track? Do we have the model for that or does it need to be something even more fundamental discussed here on how different elements aspects of science and data are coming together and being coordinated.

SHAPIRO:
Well, I think we need to be sitting in the same room more often. There's a lot of communication that takes place back and forth in the case of industry and the FDA is very formalized of oftentimes and follows certain prescribed patterns. I think there is a need to have more open and candid conversation so that people can understand the problems that we're all struggling with. You know forums like this help, but I think there needs to be other opportunities to do that too.

HAIT:
I think with another problem depends on how you look at it, but because of the perceived conflict of interest, the advisors that they're allowed to use often aren't the ones who are most familiar with the topic, and it can get

to an extreme where you have a panel who are advising the FDA -- of course they're only advisors, but that I think creates problems that I think would be nice to -- we may have gone too far -- the pendulum swung too far in this real conflict of interest versus perceived conflict of interest because that doesn't help the FDA to not have experts having those discussions with...

SESNO:
Is that a big problem?

OSTROFF:
Well, I'd like to get to a point that was just raised which is I totally agree with you that the more dialogue and the more stakeholders that you can have talking to each other, the better off I think everybody will be, and that includes patients. I just think that that's critical and you know one of the things that we are actively promoting and I will say you know -- and Bill knows this very well, you know we changed a lot as a result of some input that we received from our science board a number of years ago, and we made significant changes particularly as it relates to the area of public- private partnerships.

And we are much more external as an organization then we used to be. I think one of the examples of that is that we have we created something called Centers of Excellence In Regulatory Science, we now have four of these including one that's a consortium between UCSF and Stanford. It's a great way for us to be able to collaborate with academia to do some of the things that we need to do to generate the science that will help us make appropriate decisions, and I think that that you will see -- because we have another follow-up report coming from our science board that's supposed to be coming out within the next week or two that I think will amplify better use of some of these opportunities.

And so I think that we as an organization are always very open to seeing ways that we can partner with a variety of stakeholders, including regulated industry to do things better.

BOUTIN:
I think -- and I'm glad you said patients need to be part of that...

(CROSSTALK)

SESNO:
While Marc's thinking, when he's done we'll come to the mike's and we'll come to your questions, so go ahead.

BOUTIN:
Thank you. Patients have often been excluded and when you bring patient into the mix, and allow them to produce with the conversation the conversation almost always goes in a different direction, and it almost always ends up in a better place. Adaptive clinical trials is a great example of what part of that conversation the clinical trials are going to shift, because quite frankly if they don't you're creating situations where patients are in clinical trials that may or may not work that we could have determined.

That's unethical. That makes no sense. And with patient participation we give you the ability to say no wait, this is wrong we need to do it differently. I use J&J as an example with what they've done with the access on compassionate use that brought in patients that looked at this from the perspective of what is important, how

do we create a system that is fair, is equitable, everybody gets a shot, and the patient has been part of developing that in a very open and transparent way.

That's how we need to look at these issues. We will give you a very, very different perspective and a better outcome.

SESNO:
Ok, let's come to a question on the floor and the microphone is in the back. So who is going to start?

Lucinda Maine (Q&A):
I like all the panelists to reflect on where we are vulnerable in our scientific workforce for the kind of innovation and regulatory science that's coming down the pike.

SHAPIRO:
I will all start off since we are supposed to be responsible for helping to create that workforce. You know stable predictable research funding is critical to get people to go into the kinds of careers that we're talking about here. A day doesn't go by that somebody doesn't come into my office and tell me not only of their great disappointment of not being able to carry out their work and so forth of the fact that to resubmit the same proposal six times in order to get funded, they're leaving their career, they're leaving the field because of their frustration.

So I don't think we'll have the kind of workforce that we need if we don't have a predictable future that people can see as a career path for themselves in this area.

RICK:
I would second that. I sit on the NIH Neurological Institute Council and it's a regular topic of conversation, how the young investigators are leaving -- their emotional state, their depression at the field they've chosen is a common topic, and I think that that is certainly a significant issue, and it pushes a lot of emphasis at NIH to make sure that the funding for basic, basic research remains as strong as it can be in this era of type resources so those grants will come through.

But then I feel attention there between some of the translational and clinical research questions that no single industry player has the incentives to follow up, and so I personally might look to the government for that, but then we also look to the government to maintain the basic research workforce and I just don't think we've hit that right now because we aren't giving enough money to it.

SESNO:
It's a great -- I'm wondering how much -- how much has this changed and over what period of time?

RICK:
I'm going to ask Steve. I don't know.

(CROSSTALK)

SESNO:
Is this always part of the challenge and the background noise or...

(CROSSTALK)

RICK:
It's gone down.

SHAPIRO:
We've lost 28 percent of the purchasing power of the NIH dollar the last decade and the pay lines, the success rate of people applying for grants is just falling through the floor. And it's incredibly discouraging, and -- so yeah, I've been in this business a long time. There have always been ups and downs but nothing like the cycle that we're currently in.

OSTROFF:
You know I'm going to answer that question a little bit differently because I look it from you know FDA as an organization. And one of the things that I think is that many scientists that are out there don't look at FDA as a scientific organization, they look at NIH as doing science. You know we have about 16,000 employees at NIH and 10,000 of them are in scientific categories, and so you know recognizing -- number one that we do science and number two that there are career opportunities at an agency like FDA.

They can be very interesting career opportunities. I think is -- a message I can't say often enough, there are certain categories that we have tremendous difficulty recruiting. One of them is in statistical category, another one is informatics, and part of the informatics is because they're in such great demand these days because of everything going on around precision medicine etcetera. And so those are particular needs of ours, but I -- you know there are -- it is such a broad question.

But I think fundamentally, we need to sort of get the word out that we really need good scientists. And the last thing that I'll say is that we need people who can integrate information from a variety of scientific disciplines. This is no longer a circumstance where you can be really, really good in one area, because for an agency like FDA, we need people who understand some of the basic science around you know genomics and genetics and whole genome sequencing, the people who understand behavioral science.

And for us as an agency, to do our job right we have to be able to integrate all of that.

SESNO:
How about the question from the perspective of the private sector.

HAIT:
What I was going to say -- what was very much what Steve was talking about, the ability of scientists to handle very complex data sets that are coming off of gene sequencing, that are coming off transcriptional profiling, that are coming off a very complex proteomic methodologies that are only getting -- these data sets are only getting more complicated, and the tools to analyze those data sets can be very tricky, and you can apply one to two days and get one answer, you can play another statistical to get a completely different answer.

And you really need very deep expertise, because this is coming forward as part of companion diagnostic testing. So if you want to develop your drug with a companion diagnostic test and you think you got the analysis right, it is very important that the regulators also have the scientific expertise to say yes or no, you got it right. So I think this is going to be an even bigger issue as we move forward with companion diagnostic tests and precision medicine.

SESNO
Question over here.

QUESTION:
And I can echo that sometimes my career outlook feels bleak sometimes, but I wonder what does a private public partnership look like at the local level particularly if you're not part of a -- but we have passion for their eggs and we're interested in regulatory science, what can we do?

OSTROFF:
If that's directed towards me, you know we have -- I think it's a very good question because you know one of the things that I think that we need to do is we need to grow programs that recognize regulatory sciences as a legitimate scientific discipline. And increasingly, we do see a variety of academic settings that are really taking on regulatory science, developing programs in regulatory science, and finding sources of funding to be able to do work related to regulatory science.

I think that that is really quite vital. You know FDA is not primarily a funding agency, and so were not an agency that puts a large amount of resources out there. So we're going to have to do this in partnership with stakeholders who really see the benefit of having good science that can help in decision-making. Once you have the basic science accomplished, once you have done the work to translate that basic science into usable products that there is a lot of science that's necessary to bring those products across the finish line, and so we do have some mechanisms to be able to provide resources not only through our centers of excellence but also through something that we call the Broad Agency Announcement, where we do put out funding for opportunities that come before the agency but we're not going to be able to do this...

HAIT:
Commissioner's fellowship program, that is absolutely -- we just reviewed it...

OSTROFF:
I just spoke at our graduating class yesterday.

HAIT:
Absolutely fantastic opportunity for people who want to get into this and learn how you really get under the hood and learn how this works, phenomenal program.

SESNO:
Getting this? Are you inspired now?

Larry Hausner (Q&A):
I want to get to back to a point that Bill made before that, I don't got answered which was about the scientific -- that the scientists who were on the panel with the conflict of interest parameters that you currently have. Are you -- are you satisfied with who is there, is this causing a problem, and is this something that we need to look at to make changes in the future?

BOUTIN
Yeah -- Bill, do you want to go first?

HAIT:
I have to be very careful because if you get back to these panels it may be screwed.

(LAUGHTER)

SESNO
Pay no attention to that.

HAIT:
But I think the truth is the greater the expertise -- subject matter expertise on the panels the more rigorous and better the reviews. And that's exactly...

OSTROFF:
I have to be very careful in this area because you know we as a as a regulatory agency, given how much we regulate need to be very careful that we that -- that there isn't a perception that the process wasn't as rigorous and free of conflict of interest as possible. Having said that, we are very much aware of this particular problem and are looking at various ways that we could make sure that you know we can get the expertise that we need to be able to...

(CROSSTALK)

QUESTION:
How can you get the...

OSTROFF:
Well, you know again -- you know we're sort of looking at everything including potentially trying to lower some of the burdens that we place on our outside experts in terms of financial disclosure forms and those sorts of things, even when we have people that are inactive we require certain things that people find so burdensome that they -- we had people that have resigned simply because they don't want to keep up with all the paperwork.

So there are ways that we can potentially -- I think simplify the process that would be very beneficial, but I do think that we need to explore ways that we can make sure that we can access the expertise that we need to be able to make the most appropriate decisions without potentially jeopardizing or calling into question any of the decisions that we eventually make. It's a tough balancing act.

BOUTIN:
One of the reasons a patient community took on patient engagements in the context of benefit risk and drug development was up -- we saw the conflict of interest debate shift, and is shifted to a point that patients are finding that they were often conflicted out of advisory committees. It was a huge challenge to participate. And we thought will this is crazy where a non-voting member on an advisory board and we can't even get on because of conflicts. So we started to work with the FDA to develop this schematic where we could get very sophisticated engagement data that can inform decision-making that would be outside of the conflict of interest process.

So I think there are ways to get at this, but we have to be careful because I agree -- the pendulum has swung too far...

RICK:
Can I just follow up? Actually this is not on the ethics point, but the patient engagement effort tends to come in much earlier in the process. The advisory committee tends to be much later and don't always occur so I think it's -- that's a benefit that is far beyond -- voice in the advisory committee.

SESNO
Very first time I got behind the wheel of a car in the United Kingdom, sitting on the wrong side of the car -- road, I drove into a roundabout in the wrong direction -- this seems to me is the roundabout of science and medicine, a great amount of data, very politicized process, gigantic public opinion and public pressure, the private sector and the public sector all coming together, and all of these forces are swirling around and figuring out how you get to the other side when everybody's driving faster and faster all the time it is immensely challenging.

But it is also one of the interesting things and why you must have a career in regulatory science -- going anywhere. I want to thank the panel very much and thank you for your questions and (AUDIO GAP).

MARY WOOLLEY:
Well, thank you. I really like that roundabout analogy. Please, you don't have to stand up. The roundabout analogy really is a good one, and it goes to sitting down together and trying to solve problems, and all the while assuring that those folks that we know are up there in the capital are part of the solution and get engaged, and they're not going to get engaged unless we engage them, including on the campaign trail which is another pitch for joining us on the campaign for the Cures.

But we also want you to stay joined to Research America to keep engaging us, let us know what further questions and comments you had as a result of being part of this conversation today. Be in touch with us, to let us know what your solutions are and new ways that we might be thinking usefully to become the kind of effective advocates that we constantly aspire to. I really take the point about when you define yourself is the best it makes it harder to get better.

And I think that's true for all of us who are involved in the work that we do in different ways to assure medical progress. We know we're doing as well as we can, but we also know we can do better. And it's working together I think that is the key to that. So thank you for being here today, thank you for being part of Research America, and special thanks to our sponsors, our lead sponsor Astellas and all of you who have made this first-rate program possible. Thanks and have a great afternoon.

(APPLAUSE)

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