2015 NATIONAL HEALTH RESEARCH FORUM

STRAIGHT TALK: ADVOCACY FOR A NEW ERA IN SCIENCE

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INTRODUCTORY REMARKS

Jeffrey Bloss, M.D., senior vice president of medical affairs, Astellas Pharma Global Development

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e are all in the midst of a new era in medical science, and in healthcare overall. The changes are fast and furious, catching up or even staying ahead of the curve is clearly our challenge. 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. If we want America to remain the epicenter of 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.”

MODERATOR: Richard Harris, science correspondent, NPR

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teping back and reflecting a bit, 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 have big data, and personalized medicine becoming precision medicine... And people are turning and saying, 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, and I’m wondering if each of you would reflect on what it would take to accelerate that transition from having these fabulous tools to actually putting them to use more rapidly.”

Gary H. Gibbons, M.D., director, National Heart Lung and Blood Institute, National Institutes of Health

“It is an exciting time. I reflect on 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. 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. 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.”

Ron Mobed, chief executive officer, Elsevier

“From an engineering perspective, when I hear the question ‘do you think that things will continue to be very expensive or get cheaper?’ there’s only one answer—of course it’s going to get cheaper. We hear people talk about personalized medicine and 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 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.”

Anil Jina, M.D., senior vice president, head of global medical affairs, Shire

“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... One of the key things that I think that we would need to do to progress this is to break down silos, to enable a lot more partnership. I think whether you’re talking about 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 in partnering. But broad partnership across the board and a coordinated approach to partnership is lacking. If we really want to truly embrace this next level of precision medicine or next level of more personalized medicine, we’re going to have to break down those silos.”

Sudip S. Parikh, Ph.D., vice president and general manager of health & analytics, Battelle

“I think that the goal isn’t just about a target for a cure, it’s about that this 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 the engineering workforce seem to be provided for by NIH, in the same way the molecular biology workforce are being provided for by NIH. These are things that require a framework and require funding. We can make a lot of progress. When those things are not done in a thoughtful way, we have the same organic one-off basis of these collaborations and that’s a real problem.”

Vincent A. Forlenza, chairman, chief executive officer and president, BD

“There is a second piece of this which is: 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 costing up to 50 percent of the healthcare system, there’s a lot of room to redesign the system to create more room for innovation while you pay.”
When we think about ALS now, we think about the viral phenomenon with the ALS ice bucket challenge and [the community] raised over $115 million. And what really sticks in my mind is that there was one day in August last year where [the community] raised about $11.5 million in one day. 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 do you harness that passion to advance the field?”

Barbara Newhouse, president and CEO, The ALS Association

“I think that access to specialty diagnosis is a big issue. In a disease like ALS, for example, [patients] oftentimes are diagnosed or misdiagnosed with so many other things. So that by the time [patients] are given a diagnosis of ALS they are already progressing down the way. And in the neurodegenerative space, I think that happens a lot. I believe that happens a lot because there are so many intersections. And also, I would say 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.”

Reed V. Tuckson, M.D., FACP, managing director, Tuckson Health Connections, LLC

“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. I think related to the interconnectedness around the world is the interconnectedness of the causal factors that either produce health or lead to preventable misery and suffering. 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. It’s the research agenda that looks at the interconnectedness of these issues that produce the preventable chronic illness, which we, as a nation, will not be able to afford.”

Anne Schuchat, M.D., principal deputy director, Centers for Disease Control

“I’d actually like to say our interdependence as a species is 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 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, 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.”

Richard Kronick, Ph.D., director, Agency for Healthcare Research and Quality

“The budget for ARHQ is about one-one hundredth 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, and to work to try to make sure that evidence is actually understood and used. And we do have some pretty remarkable stories of success. The one that I started with, 50,000 fewer death in hospitals, 1.3 million fewer bad things happening, as a result of work that we have done at the agency. I and my colleagues need to tell the story better and that story needs to be amplified more effectively.”

Lucinda L. Maine, Ph.D., RPh, executive vice president and CEO, American Association of Colleges of Pharmacy

“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 immunizations. Today, that number is over 200,000, and every student who graduates is prepared to administer vaccines. There are lessons we can learn in that, that would really be applicable to other public health challenges.”
PANEL 3

**MODERATOR:** Frank Sesno, director, George Washington University School of Media and Public Affairs

"How quickly can we make technology data, science, and approvals work to suit our attention spans? On national radio, 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... 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?"

**William Hait, M.D., Ph.D.,** global head of research & development, Janssen

"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 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 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."

**Stephen M. Ostroff, M.D.,** acting commissioner, Food and Drug Administration

"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 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. The numbers speak for themselves... I think that one of the things that’s critical and again, looking at my role from the regulatory science perspective, is that it 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."

**Amy Comstock Rick, J.D.,** president and chief executive officer, Food and Drug Law Institute

"I am personally excited about the FDA—using more real-world data in its decision-making, how drugs are actually used by people and how they are 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."

**Marc M. Boutin, J.D.,** chief executive officer, National Health Council

"Everybody talks about what is called the gold standard. And that is the clinical trial process. You know what? When we say something is the best, it makes it really hard to innovate. And there is actually good research on that. When you determine something is the best, 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."

**Larry J. Shapiro, M.D.,** executive vice chancellor for medical affairs, Washington University in St. Louis.

"Speed is important. Lives are at stake. So I think it is a balance, but there are things that we could do to make things go faster... 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 and 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 (21st Century Cures Act) for the House—the House version of the bill—that deal with those things. I would hope they would stay there."