

2017 NATIONAL HEALTH RESEARCH FORUM

STRAIGHT TALK:

STRAIGHT TALK: IS A "DISEASE-FREE" WORLD
WITHIN REACH?



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KEYNOTE SPEAKER



Mikael Dolsten, M.D., PRESIDENT, WORLDWIDE RESEARCH AND DEVELOPMENT OF PFIZER “ Is the disease-free world within reach? From a science perspective, the opportunity has never been greater to take on such a bold challenge and ambition. We are at a true crossroads for scientific innovation from basic science to clinical medicines to technologies that allow diagnostics or digital devices to be used in health care. While we can dream about cures being within reach, it is not a contribution from one group. Our ecosystem must commit to further acceleration working together over boundaries, and it is a real opportunity today in this very exciting time to start the journey with a more swift speed. ”

PANEL 1



Moderator Marilyn Serafini, HEALTH CARE POLICY CONSULTANT “ Ninety-one Americans die every day from an opioid overdose and health care cost for opioid substance use disorders exceed \$26 billion a year in direct costs. With that in mind, how should we prioritize our research efforts to achieve the best return for the nation’s health while also reducing costs? ”



Gopal Khanna, DIRECTOR OF THE AGENCY FOR HEALTHCARE RESEARCH AND QUALITY “ It was the analysis of the data we collect from states that helped us identify the opioid problem a couple of years before anybody else even recognized how large and big that problem was. Data is extremely important not just to track but to identify and take a predictive approach so that solutions can be thought of from a policy point of view as well as the point of care, and most importantly for research purposes... We are working with states to identify how we can do medicine-assisted treatment so that we can figure out what might be the best way to make that happen in rural America. Because a lot of people think that it might be an East Coast or West Coast problem. But actually, opioid [addiction] is agnostic to race, age, income levels and location. ”



The Hon. Patrick Kennedy, U.S. REPRESENTATIVE, 1995–2011 “ In America, politically, socially, economically people recognize [opioid addiction] as a catastrophe and we have to do more. CDC is funding 44 states to improve their prevention programs to strengthen the prescription drug monitoring modules so that doctors could know if a person got three or four other prescriptions right before they were entering the new prescription. They are tracking hot spots so they can get there for whatever the latest Fentanyl equivalent is that’s on the streets. We’re working closely with public safety, with DEA, with DOJ to address the high-incidence drug trafficking areas. I just think this is touching too many families for us to be in denial, I think we have a turning point. ”



Anne Schuchat, PRINCIPAL DEPUTY DIRECTOR FOR CENTERS FOR DISEASE CONTROL AND PREVENTION “ When you look at opioid prescribing in America, it’s really shocking. We have a six-fold variation from high-prescribing counties to low-prescribing counties, and even though we’ve had a decrease in prescribing since 2010 in America, our rates are three-fold higher than Europe. We got into this with good intentions, but clinicians like me were taught that you can’t get addicted to these medicines if you’re really in pain. We thought they were safe and effective. It turns out for a lot of types of pain, opioids are not effective for chronic, long-term pain of many types, and you cannot always anticipate who they’re going to harm. ”

Governor Charles Baker, 72ND GOVERNOR OF MASSACHUSETTS

“ We’ve done public service campaigns and straight out media campaigns on stigma, and we’ve done a lot of work to expand our treatment and recovery capacity. We’ve increased it by almost 50 percent and the last waiver we got from the federal government for our Medicaid program dramatically expands our mental health and addiction coverage capability. And we’re the only state in the country where you can’t graduate from medical school, dental school, nursing school or pharmacy school without taking and passing a course in opioid therapy and pain management. And we’re the only state in the country where if you’re a licensed prescriber, when you go back to get your license renewed, you’re going to have to take and pass a course in opioid therapy and pain management. ”



Seth Ginsberg, PRESIDENT OF THE GLOBAL HEALTHY LIVING FOUNDATION

“ I really appreciate the conversation here and the dialogue, and the important work that everybody is doing collectively to address the epidemic of opioid addiction. But I don’t want it to come at the risk or the cost of eclipsing the genuine and incredible pain, literally, that millions of Americans are living with... We can do better, we can do both. We can help the people in pain and we can prevent the people from getting addicted, and I just want to make sure we’re addressing both sides of the ledger and I believe, we believe, that patient-reported outcomes could technically sit as a fulcrum between those sides. ”



Lucinda Maine, VICE PRESIDENT AND CEO OF THE AMERICAN ASSOCIATION OF COLLEGES OF PHARMACY

“ As I think about the opioid issue, every time I try to touch it, it just gets bigger and more complex. And I think that it is a great example of really great intentions—clinical intentions, regulatory intentions—resulting in unintended, unexpected, and really tragic consequences. I really think there’s two elements to addressing the crisis, and there’s no simple answer because there’s a lot of vectors in it. One is definitely research. And the other is education. On the research side, it goes everywhere from understanding pain better as a neurological phenomenon to finding some non-addictive strategies for addressing pain more effectively to understanding the socioeconomic and behavioral issues that would help clinicians and regulators make better choices at the individual patient level and at the population level. 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. ”





Remarks and Q&A

Scott Gottlieb, COMMISSIONER OF THE FOOD AND DRUG ADMINISTRATION “ We’re at a point in the history of medicine, similar to other great inflections in science, where fundamental principles of science and medicine became firmly established as part of a great leap in public health. Our aim is to make sure our policies match the sophistication of the science we’re asked to evaluate, and help enable this new paradigm of beneficial innovation. That’s going to be a big focus of my medical innovation access plan, parts of which we’ve already announced, and our forthcoming strategic policy road map. This road map will serve as an organizing framework, a strategic plan for unveiling and advancing the major policy efforts we plan to undertake across all our centers. ”

PANEL 2



Moderator Byron Pitts, CO-ANCHOR OF ABC NEWS’ “NIGHTLINE” “ We’re going to look at our conversation in three areas. Landscape, the challenges, the future, and then we’ll open up. How do you integrate the patient voice into the research and development?...All of you care deeply about health care. It’s your life’s work, as for people in our audience. But many of you represent different lanes. What are your tensions, and—what are your strategies for managing those tensions? Because sometimes, I would imagine, your interests are different. ”



Scott Whitaker, PRESIDENT AND CEO OF ADVAMED “ There are a lot of tensions that exist in the health care system. Clearly, an innovator and a payer, there’s a natural tension that exists there. An innovator and a regulator, a natural tension that exists there. And a healthy one, I would add. But there’s one thing at the center of all those that pulls us all together, and that’s the patient. Oftentimes—and I’ll speak from the government’s perspective—it feels like the government policy is budget-centric and not patient-centric. And if you think about policies that might be more patient-focused than budget-focused, you might get the better solution. ”



Joe Selby, M.D., MPH, EXECUTIVE DIRECTOR OF THE PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE “ The value discussion is a place where folks are trying to find common ground. Everybody can agree that something should be valuable to patients. Now, it’s absolutely true that in order to calculate value, you have to understand what patients value and how these different treatments affect that. So, that’s part of the mix there, that we need these kinds of studies that look at a wide range of outcomes...Patients have blown my mind, all of our minds, I think, in the last five, 10 years. The ways that they refocused us on the right outcomes and the right research questions, making research that I think will move faster through the pipeline because everybody agrees that it’s needed. ”



Victor Dzau, M.D., PRESIDENT OF THE NATIONAL ACADEMY OF MEDICINE “ I think technology, in terms of science discovery, is moving really quickly. Where we’re falling behind is, in fact, the taking from the pre-clinical all the way to adoption and practice. That’s where I think we need a lot of push, a lot of change. We’re moving way too slowly. Maybe we need to think about different ways of using data. First, different trial design. Adaptive trial, right? Bayesian methodology to look at new ways to look at taking data together from different early trial to a pivotal trial move faster. You heard about the idea of maybe using technology. And you know, we’ve been talking for a long time—how to integrate clinical data-base with recent clinical research so that we don’t have to rediscover every time you enroll a patient. ”

Mark McClellan, M.D., PH.D., MPA, ROBERT J. MARGOLIS PROFESSOR OF BUSINESS, MEDICINE, AND POLICY, AND DIRECTOR OF THE DUKE-ROBERT J. MARGOLIS, MD, CENTER FOR HEALTH POLICY, DUKE UNIVERSITY



“ A patient is not just someone with pain, or someone who gets a procedure, but a whole constellation of genetic predispositions, behavioral and environmental influences, preferences that influence, increasingly, what their treatment should involve. And we are not doing a very good job of aligning all these discoveries that are coming through the process, either in the development process of knowing which treatment is going to work best in which patients, where real-world evidence can help, or in the utilization process. So, still a lot of work to do there, and a big need for research on what works in getting the right treatment to the right patient. ”

Nancy Brown, CHIEF EXECUTIVE OFFICER OF THE AMERICAN HEART ASSOCIATION (AHA)



“ I haven’t heard anyone yet today talk about people who aren’t in a system. And, you know, if we look at, for example, the unfortunate uptick in death from stroke. And you peel back that data, it is African-Americans and Hispanic-Americans who have driven that number up, and it looks like the entire population has increased in terms of deaths from stroke. The same thing is happening with heart disease deaths. For 40 consecutive years, deaths from heart disease declined until two years ago. And now, there’s this uptick related to type II diabetes, related to obesity, related to all of these things that are impacting lifestyle, but there is a disproportionate effect in certain populations. And we have to be willing to step up to solve those problems, too. ”

Joel Beetsch, PH.D., VICE PRESIDENT OF GLOBAL PATIENT ADVOCACY AT CELGENE CORPORATION



“ We’re creating data lakes and data oceans and we need better analytics tools or better fishing poles. I do think that data is an important piece, but I think the patient-focused element is a critical one. There’s a lot of focus on including that patient voice, and patient-focused drug development efforts that the FDA has underway. There are European regulations that are being put into place in 2018 to make sure that that patient voice is included. We can make innovative trials that are friendly to patients to encourage them to join in a clinical trial and stay in it. ”





PANEL 3

MODERATOR Jeanne Cummings, DEPUTY BUREAU CHIEF FOR *THE WALL STREET JOURNAL* “When we talk about ending disease, there are all kinds of ramifications but there’s an awfully long road to that particular goal. And for a long time, we have been hearing a lot about research into genes and other different types of research work that initially held such promise. Can you bring us up to date on whether expectations were too high, how well is it going, what is the status? How do you have a world without disease when some parts of the world don’t have access to all of the things that they need?”



William N. Hait, M.D., PH.D., GLOBAL HEAD OF JANSSEN RESEARCH & DEVELOPMENT ONE OF THE JANSSEN PHARMACEUTICAL COMPANIES OF JOHNSON & JOHNSON “The facts of the matter are that as you age, around 50 you begin accumulating diseases. And by the time you’re 60, 70, 80, you have four or five of these things, and they’re pretty miserable. And if lifespan is increasing somewhat and you’re getting these diseases at 50, you’re going to live a longer period of time carrying along these unwanted accompaniments through life. We’re all incubating some disease, whether we like it or not, and our approach has been soon we’ll understand susceptibilities to most diseases, and by putting in real data, we’ll understand risk. And what if we could then have more knowledge to understand how we could intervene to either prevent a disease from ever happening or intercept the disease-causing process. I think if we move in that direction of prevention, how critically important it is, then I think we’ll make real progress.”



Gary Reedy, CEO OF THE AMERICAN CANCER SOCIETY “One of the things we’re still challenged with, and this has been mentioned several times today, and that’s kind of why we put an exclamation mark on it, is with all the great research that’s going on, until we really address the social determinants of health, a lot of people are not going to be able to have access to that research. The primary goal is to stay well and to not get cancer or any other disease to begin with. If we can address some of these socioeconomic issues, educational issues, access issues, that’s going to help a lot, as the research train starts to pick up speed and move faster. We have to make sure we’re addressing the other issues at the same time we’re advancing the research.”



Francis Collins, M.D., DIRECTOR OF THE NATIONAL INSTITUTES OF HEALTH “When we say a “world free of disease” we’re talking about the whole world, because if this is just a target for the developed countries then we have basically failed to live up to our responsibilities. There’s a tradition here of that kind of outreach that we need to expand. We also need to work harder to help the low and middle income countries develop their own capacity for doing research, and developing, therefore, research projects that are targeted at the needs that they see, as opposed to something that somebody outside their area has decided is the need.”



Iris Loew-Friedrich, M.D., CHIEF MEDICAL OFFICER FOR UCB “We cannot talk about a world free of disease if we are leaving out certain geographies. I think we have to be very clear in understanding what are the patient needs in these geographies, and they might be very different from first world patient needs. I think we have an obligation as a world community to really look into what are ways to provide adequate treatment and care to patients in the third world. From a UCB perspective, we are trying to address this by working with physicians in the field of epilepsy in Africa, in rural areas in China and other geographies because we have a longstanding leadership in the care for epilepsy patients and we feel a very strong obligation and commitment to ensure that we bring our medicines also to patients in the developing world.”

Mikael Dolsten, M.D., PH.D., PRESIDENT, WORLDWIDE RESEARCH AND DEVELOPMENT OF PFIZER “The balance between prevention and intervention is a key part of both public and medical policy, and unfortunately the U.S. has not been the most active country in having enough preventive programs. Whether it’s for preventing the use of painkillers of the opiate nature, or we spoke about the rise of the obesity epidemic. When it comes to infections we, in contrast, have a proud tradition here to be among the leaders in developing vaccines. There are so many more of these bacterial and viral threats, and we see a tremendous opportunity to broaden vaccines as a real pillar in our health care system. And it just makes me more enthusiastic to think about how you can probably rebalance between intervention and prevention.”



Ann Cary, RN, MPH, PH.D., FNAP, DEAN OF THE SCHOOL OF NURSING AND HEALTH STUDIES AT THE UNIVERSITY OF MISSOURI KANSAS CITY “We know that about 80 percent of health care outcomes are due to non-clinical interventions. And so, if we think about that space and we think about where the research needs to focus, we need to think about redirecting or expanding our research dollars into those areas that we know do impact, such as health behaviors, such as the social and economic determinants of health, and the physical environments that our patients are in. Precision medicine is important because it studies people where they are and we can tailor and mediate some of the interventions based on genetics. Zip code is as important as genetic code in this country in terms of healing and prevention.”



David Neal, CBE, FMedSci FRCS, SENIOR VICE-PRESIDENT FOR GLOBAL RESEARCH AT ELSEVIER “There’s a debate on balances between spending money on health and research, and one of the things we’re thinking a lot about at the moment is really unintended variation in health care. If you think of horizon one, you know, how could we improve health care right now? Whichever country you look at there is enormous variation in the quality of care given. And I think there’s a lot that could be done in terms of providing support to nurses and doctors in the workplace to give guideline-driven care. I think one of the things we could do is to try and provide better tools to inform researchers of things that are going on, and getting that practice into care more quickly.”



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