



12th Annual State of Personalized Medicine Luncheon Address

TRANSCRIPT

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EDWARD ABRAHAMS: Good afternoon, everybody. Good afternoon. My name is Edward Abrahams. I'm president of the Personalized Medicine Coalition. As you know, especially if you're a member, PMC is an education and advocacy organization representing all of the stakeholders in health care. Our mission is to promote personalized medicine because we believe that both patients, and of equal significance the health system, will benefit if we move away from one-size-fits-all medicine towards an approach that is predictive, preventative and personalized.

Unfortunately, that is easier to envision than to do, notably because our regulatory, reimbursement and educational systems were put in place long before we understood the significance of individual variation. And they don't change easily, especially absent compelling, if not overwhelming evidence, that linking diagnostics to therapy will benefit not only patients, but the health system as well. But that is our mission, to change mindsets so that personalized medicine is not an afterthought but an imperative for policymakers, no matter where they are.

Which brings us to this afternoon's 12th annual State of Personalized Medicine Luncheon Address, a series that gives leaders in the field a chance to define their respective visions of the future. But before I introduce Peter Hoehn, global leader for companion diagnostics at Johnson & Johnson, who will in turn introduce our keynote speaker, I want to acknowledge the hard work of PMC's staff. While it's customary to thank the staff last but not least, I would like to thank them first and foremost. And so – (applause) – I want to introduce them all to you.

Amy Miller, executive vice president; Daryl Pritchard, vice president for science policy; Mary Bordoni, membership director; Faswilla Sampson, director of operations; Chris Wells, communications director; and David Davenport, office administration. I'd like to ask them all, without too much embarrassment, to stand. (Laughter, applause.)

I would also like to acknowledge PMC's board, which met today, including our chair Bill Dalton of M2Gen, a spinoff of Moffitt Cancer Center; Steven Eck, Astellas; Amy Abernethy, Flatiron; Donna Cryer, Global Liver Institute; Michael Kolodziej, Aetna; Howard McLeod, Moffitt Cancer Center; Michael Pellini, Foundation Medicine; Howard – oh, I already said that – Michael Pellini, Foundation Medicine; Kim Popovits, Genomic Health; Lori Reilly, PhRMA; Hakan Sakul, Pfizer, Jay Wohlgemuth, Quest Diagnostics. These people are a veritable who's who of personalized medicine. And I'd now like to ask our board members to stand. (Applause.)

Finally, I want to introduce Peter Hoehn of Janssen Diagnostics, the sponsor of today's luncheon. Today's luncheon focuses on the pharmaceutical industry and its commitment to personalized medicine, which is outlined in an article that Steven Eck and I wrote and published in the current issue of the *Journal for Precision Medicine*, which we shared with you at your table seats today. And I hope you'll take it with you and read about what we have argued. We are privileged to have Peter Hoehn, who comes from the diagnostics division of a large pharmaceutical company, to set the stage for us.

Peter, and this is very significant, is responsible for setting overall business objectives and strategic priorities for diagnostic solutions at J&J, and for leading the development and implementation of identified companion and complementary diagnostic opportunities to support drug development across



all indications. With thanks for his support and Janssen's long-time participation in the Personalized Medicine Coalition, it is my pleasure to turn the podium over to him. (Applause.)

PETER HOEHN: Thank you, Ed. It is a pleasure and an honor to be here today. And before I start, I just wanted to thank the PMC for your longstanding commitment to personalized medicine. I think when PMC started some 12 years ago, personalized medicine seemed like a distant vision. But today it is really part of the conversation about every change to health care models, and an essential element of many policy initiatives. And this is no small part to your leadership and commitment. So thank you.

The growth of personalized medicine is also evidence by the prominence it now plays on the agendas of almost every major pharmaceutical company. And to that end, I'm very thankful for the opportunity to introduce today's keynote speaker, Steve Ubl, president and CEO of PhRMA. But before I invite Steve up to the podium, I wanted to reflect on this journey of personalized medicine and the road ahead. You can say that the current vision of personalized medicine started some 25 years ago at the beginning of the project to map the human genome. That was the very exciting time, but also seemed at time that the promise might not live up to the hype.

As I was thinking about my remarks today, I recall something I came across four years ago. For my son's 18th birthday, his grandmother gave him a copy of TIME Magazine from the week that he was born, January 17th, 1994. This was a special issue on genetics. And the cover said: Genetics, the future is now. New breakthroughs can cure diseases and save lives. So when I came home from work and I saw that cover in 2012, I turned to my then-18-year old son and said: Kyle, nothing has really happened in your lifetime. (Laughter.)

Of course, I was being somewhat factious, but we must admit that the breakthroughs we were hoping for have taken some time to develop. That is often the case with new advances in technology and science. But today, though, I truly do believe we are at an inflection point. Genetic sequencing is now routine. New diagnostic technologies have expanded the breadth and possibility of new information about diseases. And we have seen a steady rise in the number of targeted therapies, particularly in oncology.

In addition to these scientific advances, market trends also point towards growth in personalized medicine. Today every health care system around the world is demanding better outcomes and lower costs. And for their part, patients and providers are demanding and expecting more access to critical information about treatment decisions. These trends will also drive the growth of personalized medicine.

And Janssen, we welcome this new model. We're dedicated to improving outcomes to the patients we serve. We also recognize that diagnostics will be critical to our success. We know it will no longer be good enough to provide a drug with the mere hope that a patient will respond, but that science and society will demand more of us, that we must identify people with diseases before they progress too far, that we must identify the right people who will respond to our therapies, and we must monitor their response. This is why, at Janssen, we created Janssen Diagnostics, a diagnostic unit within our pharm business for the purpose of bringing diagnostic and personalized medicine solutions together in support of our pharm strategies.



We've all see the criticality of diagnostics in cancer treatment, but we should realize that advances in many other disease states will also require the integration of diagnostics and therapy. One great example is Alzheimer's disease. Today more than five million people in America are living with Alzheimer's and more than 3 million cases are identified every year. Unfortunately, there are no drugs on the market to halt or slow the disease. Over the last few decades, though, Janssen and many other major pharmaceutical companies have invested billions of dollars in R&D, and new drugs are in development which have the promise to slow the disease or halt it altogether.

But to be effective, these drugs will likely need to be given before symptoms appear – symptoms like behavior change and memory problems. We will have to identify pathology in the brain. So to do this, we will need diagnostics. But to be effective as a screening tool, these diagnostics will need to be low cost and noninvasive. And since many patients will be on therapy for many, many years, we will also have to be able to identify who will progress rapidly, and therefore need the drug right away, and which patients may be able to wait and benefit from nondrug therapy. And we'll also be able – have to be able to prove that the drug is working and identify patients when they may no longer benefit.

This is truly personalized medicine. And it will be critical in the fight in diseases like Alzheimer's. But it will not come easy because while we can see the criticality of diagnostic innovation advancing together with or even in front of pharmaceutical innovation, this often isn't the case. Our system today does not adequately foster diagnostic innovation. Pathways for both regulatory approval and reimbursement are uncertain and costly, and reimbursement levels often do not typically reflect the test's true value. And a murky IP environment can make it difficult to attract investment for novel diagnostics.

So while I truly believe we are on an inflection point for personalized medicine, we still have much work to do. We have to continue to push for policy changes, such as diagnostic regulatory reform and improvements in reimbursement. And to better foster the required investment for innovation, we should advocate for appropriate incentives such as enhanced exclusivity or reimbursement for first-mover innovators. And of course, as an industry, we have to continue to do our part to develop new innovation models such as precompetitive consortiums and industry-government partnerships that can advance learning and provide shared access to invaluable data.

At Janssen we share a common focus with all of you to advance personalized medicine on behalf of patients. We know much work remains, however we're excited about the progress that we've made to date and are confident that through our joint efforts we can now truly unlock the promise of personalized medicine and make sure that the bold headlines that we generate today about the future of health care really do come true.

And with that, I would like to introduce Steve Ubl. Steve, as you know, is president and CEO of the Pharmaceutical Research and Manufacturers of America, or PhRMA, which represents America's leading biopharmaceutical companies. Steve was named PhRMA president and CEO in September of 2015, after more than 10 years as president and CEO of the medical technology association AdvaMed. In his role as President and CEO of PhRMA, Stephen leads PhRMA's working preserving and strengthening health care – a health care and economic environment that encourage medical innovation, new drug discover, and access to lifesaving medicines.



Stephen is recognized around the world as a leading health care advocate and policy expert who collaborates successfully with diverse stakeholder groups. He is routinely recognized as one of Washington's most effective advocates by political publications and has been named by *Modern Healthcare* one of the hundred most influential people in health care. Please join me in welcoming Steve as he provides his thoughts on the value and promise of personalized medicine. (Applause.)

STEPHEN UBL: Well, thank you very much, Peter, for that nice introduction and terrific set of remarks. So I'm relatively new to PhRMA, having come from the medical device industry, so bear with me as I give you my initial observations I'm really starting to fill out my picture of the other side of this equation, understanding the diagnostics role, I see some of my former colleagues from AdvaMed in the room, but understanding how that piece marries up to the biopharmaceutical innovation side.

But I want to start by recognizing Ed and Amy for their terrific work. PhRMA's very proud to be a founding member of the PMC Coalition. And there's been a lot of good work that's been done in this space thanks to your leadership. So it's a pleasure to be with you here today.

As Peter said, this has been a long road. When you think back to the early '90s with the human genome project and all the excitement that was built around the scientific advances that would be born, and then you fast forward 10 years and there were really only a couple of those targeted interventions that were identified – the HER2+ and CML discoveries. And fast forward again another five to seven years, and there was a growing sense of skepticism, I think, that the human genome project was overhyped or, worse yet, that the biopharmaceutical industry in some fashion was standing in the way somehow of forward progress in the personalized medicine space.

But I'm pleased today to say the skeptics were clearly wrong. There's lots of excitement around the fact that you've got really tremendous growth in the number of targeted therapies that have been identified. So 13 in 2006, compared to today at 140 targeted therapies identified. And just last year at FDA, you know, 28 percent of all medicines approved were for targeted therapies. So really a tremendous story, I think. And please that the skeptics were wrong.

I want to try to open the aperture a bit on the discussion in the sense that this discussion is about a lot more than just targeted therapies. There are lots of ways that those therapies are revolutionizing patient care. So whether it's giving patients better information about their genetic profile, allowing – empowering, really, patients to change their behavior of lifestyle in accordance with that information, earlier disease detection as Peter alluded to, and better molecular-based diagnostics, or being able in real time to follow disease progression and adjust treatment accordingly. You know, I didn't want to talk about each of these aspects, but I think it tells the broader picture of how targeted therapies are really changing the way patient care is delivered today.

And just stepping back as well, you know, that tells the clinical story, but there's a lot of cost savings that come from the system as a whole due to targeted therapies as well – again, earlier diagnosis, getting the right drug to the right patient at the right time, and preventing complications. Recent research suggests that targeted therapies, for example, can reduce chemotherapy which has, as we all know, tremendous human costs, but also system-wide costs, as well as gene-based tests that can better target blood thinners that have reduced the potential for stroke in 17,000 cases per year.



I want to just go through some recent examples that I think are really compelling and show the progress that we're making. I mean, Jimmy Carter, you know, recently diagnosed with metastatic melanoma, which is one of the most deadly forms of cancer. You know, just a few years ago that would have been a death sentence. And it's just remarkable that former President Carter is no longer receiving treatment. And it's just – I think the advances in immunotherapy are really embodied in this story. And think about, you know, Sunday school that he will be teaching, the buildings and Habitat for Humanity houses that he'll be building well into his 90s, and it's just a really compelling story that 40 percent of patients with this diagnosis are going to live for three years or more.

If you look at non-small cell lung cancer, it's a similar story. This chart is a bit of an eye chart on the left-hand side, but it shows that two-thirds of advanced non-small cell lung cancer have an actionable genetic mutation. And the right side is a story that we're familiar with at PhRMA. Matt Ellefson, who's been featured in an "I'm Not Average" campaign that we're waging. You know, Matt has just this tremendous story of how he took charge of his own care and set up a Google Alert to learn as much as he possibly could about what was happening in the research side and clinical trials with regard to his condition. He learned about an improved treatment that treats the ALK variation, got tested for that variation, found that he had it, and again a powerful story of a case where he was told he had a 5 percent chance of being alive five years and is thriving after seven.

Another great example I think is the statin story. It's important on a number of levels. There's probably been no more important breakthrough in reducing deaths associated with heart disease. But not all patients are effectively treated by statins. We know that there are hereditary variants that patients are treatment resistant to statins. And that's why PCSK9s are so important. I think this story also tells the lifecycle story of the industry that's underappreciated in this cost context, you know, discussion that we're having around drug pricing, because statins are now a generic medicine available for really pennies on the day. But we have to have headroom for innovation around important innovations, like PCSK9s, that are so valuable for patients that are not responsive.

And if you look at the pipeline, I think it's even more exciting. So 42 percent of all medicines in the pipeline today have the potential to be targeted therapies. And 73 percent of cancer medicines have the potential to be personalized medicines. So I think we're really on the cusp of a really exciting era. And, you know, again, I'll stipulate that I am not a scientist, I am not a physician, but I've learned so much about what's on the horizon. And truly, you think about CAR T, it's like science fiction. You know, we're talking about the next horizon in personalized medicine where we're going to remove an individual's T-cells, genetically re-engineer them to better target cancer cells and eliminate the defenses that cancer tumors have, and reintroduce them into the human body – it's just really, truly incredible technology that we're seeing on the horizon.

And you know, I think it's – there's not a lot in Washington that brings people together these days, but I'm excited by the broad bipartisan support that these initiatives have. And I think policymakers, again across the political spectrum, want to seize the opportunity and build on the progress that's being made. So whether it's the Precision Medicine Initiative, the Cancer Moonshot, the 21st Century Cures Initiative taking shape in the Congress, people understand that we have the opportunity to make serious headway in the treatment of cancer and other deadly diseases. And they want to make sure that we, again, can stand on the shoulders of previous generations and make progress.



It's not all a good news story. I have to say that one of the – one of the things that I bring from my device experience to PhRMA is the recognition that there's nothing that chills investment like uncertainty. And it's a fact in the device space that really over the last five years, investment in startup medical technology companies – you know, the true early-stage companies – fell by something like 72 percent. And why is that? Well, because of the issue – a lot of the issues that Peter referenced earlier, where you've got uncertainty around the regulatory pathway. Still the case in the device industry, at least, that you've got products being introduced in Europe several years before they're being introduced in the U.S. Lots of uncertainty around reimbursement, around whether breakthrough technologies would be paid for. We talked about the diagnostic uncertainty.

My point is just that we shouldn't take progress for granted. And we need a robust policy ecosystem to continue to support innovation because investors and supporters of this industry can move to other places, as they did in the device space, something that I know that my former colleagues at AdvaMed are very focused on in terms of rehabilitating that particular ecosystem. But it's a long way of introducing the next topic I wanted to raise, which is the Part B Demonstration Project recently released by CMS, which we think really takes us a step backwards. You know, we talked earlier about the initiatives in Congress to really seize the opportunity. We think this has the potential to hinder patient access to tailored treatments and cures.

Why do we think that? I'll just give you a few reasons. One is, just on a process basis, it seems like a clear overreach of the authority that Congress gave to CMS. So what we envisioned with the demonstration project, right, is that we would test certain things in a limited venue, we would take feedback along the way. If we found something that worked, a policy intervention, we might roll it out more broadly over time. Instead, in this case, you have a situation where 75 percent of the country mandated to participate day one. Seventy-five percent of Part B drugs covered by the demonstration. Again, it doesn't feel to us like the spirit that Congress envisioned in terms of testing concepts and moving them forward.

The other point I would make is that, you know, third party research suggests that the cut off for whether – when a physician prescribes a medicine, will do so at a loss or at a gain, is about \$480. So if there's a novel, new breakthrough therapy that costs more than \$480, the system is going to be biased against prescribing those interventions. And there may indeed be a bias towards older, less-effective medicines that may be cheaper. And I think, you know, a lot of what gets lost in this discussion is what is the impact on individual patients? What do patients care about? Patients want to make sure that the physician is prescribing the medicine that is most appropriate for them.

The third point I would make is – and we're still learning what this means; it's pretty ambiguous in the way that the proposal's been crafted – but reference pricing. What reference pricing essentially means is that you're going to lump together products. And it has the assumption that they're interchangeable. And again, that cuts against the very foundation of what personalized intervention is meant to be. So in a sense, you have – you have a policy that's trying to incentivize homogeneity when the science and really the path forward is going to lead toward more heterogeneity and individual treatment. So for those reasons, you know, we think this policy is kind of a step backwards and could hinder patient access.

Another thing that we worry about is the evolution of value frameworks. And I have to say at the outset that we all understand that we're moving to a value-based world, and that value assessments can be quite



helpful, particularly for patients who are trying to work through multiple treatment options in a particular space. So leaning again back on my device experience, we used to talk a lot about prostate cancer. You know, if you – there are lots of modalities that are aimed at prostate cancer. You can burn the tissue, freeze the tissue, prostatectomy, watchful waiting, lots of ways to go at the condition. If you're a patient, you might well want to have as much information as you can possibly have on the clinical benefits of those interventions.

I think where value assessments go astray is when they try to superimpose cost requirements that can be arbitrary. So ICER, for example, just to highlight one model that we're concerned about, has an arbitrary threshold, in our view, that basically says: We should spend GDP+1 on health care. And within that, there's a certain amount that should be allocated for pharmaceutical spending. So by definition if you have a number of breakthroughs in any given year you're devaluing those interventions. And moreover, you're sort of staking a snapshot in time.

Again, leaning back on my device experience, David Cutler, who's a well-renowned health care economist, did a study of angioplasty over a 10-year period. And basically found initially not clinically effective, not cost effective. Ten years later, as technology improves, stenting was introduced, physician technique improved, it was overwhelmingly clinically effective and cost effective. My point is that new pharmaceutical interventions and innovations were not all well-understood at launch what the implications are going to be for patients.

And so I think as we move to value frameworks, and there are frameworks that are being developed that we think are more patient-centric and more aimed at making sure physicians and patients can make decisions – like, NHC has done important work in this area, ISPOR, ASCO and others. So this is a nascent science. There's no magic box that you can evaluate a medicine, especially at its inception. And so we should proceed with caution and not send signals that devalue breakthroughs.

And so we do think, however, you know, I want to make this very clear and it's one of the things I've been trying to bring to PhRMA, is that there are pragmatic, consumer-oriented steps that we can take that address healthcare costs holistically and make progress. So as we move to a value-driven health care system, there are steps that we can take to try to further the efforts. So you know, with regard to value-based purchasing arrangements, there's some of these that have been forged – you know, Cigna, Anthem, with Lilly in recent weeks, for example. But it's still the case that FDA and law and regulation really chill some of these discussion.

It's very difficult for manufacturers to communicate with payers before a product is approved. And payers would benefit from having that visibility to the industry's pipeline, but you can run afoul of FDA law and regulation. Even when a product is approved, and you might know information about something that's on-label – say, for example, that the product can reduce hospital readmissions. That could form the foundation of an outcomes-based arrangement. That can run afoul of FDA law and regulation or the OIG statute, for example. So we think there are pragmatic steps that we could take to update, really modernize statutes that were really written for a fee-for-service world that we're no longer living in, to simulate more of these – more of these arrangements.

On the FDA side, as has been discussed before as well, we think there are similar pragmatic steps that can be taken to more rapidly validate, qualify biomarkers, to use those biomarkers as surrogate



endpoints, to harness the power of real-world evidence so that we don't have to try to answer all the questions up front, but that we're learning as we go. These are steps that we think can lower development costs and enhance competition by bringing products to patients more quickly. And just in closing, and this is related to the biomarker point, and Dr. Califf is an expert in this area, but adapting clinical trial design to better use those pragmatic tools as well.

So I use the lung map example. Instead of patients having to identify trials, you know, go through the enrollment process, the IRB process one step at a time, having a trial that's adaptive and set up so that, you know, patients have essentially a one-stop shop where there's multiple arms and it doesn't matter what the variant they're tested for, they're going to get assigned to the treatment that's most appropriate to them. Or even if they don't have a genetic marker, there's going to be a treatment arm. There'll be no placebo in this case. So I think together biomarkers and a more effective model for clinical trial design can help us move in the right direction.

So I'm going to stop there. I'm delighted to be here, and happy to take any questions you might have. (Applause.)

MR. ABRAHAMAS: Thank you very much. (Inaudible.) And while I give you some – we would ask you to go to the microphones when you ask a question. And we'll call on Amy Miller to ask the first one.

MR. UBL: No PhRMA staff questions. (Laughter.) No, just kidding.

Q: Thank you.

Under your 16-year stewardship at AdvaMed, you saw that diagnostic tests were so novel that you started AdvaMedDx within the larger organization. Can you explain to us how your experience with diagnostics can enhance your stewardship of PhRMA, please?

MR. UBL: No, it's a great question. I start off on the proposition that diagnostics is, like, the least appreciated aspect of health care. You know, 2 percent of spending, drives 72 percent of decision making, but the magic happens somewhere else. So if you go to the doctor's office, you get a blood test, you don't hear anything – hopefully. Or, if you do, you don't appreciate how that blood sample got to a treatment outcome. It's the Rodney Dangerfield of the health care system. (Laughter.) It is so, you know, underappreciated.

And what we found at AdvaMed is that, you know, you have this great diversity of companies, small, medium and large, that are in this space. Great uncertainty in the regulatory pathway and reimbursement area. And we really felt like we needed a dedicated focus to get critical mass in sort of a fragmented environment. And I'm looking at Katira (ph) and, you know, and Kalayha (ph), who I worked with at AdvaMed, Andy Fish and others, who really have done a phenomenal job of really working with PMC and other stakeholders, you know, to generate more appreciation for the impact of diagnostics and the need for substantial regulatory and reimbursement reforms.

What that has taught me or brought me, you know, is just a really solid foundation at PhRMA and appreciation for how you need both parts of the puzzle. You know, you absolutely need to have better information to stratify risk. You know, the Alzheimer's point was a great point. I mean, we need



diagnostics to make sure that we're allocating resources appropriately in the health care system. And that is the right way to solve the cost challenges, not the wrong way like the Part B Demo or gravitating toward price controls or what have you, but getting the right drug or right intervention to the right patient at the right time.

Q: Zobair Younossi (ph) from Inova Health System.

Thank you very much for an excellent presentation. I think the – you know, look at assessment of evidence for value-based medicine, or cost effectiveness really was done for an old era. That is really changing rapidly with personalized medicine and precision medicine. How do you suggest we change this conversation to actually develop new ways of assessing evidence that's based on precision medicine sort of parameters, rather than sort of the old clinical trial, randomized clinical trial, or method analysis approaches? And the same thing, the threshold for willingness to pay that was done in old model, how do we change this so that those thresholds are no longer applicable, as you suggested, to sort of elevate the early on personalized medicine sort of approaches that we need to take on?

MR. UBL: That's a great question. Unfortunately, I don't have all the answers. I think that the signs of a value assessment is still nascent. And it's particularly nascent when we think about how to get the patient perspective reflected in those discussions. So there's obviously going to be a continuing role for things like qualities and other metrics that are going to be used in the value assessment framework. But I'll just – I'll give you one example. So my son, as many of you know, diagnosed with Type 1 diabetes about a year, year and a half ago, which has given me a totally different perspective on the work that I do. But, you know, A1C levels are the governing variable here that we're going to measure a lot of interventions against.

And it's not that meaningful on a day-to-day basis. I can just tell you that my 12-year old son, when he's out of range, you know, a pre-teen with Type 1, not always pretty – let me just tell you. (Laughter.) So maybe it's more useful to look at things like time in range as a metric for when you're evaluating interventions in that space. My point is that we have to get a lot better at understanding what patients really want and value. And right now, that's not happening. You know, I look at ISOR or I look at some of the other frameworks that, again, are, I think, driven more from the payer perspective, looking at more arbitrary metrics. And we really need to appreciate these frameworks. But it's going to take some time. We're going to have to get better – both on the regulatory side of how we incorporate the patient perspective, as well as on the value assessment side.

MR. ABRAHAMS: If there aren't any more questions, I'm going to ask one. Once upon a time, it was said that the pharmaceutical industry opposed personalized medicine because it would segment the market and you wouldn't be able to sell one to everybody. And obviously that's now no longer the case. Yet, we do have the issue of having to develop drugs for small populations. Inevitable, those drugs are going to be more expensive. What do we do to make policymakers more accepting of that proposition generally?

MR. UBL: Yeah. I think it's a really good point, and question. I would hearken back to the slide I put about statins. You know, we just need to do a better job of communicating the life cycle of the industry and the reality that, you know, again, statins are, you know, probably the single greatest contributor to GDP of any intervention, if you look at the reductions in heart disease and so forth. And now is



available for pennies on the day. But we have to have head room for innovation. We have to have incentives in the system so that we have an incentive for breakthrough interventions like PCSK9s.

And I just think that, you know, relentless education about the way the marketplace has evolved. I mean, again today you have a situation where you have rapidly consolidating payers and PBMs. There's absolutely fierce marketplace competition that's driving down costs. You have branded competition occurring more quickly than ever before. You look at the hep C interventions where you had, you know, one product on the market, within a year, year and a half, you've got two or three, price has come down 60 percent. So I think just relentless education about the life cycle and the importance of head room for innovation.

MR. ABRAHAMAS: And I see we do have – Peggy.

Q: Hello. I'm Peggy Eastman with *Oncology Times*. I'm not sure if this is working.

This is a cost-related question also, and that is some of these targeted medicines are really, really, really expensive. And there's concern among patients that they are having to pay more out of pocket for these drugs because their insurance companies will not cover the entire cost. What do you tell patients when they say, you know, I'm not sure I can afford this medicine which could save my life?

MR. UBL: I appreciate the question. I mean, I think it is unfortunate that some patients are having difficulty accessing the medicines they need. And it's why a lot of our companies have really stepped up to help support patients in that regard. But cost and affordability are two different questions.

And here, you know, again, I reflect back on my – I did work in the hospital industry before the device industry. But if you look at most medical interventions, you're going to a hospital, you're paying your deductible, and you're on your way. Unfortunately, with regard to pharmaceuticals, you're going to the pharmacy and you're feeling that out-of-pocket exposure with a much greater sense of immediacy. And unfortunately, payers – you know, you have a situation where payers are imposing out-of-pocket exposure to patients at a higher rate than their medical costs are growing.

So I think we need to have a discussion around the quality of insurance. We need to make sure that patients have access to the information that they really need. You know, is my doctor in the network? Is my hospital in the network? Is the drug I need covered by my plan? Is it on a formulary? Maybe it's on a tier that makes it less accessible because co-insurance is being imposed at a higher rate. So we need to have a candid conversation about the quality of insurance in the wake of the ACA, because it is imposing significant new costs on patients. And I think – so the cost and affordability question are really two different questions, in my view.

Q: Hi. Since you mentioned leaning on your device experience, what are some key differences you've learned between the two industries? And do you think the legislative priorities of the drug and device industry are aligned with each other?

MR. UBL: The last part again? I'm sorry.



Q: Are the legislative priorities of the drug and device industries aligned with each other, complementary?

MR. UBL: I think so, maybe just in reverse order. I think that, you know, you take a look at what we're talking about today in terms of diagnostics, I think we're 100 percent aligned on the need, you know, to ensure that we have a more certain regulatory and efficient regulatory pathway, and better incentives for new diagnostics at CMS. You know, I think there are similarities in the way the industries are composed. I certainly feel like it's been a solid foundation. But, you know, there are also, you know, key differences in the innovation model, whereas in the device side you've got, you know, sort of an iterative almost like software – very short life cycles.

As opposed to the pharma side, where you've got a molecule that doesn't change for some period of time, and you have much longer development times. I mean, I think that the presentation – earlier in the presentation when we walked through what's happened between the '90s and today sort of illustrates the point that we've all been making indirectly, which is it takes a long time. The science is really hard. It's probably never been harder to get a new drug from the bench to the bedside. And I think that progress sort of illustrates that. So those are some of the key differences. But I find the issues to be highly complementary and aligned.

Mmm hmm. Sorry.

Q: Hi. Amy Abernethy (ph) – Flatiron Health.

So I'm actually going to switch my hat for a second and put on my doctoring hat, as melanoma doc. And I reflect back to 2001 when STI-571 was first published in *The New England Journal*. And thought to myself, I'm never going to remember all the diagnostics, all the targeted agents, all the way that I personalize care of this patient. As we think about advancing personalized medicine in the clinic, what are the things that you're talking about at PhRMA and in Washington to try and help doctors do that?

MR. UBL: Wow, that's a great question that I also can't answer. (Laughter.) I just want to be honest that, again, I'm still learning an awful lot about this space. But I think it's an issue that we should tackle in terms of – maybe through the PMC or at PhRMA. I appreciate the question, because ultimately – and I maybe I'll just make a slight digression here – in my view, the biopharmaceutical industry exists for the very purpose of giving physicians better tools to treat their patients.

And so in the face of some criticism in the physician community, my council to our industry has been at some level you have to embrace that. You have to have a conversation with the physician community because at the end of the day it's far better to argue about value in the private market with your customer than to have others superimpose through blunt instruments, like the Part B Demo, or other instruments, how that negotiation ought to occur.

But to your point, we can produce best interventions. But if physicians don't appreciate them or have tools that they can use to apply them in practice, it's all for naught. So I'd be interested in exploring that further. I'm just being honest, I don't have an immediate silver bullet for you.

Q: I'm ready to go. (Laughter.)



MR. UBL: All right.

Q: Hi. Ferdous Al-Faruque with *The Gray Sheet*.

You seem to be touting a lot the potential of real-world evidence. And it's interesting, because in the ongoing MDUFA negotiations it seems your former employer, AdvaMed, has kind of said, hey, we're not so sure the jury is in on that, on the potential on RWE. I was wondering, now that you're – you know, what your thoughts are on RWE, you know, because FDA really wants to build their national evaluation system Health IT. Where is your thoughts on trying to get that kick started and the potential of RWE from the NEST (ph) framework or system?

MR. UBL: Yeah. I think I'll avoid speaking for my former employer. (Laughter.) Especially since they're well-represented in the room. And I know they'd be happy to follow up with you on the device perspective. But you know, I will say that, you know, speaking directly, the reality is that on the device side it's a little more complicated because of the heterogeneity of the products involved and the complexity of getting that information captured, whereas on the drug side you have much more granular information that facilitates access to RWE in a more user-friendly way.

But I think that at the end of the day both industries will benefit from having the real-time capability of answering questions, you know, that can't be answered up front in the market as you get more information about the products involved, and give regulators more confidence, and everybody more confidence, that they can make those decisions, for example, in phase II when the data is overwhelming, if they have real tools that they can use to capture information once a product's on the market.

Q: Sorry, quick follow up. On the NEST (ph) issue, is there anything from a legislative perspective or a regulatory perspective that could be done that would maybe improve the assurance that RWE in the device side would help bring products to market, give more assurance to researchers and investors?

MR. UBL: I've sort of lost the thread on the device side, I got to be honest with you. I don't know, you know, where that stands today. But again, I imagine that in the context of 21st century cures, or the device user-fee negotiations, that there will be, you know, further dialogue around how best and when, you know, to move in this direction.

MR. ABRAHAMS: Let's make this the last question.

Q: Dena Minning, M.D., Ph.D., and candidate for Congress. Thanks for the kind words about Repatha. I actually led development of Repatha at Amgen.

So I appreciated the dialogue that you had regarding patient access rather than drug pricing. I think that's the right way to think about it, right? Drug pricing is one piece of the puzzle, but it's really patient access that's the issue. So I'd like to hear your thoughts on what PhRMA plans to do to get that message out there. I mean, look, the public, they're not fans of insurance companies. But yet we're not hearing any rhetoric about this. So what are PhRMA's plans to do that, as well as what can the industry do better? But secondly, also, what can we do to really change this dialogue about price, price, price, but patient access and value?



MR. UBL: Mmm hmm, great question. And appreciate the fact that you're getting into the arena and running for office.

You know, I think what we're going to be doing at PhRMA is really focusing again on some of these pragmatic steps that we think we should take and that are – again, these are not expensive things, they're not things that are politically polarizing. They're steps that could be taking to bring us to a more value-based discussion. And so we're going to continue to drive on, you know, the FDA reforms, moving to value-based purchasing, consumer-oriented information, hopefully distorting some of the marketplace distortions that exist today. You know, the reality is you've got a patchwork of guaranteed rebate mechanisms that are in place. You've got a 340-B program that has grown exponentially over time, even as the uninsured population has diminished.

So those things all contribute to the political discourse and dynamic. So we think we have to continue to educate policymakers inside the beltway and out about the value that our products are bringing. We're going to do that more aggressively in the months ahead. And we're going to drive a pragmatic agenda. And we're also going to, you know, look at the system as a whole. You know, to your point, I mean, quality of insurance is a real issue that impacts a wide variety of stakeholders – the hospital industry, the PBM industry. We have to start asking questions about, you know, the rebates that our companies are providing. Are they flowing to patients? You know, these are legitimate questions that we have to explore and really try to reorient the debate away from one small set of inputs that are a relatively small portion of overall health care spending to system-wide changes that can drive us towards a value-based system.

MR. ABRAHAMAS: Please joining me in thanking – (inaudible). (Applause.) On behalf of PMC, I'd like to thank Steve Ubl for his leadership, for his support of the Personalized Medicine Coalition, and most importantly for PhRMA's support of personalized medicine. So I'd also like to thank the audience for coming. And I hope we'll see you during the year at many of our policy meetings, at our conference at Harvard Medical School in November. But please stay engaged. It's not going to happen by itself. So thank you very much. (Applause.)

(END)