



CONFERENCE SUMMARY

An Overview of Challenges and Solutions in Science,
Business and Policy

12TH ANNUAL

○ ○ ○ **PERSONALIZED**
○ ○ ● **MEDICINE**
● ● ● **CONFERENCE**

November 15–17, 2016 · Harvard Medical School

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“What we need is a national conversation of experts. Across the industry, across government, the best people in this country to come together to tell us how to maximize value for patients. That is the big challenge in our future.”

— Mark Levin, Partner, Third Rock Ventures

Introduction

In November 2016, nearly 500 of the world's leading researchers, investors, industry professionals, policy experts, payers, clinicians and patient advocates convened at Harvard Medical School during the 12th Annual Personalized Medicine Conference to discuss the science, business and policy issues facing the emerging field of personalized medicine.

The conversations, spanning three days and including 18 sessions, revealed that although the personalized medicine landscape is fraught with challenges, the opportunity to improve upon one-size-fits-all medicine has, in some instances, driven personalized medicine all the way to the point of care. Participants agreed on the need for solutions, especially to ongoing challenges regarding regulation and reimbursement. Inspired by bipartisan support for the field, most remain optimistic about the field's future and potential.



- The 12th Annual Personalized Medicine Conference took place in the Joseph B. Martin Conference Center at Harvard Medical School.

Setting the Standard

Overcoming One-Size-Fits-All



“Standard of care is just not good enough.”

— Michael J. Pellini, M.D., Chairman, Board of Directors, Foundation Medicine; Board Member, Personalized Medicine Coalition

Recognizing that a significant percentage of patients does not respond to initial treatment, most stakeholders agree that the one-size-fits-all treatment paradigm needs improvement. Conference participants noted, however, that clinicians still typically explore personalized medicine only as a last resort, and that the chances of survival are therefore often drastically reduced before targeted therapies are prescribed. Many participants acknowledged that changing the status quo will be difficult.

“The best change will come when precision medicine becomes common practice, not just a specialty,” Raju Kucherlapati, Ph.D., Paul C. Cabot Professor of Genetics, Harvard Medical School, explained after accepting PMC’s 2016 Leadership in Personalized Medicine Award.

Despite the challenges associated with changing clinical practice, personalized medicine’s proponents, who include national and international leaders from the scientific and policy communities, noted that the field holds great promise for both patients and the health care system.

“Precision medicine, if used properly, would be enormously beneficial to public health,” said Victor Dzau, M.D., President, National Academy of Medicine.

Defining Success

A Patient's Perspective



“I know what it feels like when [personalized] health care works.”

— Eric Dishman, Director, *All of Us* Research Program, National Institutes of Health

The field's stakeholders are especially encouraged by personalized medicine's ability to improve patient outcomes. Eric Dishman, Director, *All of Us* Research Program, National Institutes of Health, illustrated the power of the patient's perspective on the second day of the conference. Speaking as a patient, Dishman explained that his experience being treated as a statistical average constantly undermined his lifelong battle with a misdiagnosed cancer. After decades, he was only able to beat his cancer when his medical team developed a treatment plan that was personalized for his goals and tailored to his genome.

“The cost of imprecision is huge on the bodies and souls of patients and families who have to go through trial-and-error [medicine],” he said. Dishman expressed his hopes for the *All of Us* Research Program, through which scientists will leverage data from one million volunteers to identify precision treatments for a range of diseases and conditions. That effort, he said, will help the country deliver on the promise of personalized medicine.

Envisioning Value

An Emerging Challenge



“We need a way of converging on a common formula [to measure value].”

— Peter Bach, M.D., M.A.P.P., Director, Center for Health Policy and Outcomes, Memorial Sloan Kettering Cancer Center

Although personalized medicine’s benefits for patients like Dishman are clear, many advocates contend that the field needs a universally accepted explanation of how personalized medicine adds value to the health care system overall. Stakeholders, however, define value differently, which complicates efforts to develop a single value proposition.

“We have an opportunity to advance value in health care, but we need to get past the status quo,” said Michael Sherman, M.D., M.B.A., Senior Vice President, Chief Medical Officer, Harvard Pilgrim Health Care, who is also a PMC board member.

Panelists who participated in the discussion about value agreed that patient advocates, physician groups, drug manufacturers and payers often struggle to find common ground when defining value, in part because patients are not as involved in the creation of value frameworks as they should be. In order to arrive at a common definition, panelists said, the community needs to adopt a more holistic view of the concept.

Addressing Uncertainty

The Diagnostics Debate



“We realized early on that our paradigm as to how we evaluate [diagnostic and laboratory-developed] tests was going to be seriously tested with next-generation sequencing.”

— Alberto Gutierrez, Ph.D., Director, Office of In Vitro Diagnostic Device Evaluation and Safety, U.S. Food and Drug Administration

The field’s stakeholders also recognize that the **extraordinary growth of the genetic testing space**, which now includes more than 60,000 products,¹ has posed challenges for the regulatory paradigm at the U.S. Food and Drug Administration (FDA). The regulatory pathway for laboratory-developed tests (LDTs) remains unclear, and ambiguity regarding the threshold of evidence required to support reimbursement of genetic tests continues to discourage investment in developing new and better diagnostics.

“For personalized medicine to thrive, we need standards for communicating the value of diagnostics,” explained Peer M. Schatz, M.B.A., CEO, QIAGEN.

Alberto Gutierrez, Ph.D., Director, Office of In Vitro Diagnostic Device Evaluation and Safety, FDA, indicated that the agency might reconsider its decision to finalize draft guidance on regulating LDTs.² With no solution readily apparent, conference participants made a commitment to working with FDA on a clear regulatory pathway for the diagnostics industry going forward. FDA formally announced its decision to delay final guidance the day after the conference ended.

1. NextGxDx. *How Many Genetic Testing Products Are There?* (#PMWC16). Accessed October 31, 2016, at <https://blog.nextgxdx.com/2016/01/24/how-many-genetic-testing-products-are-there-pmwc16-infographic/>.

2. Ray, T. FDA holding off on finalizing regulatory guidance for lab-developed tests. *GenomeWeb*. November 18, 2016.

Rethinking Reimbursement

The Payer Perspective



“[The top challenges facing personalized medicine are] reimbursement, reimbursement and reimbursement.”

— Alexis Borisy, Partner, Third Rock Ventures

With a significant number of payers speaking at the conference, attendees also spotlighted the need for clarity in relation to the kinds of evidence necessary for reimbursement. Payers noted that they rely on evidence published in peer-reviewed literature when making reimbursement decisions, and emphasized their willingness to work with industry to advance personalized medicine where appropriate. Although the dialogue did not define any clear partnership models, many participants saw payers’ willingness to engage in the dialogue as a first step toward an improved reimbursement landscape for the field. Some payers acknowledged that personalized medicine tests pose challenges for traditional reimbursement models.

“The complexity [of diagnostic tests] has grown tremendously, and what we’ve realized is that this is a different beast,” said Matthew Fontana, M.D., Vice President, Chief Medical Officer, Pharmacy, Health Care Service Corporation. “It needs to be treated differently.”

Personalizing Care

Successful Strategies



“[We’re] not talking about something that may happen someday. [We’re] talking about what’s happening now.”

— Howard McLeod, Pharm.D., Medical Director,
DeBartolo Family Personalized Medicine Institute,
Moffitt Cancer Center

The conference also illuminated how, despite ongoing regulatory and reimbursement challenges, the health care community is finding ways to integrate personalized medicine into clinical care. Participants from hospital systems elucidated strategies for overcoming challenges in physician education, patient empowerment, value recognition, managing data and access to care.

Lincoln Nadauld, M.D., Ph.D., Executive Director, Precision Medicine and Precision Genomics, Intermountain Healthcare, noted, for example, that Intermountain employs a team of reimbursement experts to work directly with each patient’s insurance provider to facilitate access to targeted therapies, while Amy Abernethy, M.D., Ph.D., Chief Medical and Scientific Officer, Flatiron Health, emphasized the importance of an infrastructure that makes data available to physicians at the point of care. PMC’s Health Care Working Group, led by Daryl Pritchard, Ph.D., Vice President, Science Policy, PMC, will publish a peer-reviewed essay early in 2017 that lists 39 specific strategies for integrating personalized medicine into the clinic.

Pioneering Precision

A Glimpse of the Future



“It’s not really ‘should we do this.’ We have to do this. We don’t get to decide what the biology of these diseases are, we just have to work with it.”

— Barbara Weber, M.D., Interim Chief Medical Officer, Neon Therapeutics

Unencumbered by the field’s policy challenges, scientific experts pointed out that heterogeneous disease biology necessitates the move toward personalized medicine. To demonstrate how the scientific community is breaking new ground, company representatives presented the latest research in neoantigen biology for cancer immunotherapy and steropure nucleic acid therapeutics in rare genetic disease treatment, among other areas.

“Sometimes personalization is a step on a path rather than the end state,” said David Altshuler, M.D., Ph.D., Executive Vice President, Global Research, Chief Scientific Officer, Vertex.

Conclusion



“The most essential element of leadership is the ability to describe a vision of the future so compelling that others will gladly join you in making the vision a reality.”

— William S. Dalton, Ph.D., M.D., CEO, M2Gen;
Director, DeBartolo Family Personalized Medicine
Institute, Moffitt Cancer Center; Board Member,
Personalized Medicine Coalition

The policy challenges for personalized medicine are **real and complex**, but the science behind the field continues to drive targeted therapies to the market. An appropriate, well-defined regulatory environment for diagnostics and a clear business model for developing personalized medicine products that are reimbursed by payers would accelerate medicine’s evolution toward personalized medicine. The scientific and business communities, inspired by the promise of improved patient outcomes and a more efficient health care system, believe they can and will overcome the challenges facing the field. Daniel O’Day, M.B.A., CEO, Roche Pharmaceuticals, aptly summarized that determination during a fireside chat with CNBC Reporter Meg Tirrell on the final day of the conference.

“At the end of the day, under any government system around the globe, if you bring a transformational medicine that cures a subset of patients or transforms a patient’s experience with a disease, I think it finds its way through any government system to be supported,” he said.

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About Us

The Personalized Medicine Coalition (PMC), representing innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.



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SAVE THE DATE

13th Annual Personalized Medicine Conference
November 14–16, 2017



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1710 Rhode Island Ave., NW · Suite 700 · Washington, DC 20036
202.589.1770 · pmc@personalizedmedicinecoalition.org