



THE 14TH ANNUAL

# PERSONALIZED MEDICINE CONFERENCE

Preparing for the New Possible

CONFERENCE SUMMARY

November 14–15, 2018 • Harvard Medical School



“We have science and medicine and technologies that are far outpacing a structure that was really set up in a completely different era.”

— Peter Juhn, M.D., M.P.H., Global Head of Value-Based Partnerships, Amgen

# Introduction

Academics, clinicians, industry representatives, patient advocates, payers, and policymakers, among others, agree that the latest breakthroughs in medicine are challenging health systems around the world.

In November of 2018, nearly 500 academics, clinicians, industry representatives, patient advocates, payers, policymakers, and others involved in the delivery of health care convened at the 14<sup>th</sup> Annual Personalized Medicine Conference at Harvard Medical School to discuss the science, business, and policy issues facing the evolving field of personalized medicine.

During a panel discussion on the second day of the conference, Amgen Global Head of Value-Based Partnerships Peter Juhn, M.D., M.P.H., aptly summarized participants' perspectives about the landscape for the field, noting, "We have science and medicine and technologies that are far outpacing a structure that was really set up in a completely different era."

The science, participants agreed, has delivered extraordinary opportunities to predict, prevent, and treat disease in previously unimaginable ways by leveraging more efficient genomic sequencing technologies, artificial intelligence, digital health, and advanced data analytics to maximize the impact of a powerful pipeline of personalized tests and treatments.

But participants also acknowledged the need to align outdated public policies and medical practices with the principles of the field.

They concluded that the speed at which health systems around the world integrate these new tools to realize the benefits of targeting increasingly impactful personalized treatments to only those patients who will benefit will depend largely on decision-makers' willingness to put in place supportive regulatory and reimbursement policies; prioritize studies about the clinical and economic utility of personalized health care; and adopt innovative reimbursement approaches that facilitate sustainable access to therapies that translate higher up-front costs into a more effective and efficient health system.



“Making sure that we target the patient population improves the transformational benefit of medicines.”

— Daniel O’Day, CEO, Roche Pharmaceuticals

# Targeting Treatment

Pharmaceutical companies are increasingly developing highly personalized therapies that are designed to treat only the small subsets of patients who will reap their unprecedented benefits. Patients welcome this approach.

Pharmaceutical industry representatives and patient advocates stressed the urgent need for groundbreaking treatments that can dramatically inhibit or even cure deadly and costly diseases such as cancer, cystic fibrosis, and Alzheimer's disease. Participants agreed that the science underpinning personalized medicine presents opportunities to deliver those kinds of treatments.

Roche Pharmaceuticals CEO Daniel O'Day, for example, observed during a fireside chat with CNBC Reporter Meg Tirrell on the second day of the conference that the pharmaceutical industry is responding to a "world [that is] no longer prepared to accept marginally differentiated medicines" by developing highly personalized therapies that are designed to treat only the small subsets of patients who will reap their unprecedented clinical benefits.

"Making sure that we target the patient population improves the transformational benefit of medicines," O'Day explained.

Citing a PMC-commissioned study demonstrating that most patients prefer personalized medicine to the existing standard of care, *Genome* magazine Founder and Personalized Medicine Coalition Board Member Susan McClure said patients are excited about this new direction of pharmaceutical research. She and other participants advocated for the involvement of patients in every step of the process, noting that informed patients can drive modernized policies and practices that keep pace with scientific progress.

"Never underestimate a person's ability to grasp complex information when their life depends on it," McClure said.



“My concern is that the field  
cannot move fast enough.”

– Emily Kramer-Golinkoff, M.B.E., Co-Founder,  
Emily’s Entourage; cystic fibrosis patient

# Expanding Frontiers

Although some people are already benefitting from personalized medicine, many are still reliant on one-size-fits-all approaches. Continued investments in science and increased awareness of developments in the field will help accelerate progress.

Patients noted that although some people are already benefitting from extraordinarily impactful targeted treatments, many are still reliant on standard, one-size-fits-all therapies that fail to help a significant percentage of those who are treated with them.

In cystic fibrosis, for example, Emily Kramer-Golinkoff, M.B.E., a patient advocate who in 2011 co-founded Emily's Entourage, a nonprofit that raises money and awareness to help find a cure for forms of cystic fibrosis caused by rare, "nonsense" mutations, noted that scientific research has now demonstrated that the disease can be caused by more than 2,000 genetic variations. This research has ushered in a new, personalized standard of care in which 90 percent of patients can benefit from a targeted therapy.

But Kramer-Golinkoff, unfortunately, is part of the 10 percent of cystic fibrosis patients for whom no targeted therapy is available.

Participants advocated for collaborative efforts to accelerate progress.

"I have a lot of faith in science," Kramer-Golinkoff said. "My concern is that the field cannot move fast enough."

Bryce Olson, a prostate cancer patient, noted that even when personalized medicine options do exist, lagging awareness about the field among providers and patients means many patients never learn about them. Reflecting on his own health care journey, he noted that he did not find out about personalized medicine until he had exhausted the other possibilities. He encouraged other patients to ask their doctors about the field.

"We need to demand [personalized medicine]," Olson said.



“The barriers [to developing diagnostics] are really more around putting [policies] in place that support the science that underlies the field.”

— Kimberly Popovits, Chairman of the Board,  
CEO, President, Genomic Health; Board Member,  
Personalized Medicine Coalition



# Developing Diagnostics

Unclear regulatory and reimbursement policies are inhibiting efforts to commercialize the diagnostics that guide treatment to only those patients who will benefit.

Investors and diagnostics industry representatives emphasized that although technological developments such as the decreasing cost of genomic sequencing underlie powerful new diagnostics that can guide treatments to only those patients who will respond to them, unclear regulatory and reimbursement policies are inhibiting efforts to bring those diagnostics to the market so that patients can benefit.

“The barriers [to developing diagnostics] are really more around putting [policies] in place that support the science that underlies the field,” explained Personalized Medicine Coalition Board Member Kimberly Popovits, who is Chairman of the Board, CEO, and President of Genomic Health.

Participants noted that the scientific discoveries underpinning personalized medicine are informing the development of increasingly sophisticated laboratory-developed tests (LDTs). The ways in which these new tests should be regulated and reimbursed remains an open question, however, an uncertainty that has made investors wary of betting on new diagnostic tools to guide treatments.

Julie Khani, M.P.A., President, American Clinical Laboratory Association (ACLA), and Lauren Silvis, J.D., Chief of Staff, Immediate Office of the Commissioner, FDA, both voiced support for a legislative solution that would facilitate a modernized, predictable regulatory framework going forward.

In relation to reimbursement, participants recognized that the implementation of the Protecting Access to Medicare Act (PAMA) in the United States has resulted in decreased payment rates for some key personalized medicine tests, and noted that many diagnostics companies do not have the resources to satisfy the evidentiary requirements of multiple commercial payers, whose coverage policies can vary significantly.



“Unless we come to some agreement among stakeholders about ... questions of assessing value, we will have a difficult time realizing the full potential of personalized medicine.”

– David P. King, J.D., Chairman, CEO, LabCorp

# Assessing the Approach

Definitive studies that demonstrate the clinical and economic value of personalized health care strategies in various disease states will help facilitate improved patient access to personalized medicine by convincing payers and providers to align their policies and practices with the field's principles.

Industry representatives, payers, clinicians, and researchers agreed that definitive studies that demonstrate the clinical and economic value of personalized health care strategies in various disease states will help facilitate improved patient access to personalized medicine by convincing payers and providers to align their policies and practices with the field's principles. Participants said these studies must deliver evidence that is equally compelling to patients, payers, and providers, all of whom play an essential role in the development and implementation of personalized prevention and treatment plans.

“Unless we come to some agreement among stakeholders about ... questions of assessing value, we will have a difficult time realizing the full potential of personalized medicine,” LabCorp Chairman and CEO David P. King, J.D., said in his keynote address during the second day of the conference.

Early studies, participants noted, suggest that some of the most advanced genomic sequencing tests already show moderate clinical and economic utility, but could be much more impactful if clinicians used the test results more consistently to match patients with personalized treatments. Many believe artificial intelligence can help alleviate this “practice gap.”

“The discussion around A.I. is going to be vital if this is going to become really useful in the future, because physicians cannot keep up with the [medical literature],” said Kristine Bordenave, M.D., F.A.C.P., Corporate Medical Director, Humana.



“[Many personalized therapies] have a very high cost [and] a very high impact, but they don’t work for everyone. We need to figure out how to pay commensurate with the results.”

— Michael Sherman, M.D., M.B.A., M.S., Chief Medical Officer, Senior Vice President, Harvard Pilgrim Health Care; Board Member, Personalized Medicine Coalition

# Rethinking Reimbursement

To facilitate patient access to personalized therapies that translate higher up-front costs into unprecedented downstream benefits, industry representatives, payers, and policymakers must develop modernized, ‘value-based’ reimbursement models.

Industry representatives, investors, patients, and payers underlined the need for modernized reimbursement policies that incentivize the development of personalized therapies.

Many participants expressed concern that increasingly cost-conscious policymakers may enact sweeping policies that drive down the reimbursement rates for all therapies regardless of how they may benefit patients and society, thereby discouraging the development of personalized therapies that are designed to translate higher up-front costs into a more efficient and effective health system by targeting treatments to only those patients who will benefit from them. Participants advocated instead for “value-based” reimbursement arrangements that tie payments to patient outcomes.

“[Many personalized therapies] have a very high cost [and] a very high impact, but they don’t work for everyone,” said Personalized Medicine Coalition Board Member Michael Sherman, M.D., M.B.A., M.S., who serves as Chief Medical Officer and Senior Vice President of Harvard Pilgrim Health Care. “We need to figure out how to pay commensurate with the results.”

During her opening keynote address, Brigham Health President Elizabeth Nabel, M.D., confirmed that insufficient reimbursement rates associated with the delivery of the highly personalized chimeric antigen receptor (CAR) T-cell therapies that can deliver lasting benefits to cancer patients in just a few doses are already discouraging smaller provider institutions from facilitating access to them. She said providers are currently subsidizing the cost of administering CAR T-cell therapies, an arrangement she described as “not sustainable” even for academic medical centers.



“We have to change the system  
[if we want] to cure disease.  
I don’t think it’s a choice we  
have. It just has to be fixed.”

– Salveen Richter, C.F.A., Vice President, Research Division,  
Goldman Sachs

# Conclusion

Patients will increasingly demand inclusive solutions to the systemic challenges that threaten to discourage the development of the personalized tests and treatments they want and need.

Participants concluded that because the science underpinning personalized medicine is poised to deliver the kinds of transformational health benefits patients want and need, patients will continue to demand inclusive solutions to the systemic challenges that threaten the field going forward. The onus, they said, is on decision-makers to facilitate the advancement of the field — sooner rather than later.

“We have to change the system [if we want] to cure disease,” said Salveen Richter, C.F.A., Vice President, Research Division, Goldman Sachs. “I don’t think it’s a choice we have. It just has to be fixed.”

Participants emphasized that patients from diverse backgrounds must be involved in the solutions that move the field forward.

“[Personalized medicine] is applicable to all of us, therefore all of us need to be [involved], including people who have historically been excluded,” explained Personalized Medicine Coalition Board Member Amy Abernethy, M.D., Ph.D., Chief Medical Officer, Chief Scientific Officer, Senior Vice President, Oncology, Flatiron Health. “[We will need] to be intentional to make that happen.”

Looking ahead, policymakers from around the world were optimistic about their ability to capitalize on the opportunities the field presents.

“This is not a big deal,” Liisa-Maria Voipio-Pulkki, M.D., Ph.D., Director General, Chief Medical Officer, Ministry of Social Affairs and Health, Finland, contended. “This is just a revolution.”

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# About the Conference

The Annual Personalized Medicine Conference defines the landscape and outlook for personalized medicine in science, business, and policy.

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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.





# SAVE THE DATE

15<sup>th</sup> Annual Personalized Medicine Conference  
**November 13–14, 2019**

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