Preparing for the New Possible: A Summary of the 14th Annual Personalized Medicine Conference at Harvard Medical School

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But participants also acknowledged the need to align outdated public policies and medical practices with the principles of the field.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

Photo courtesy of the Personalized Medicine Coalition

The following summary of the *14th Annual Personalized Medicine Conference: Preparing for the New Possible* draws on these conversations to outline the evolving landscape for personalized medicine and describe several emerging solutions that will help pave the way for a new era in medicine that benefits both patients and health systems.

Targeting Treatments

Pharmaceutical industry representatives and patient advocates who attended the conference 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.

Gilead Chairman and CEO Daniel O'Day (then-CEO, Roche Pharmaceuticals) 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 study commissioned by the Personalized Medicine Coalition demonstrating that most patients prefer personalized medicine to the existing standard of care, *Genome* magazine Founder and 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.

Expanding Frontiers

Patients also 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.

Emily Kramer-Golinkoff, M.B.E., is 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. Kramer-Golinkoff 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.

Photo courtesy of the Personalized Medicine Coalition

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

Developing Diagnostics

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 responder populations, 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, 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 in the United States has resulted in decreased payment rates for some key personalized medicine tests. Participants also raised the point that many diagnostics companies do not have the resources to satisfy the evidentiary requirements of multiple commercial payers, whose coverage policies can vary significantly.

Assessing the Approach

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.

Rethinking Reimbursement

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. Such policies may well discourage 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."


David P. King, J.D.

Photo courtesy of the Personalized Medicine Coalition

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.

Conclusion

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 former Personalized Medicine Coalition Board Member Amy Abernethy, M.D., Ph.D., who recently left her position as Chief Medical Officer, Chief Scientific Officer and Senior Vice President for Oncology at Flatiron Health to serve as Principal Deputy Commissioner for Food and Drugs at FDA. "[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." ■