



THE 13TH ANNUAL

PERSONALIZED MEDICINE CONFERENCE

From Concept to the Clinic

CONFERENCE SUMMARY

November 14-16, 2017 • Harvard Medical School



“[I am] in awe of the precision medicine undertaking and what it has accomplished. At the same time, [I am] shocked that we have been unable to move much, much faster.”

– Jay T. Flatley, Executive Chairman, Illumina

Introduction

Leaders in personalized medicine are ‘in awe’ of the field’s potential but ‘shocked’ by the incremental pace of progress in regulation, reimbursement and clinical adoption.

In November of 2017, nearly 500 of the world’s leading researchers, investors, industry executives, policy experts, payers, clinicians and patient advocates convened at Harvard Medical School during the 13th Annual Personalized Medicine Conference to discuss the science, business and policy issues facing the evolving field of personalized medicine.

Shortly after he accepted the 13th Annual Leadership in Personalized Medicine Award on the first day of the conference, Jay T. Flatley, Executive Chairman, Illumina, aptly summarized participants’ perspectives on personalized medicine as he described his “shock and awe” at the state of the field.

Many, he said, are “in awe” of the field’s potential. Participants noted that scientific and technological developments in areas like next-generation sequencing, gene therapy and gene editing, for example, anticipate a new era in which doctors can find the most effective treatment for a patient much sooner based on a more complete understanding of the molecular mechanisms that cause each patient’s disease.

Many conference participants, however, in Flatley’s formulation, are “shocked” by incremental progress in regulation, reimbursement and clinical adoption even as the science has raced forward. Outdated policies and practices, they note, are inhibiting the health system’s ability to commercialize personalized diagnostics, evaluate personalized treatments and help providers move toward medical paradigms in which treatment decisions are based on the specific characteristics of each patient instead of – as is most often the case today – on population averages.



“[Scientists] can now ask ‘If we only had to [treat a patient] one time, what would it allow?’”

— Jeffrey D. Marrazzo, M.B.A., M.P.A., CEO,
Spark Therapeutics

Breaking The Mold

One-time, highly personalized treatments have begun to challenge the constructs of health systems that are optimized to facilitate access to daily, one-size-fits-all maintenance medications.

Industry executives, scientists and bioethicists stressed that the one-time, highly personalized treatments made possible by developments in gene therapy and clustered regularly interspaced short palindromic repeats (CRISPR), which could have unprecedented clinical value, have begun to challenge the existing constructs of health systems, which are still optimized to facilitate access to daily, one-size-fits-all maintenance medications.

Participants noted that existing regulatory and reimbursement policies do not account for the possibility that a single intervention at a specified point in time may continue to deliver value over the course of an entire lifespan. But they agreed that finding ways to make the treatments accessible to patients is imperative to the future of medicine.

“It’s the patients who are waiting, and I think their moral pull is pretty strong,” said Arthur L. Caplan, Ph.D., Drs. William F. and Virginia Connolly Mitty Chair, Director, Division of Medical Ethics, New York University Langone Medical Center.

Thomas J. Lynch, Jr., M.D., Executive Vice President, Chief Scientific Officer, Research & Development, Bristol-Myers Squibb, added that emerging evidence about the value of combination treatment regimens would also transform the clinical paradigms currently shaping the use of targeted therapies in oncology.

“Understanding how to combine multiple targeted treatments, I think, is how we will make a difference,” Lynch said.



“We think a lot about access [to diagnostics], and it’s not just reimbursement as a buzz word.”

— Jacob S. Van Naarden, Chief Business Officer,
Loxo Oncology

Commercializing Diagnostics

Unclear regulatory requirements and inconsistent reimbursement practices are limiting the number of personalized medicine tests available to patients.

Both diagnostic and pharmaceutical industry representatives voiced concerns over the ongoing challenges associated with commercializing next-generation sequencing tests, which can dramatically improve the quality of cancer care by evaluating a patient's candidacy for multiple personalized treatment options at once. Because of unclear regulatory requirements and inconsistent reimbursement practices for next-generation sequencing options, they noted that in most cases providers who want to consider targeted treatments must either assess a patient's candidacy for one option at a time through separate FDA-approved companion diagnostics or order a laboratory test that is not FDA-approved and probably not covered under the patient's insurance plan. In both cases, the patient's disease can wreak havoc before the results are returned.

"We think a lot about access [to diagnostics], and it's not just reimbursement as a buzz word," said Jacob S. Van Naarden, Chief Business Officer, Loxo Oncology.

To mitigate the challenges, patient and industry representatives advocated for a more transparent and efficient regulatory pathway for these tests as well as a comprehensive review of existing reimbursement practices in the public and private sectors – both of which have been priorities for proponents of personalized medicine since the field's inception more than a decade ago.

Sean Khozin, M.D., M.P.H., Acting Associate Director, Oncology Center of Excellence, FDA, indicated that the agency is now piloting a new pre-certification regulatory pathway for next-generation sequencing tests that is designed to streamline regulatory requirements by focusing oversight efforts on test manufacturers.

"There's a shift that's occurring right now," Khozin said. "We need to, in the appropriate situations, turn the focus away from the product itself and look at the manufacturer."



“Patients take a much longer term approach to their treatments and the related decisions than is captured in [current methodologies].”

— Andrea Stern Ferris, M.B.A., President, Chairman of the Board, LUNGeivity Foundation

Evaluating Therapies

A lack of consensus on methods for determining the economic value of personalized treatment approaches threatens to lock promising options in perpetual reimbursement negotiations — where patients cannot access them.

Conversations among patient advocates, industry representatives, payers and the developers of so-called value assessment frameworks that sometimes influence coverage decisions reflected a lack of consensus on the most appropriate methods for determining the economic value of personalized treatment options, suggesting that a consistent reimbursement landscape whereby large populations of patients can immediately access the next generation of personalized therapies is not a given.

“There’s actually teeth to value frameworks,” said Robert Dubois, M.D., Ph.D., Executive Vice President, Chief Science Officer, National Pharmaceutical Council. “Payers use these to decide what patients get access to.”

Participants agreed that the advent of gene therapy and gene editing, which promise to deliver unprecedented medical value through one-time personalized treatments, will be especially challenging for payers given that health systems have been designed to pay for daily maintenance medications. Others noted that value assessment frameworks will likely play an increasingly important role in coverage and payment decisions, and stressed the importance of ensuring that the next generation of frameworks can be tailored to reflect patient preferences.

“Patients take a much longer term approach to their treatments and the related decisions than is captured in [current methodologies],” said Andrea Stern Ferris, M.B.A., President, Chairman of the Board, LUNgevity Foundation. “They think ‘how am I going to live three years, five years — how am I going to string things together?’ How do you capture that in the current models?”

Michael Sherman, M.D., M.B.A., M.S., Chief Medical Officer, Senior Vice President, Harvard Pilgrim Health Care; Member, Board of Directors, Personalized Medicine Coalition, noted that a willingness on behalf of pharmaceutical companies to participate in outcomes-based contracts can sometimes facilitate expanded access to personalized medicines, while Steven Pearson, M.D., M.Sc., Founder, President, Institute for Clinical and Economic Review (ICER), welcomed thoughts on how to improve ICER’s value assessment framework so that it reflects the goals of personalized medicine.



“It’s one thing to talk about personalized medicine. It’s another to have your health system invest in it.”

— Howard L. McLeod, Pharm.D., Medical Director, The DeBartolo Family Personalized Medicine Institute, Chair, Department of Individualized Care Management, Senior Member, Division of Population Sciences, Moffitt Cancer Center; Member, Board of Directors, Personalized Medicine Coalition

Personalizing The Clinic

Challenges in education, patient empowerment, value demonstration, IT and access are slowing the pace at which health systems are integrating personalized medicine into clinical practice.

Provider representatives stressed that incorporating personalized medicine technologies into clinical work streams requires substantial investments of time and money to address complex challenges in education, patient empowerment, value demonstration, IT and access. As a result, participants noted that many community hospital systems, with fewer resources, have been reluctant to embrace the new paradigm.

“It’s one thing to talk about personalized medicine,” explained Howard L. McLeod, Pharm.D., Medical Director, DeBartolo Family Personalized Medicine Institute, Chair, Department of Individualized Cancer Management, Senior Member, Division of Population Sciences, Moffitt Cancer Center; Member, Board of Directors, Personalized Medicine Coalition. “It’s another to have your health system invest in it.”

Senior leaders from Mission Health and Vanderbilt University School of Medicine advocated for the continued availability of grant funding to support initial infrastructure investments and mitigate the costs of patient participation, while clinical experts recognized molecular tumor boards as a valuable analytical and educational tool. Provider representatives also agreed that the success of integration efforts depends in part on clearly articulating the shortcomings of one-size-fits-all medicine to all relevant staff members, and Lincoln Nadauld, M.D., Ph.D., Executive Director, Precision Medicine and Precision Genomics, Intermountain Healthcare; Member, Board of Directors, Personalized Medicine Coalition, added during a case study presentation that the shortcomings of the current standard of care had also served as the impetus for Intermountain’s participation in a multi-stakeholder data-sharing initiative.



“We are going to need a collaborative effort [for personalized medicine] to help people like me.”

– Bryce Olson, Global Marketing Director, Health and Life Sciences Group, Intel Corporation; Stage 4 Prostate Cancer Patient

Conclusion

Cross-sector collaboration represents the most promising model for accelerating the pace of progress.

Bryce Olson, a stage 4 prostate cancer patient and Global Marketing Director, Health and Life Sciences Group, Intel Corporation, aptly summarized participants' conclusions about the future of the field during the second day of the conference.

“We are going to need a collaborative effort [for personalized medicine] to help people like me,” Olson said.

Participants concluded that tackling the challenges associated with commercializing diagnostics, evaluating therapies and personalizing clinical care will require innovative partnerships. They noted, for example, that in 2017 a diagnostic company had teamed up with three different pharmaceutical companies to secure regulatory approval for a next-generation sequencing test; a drug company had collaborated with a health insurance company on a risk-sharing payment agreement in which the company pays for a personalized drug only when the drug works; and several clinical institutions had collaborated with an IT company to organize a data-sharing initiative that helps providers make more informed treatment decisions and match patients to clinical trials. Additional cross-sector efforts, participants agreed, would help facilitate a health system that is more proactive and effective.

“We are still in a break it and fix it health care system,” said Joshua Ofman, M.D., M.S.H.S., Senior Vice President, Global Value, Access and Policy, Amgen. “We need to make the transition to a predict, prevent and protect health system.”

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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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14th Annual Personalized Medicine Conference
November 13–15, 2018

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