



Strategic Plan

2020

MISSION

The Personalized Medicine Coalition, 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. Believing that paradigm shifts, especially in medicine, do not happen just because the science and new technologies suggest they should, PMC supports investment in and adoption of personalized medicine through education, advocacy, and evidence development.

SUMMARY

The number of personalized treatments on the market, which PMC uses as a proxy for progress in personalized medicine, has increased dramatically since 2008, when there were five personalized medicines available. Today there are more than 170 personalized therapies on the market in the U.S., a number that continues to grow as the Food and Drug Administration rapidly approves more tests and treatments that can facilitate more effective and efficient health care based on the use of diagnostic tests that determine which medical treatments will work best for each patient. By combining the data from those tests with an individual's history, circumstances, and values, health care providers can develop targeted treatment and prevention plans.

To help ensure that increasingly cost-conscious decision-makers in Congress, at government agencies, and in the private sector continue to pursue personalized health care that can translate up-front costs into a more effective and efficient health system, PMC will in 2020 leverage its credibility as an international, multi-stakeholder organization focused on:

- **Education:** To raise the profile of personalized medicine, PMC will proactively educate decision-makers about how the field can make the health system more effective and efficient by targeting treatments to only those patients who will benefit.
- **Advocacy:** To help facilitate the development of personalized medicine tests and therapies, PMC will advocate for continued investments in biomedical research as well as modernized regulatory, coverage, and payment policies that support access to personalized medicine.
- **Evidence Development (Sponsored Research):** To encourage the swifter adoption of personalized medicine by health care providers, PMC will help build the evidence base necessary to demonstrate the clinical and economic value of personalized medicine.

EDUCATION

To raise the profile of personalized medicine, PMC will proactively educate decision-makers and the public about how the field can make the health system more effective and efficient by targeting longer lasting treatments to only those patients who will benefit.

The educational activities described below also help PMC's members and other stakeholders track and tackle key challenges facing the field.

- ***Personalized Medicine at FDA: A Progress & Outlook Report*** will demonstrate that FDA is increasingly focused on advancing personalized medicine based upon the field's potential to improve care for patients and the health system. It will also explore the outlook for continued progress.
- ***The Personalized Medicine Report: Opportunity, Challenges, and the Future*** will demonstrate how available personalized medicine products and services can make patients healthier and lower systemic costs. The report will also outline the challenges facing the field.
- **A Minimum of Two Briefings on Capitol Hill** will underline the benefits of personalized medicine as a new Congressional Personalized Medicine Caucus begins to consider its priorities in health care.
- **The PMC Member Update**, an electronic progress report disseminated monthly to 2,000+ leaders in health care, tracks key trends shaping the landscape and outlook for the evolving field.
- ***Personalized Medicine in Brief***, published bi-annually in print and as a PDF, presents an overview of the status of personalized medicine and PMC's efforts to advance it.
- ***The 16th Annual Personalized Medicine Conference at Harvard Medical School*** will convene the world's leading researchers, investors, industry executives, policy experts, payers, clinicians, and patient advocates to define the landscape and outlook for personalized medicine in science, business, and policy.
- ***The 16th Annual State of Personalized Medicine Luncheon Address*** will update decision-makers on progress in personalized medicine by bringing a leader in health care to the National Press Club to make a public statement about the significance of the field.
- **PMC Representation at Prominent Industry Conferences** will help ensure that personalized medicine topics remain a focal point for industry representatives. In addition to accommodating ad-hoc speaking requests, PMC will again co-organize the "PMC/BIO Personalized Medicine & Diagnostics Track" at the *2020 BIO International Convention* and partner with the Cambridge Healthtech Institute on key sessions at the *2020 Tri-Molecular Medicine Conference* in San Francisco.
- ***Personalized Medicine 101: An Educational Campaign*** will underline the benefits of personalized medicine by distributing fact sheets to members of the Congressional Personalized Medicine Caucus and their staff; publishing articles about the field as part of educational inserts in the *USA Today*; and developing a downloadable slide deck that PMC's network of 6,000+ leaders in personalized medicine may use to communicate about the field.
- **PMC's Engagements With International Consortia**, including the International Consortium for Personalised Medicine and the World Economic Forum, will keep the Coalition informed about efforts to advance personalized medicine around the world and allow PMC to keep other countries apprised of progress in the U.S. PMC will share insights about the international landscape with its members as often as possible.

ADVOCACY

To help facilitate the development of personalized medicine tests and therapies, PMC will advocate for continued investments in biomedical research as well as modernized regulatory, coverage, and payment policies that support access to personalized medicine.

- **Cultivating Support for Personalized Medicine:** Public and private sector support for initiatives that drive progress in personalized medicine is essential to the future of the field.
 - Shaping an Agenda for the Congressional Personalized Medicine Caucus: After inspiring the formation of a Congressional Personalized Medicine Caucus through its advocacy on Capitol Hill last year, PMC will play a leading role in shaping an agenda for the caucus in 2020.
 - Increasing Available Research Funding: Investments in scientific research conducted by the National Institutes of Health drive many of the discoveries that underpin personalized prevention and treatment strategies. Through congressional testimonies, public statements, and meetings with members of Congress, PMC will actively advocate for increases to the NIH budget for FY 2021.
 - Setting Priorities for Patient-Centered Research in Personalized Medicine (funded by PCORI): To ensure that the principles of personalized medicine are incorporated into health research, PMC, using an award from the Patient-Centered Outcomes Research Institute (PCORI), will continue to develop a set of patient-centered principles to advance research underpinning personalized medicine.
- **Modernizing Regulatory Policies:** By approving and clearing personalized medicine products and services in an efficient manner, FDA facilitates patient access to the tests and treatments that make personalized health care possible. PMC will continue its efforts to ensure that legislation, emerging guidances for stakeholders, policies, and major initiatives preserve and improve the regulatory landscape for the field.
 - Resolving FDA's Concerns About Drug-Gene Interactions Referenced in Pharmacogenetic Test Reports: Recognizing FDA's increased interest in the strength of the evidence underpinning references to certain drug-gene interactions included in pharmacogenetic test reports, PMC will explore how it might collaborate with FDA and other stakeholders to resolve the agency's concerns in a way that does not stifle the development and integration of pharmacogenetic tests into clinical care.
- **Modernizing Coverage and Payment Policies:** To ensure that patients have access to personalized medicine, PMC advocates for flexible coverage policies and adequate payment rates for personalized medicine products and services. PMC will continue to work with the Centers for Medicare and Medicaid Services and private payers to inform strategies that can document the value of and facilitate access to personalized medicine, to include advocacy for arrangements based on patient-centered value assessments.
- **Modernizing Value Assessment Methodologies:** By influencing decisions made by patients, providers, payers, and policymakers, value assessment frameworks (VAFs) could accelerate or undermine progress in personalized medicine. PMC will continue to inform the development of VAFs and health technology assessments that account for the value of personalized medicine. PMC will also organize a webinar to demonstrate the importance of aligning assessment methodologies with the field's principles.
- **Encouraging Responsible Data Sharing:** Recognizing that consumers must trust institutions from various sectors of the health care ecosystem to share patient-provided data in responsible and transparent ways if the interventions underpinning personalized medicine are to be efficiently vetted and adopted in clinical settings, PMC will support the development of data-sharing principles that encourage institutions to share data in ways that do not jeopardize the health system's interest in engaging patients as fully informed research partners.

EVIDENCE DEVELOPMENT (SPONSORED RESEARCH)

To encourage the swifter adoption of personalized medicine by health care providers, PMC will help build the evidence base necessary to demonstrate the clinical and economic value of personalized medicine.

The projects described below will also help PMC's members and other stakeholders track the extent to which compelling evidence about the value of personalized medicine convinces health systems to adopt personalized prevention and treatment strategies.

- ***Defining the Clinical Utility of Genomic Testing in Cancer Care: A Roundtable Report*** will advance a more comprehensive definition of clinical utility in cancer care that can prompt more widespread clinical adoption of genomic testing technologies by highlighting underappreciated benefits of testing. The report will summarize conclusions developed in October 2019 by a PMC-organized roundtable of clinical guideline developers, clinical laboratories, patients, payers, and providers.
- ***The Integration of Personalized Medicine into US Health Systems: A Peer-Reviewed Research Article*** will explore through survey research and interviews the extent to which providers throughout the U.S. health care system are already integrating personalized medicine in clinical settings.
- ***The Cost-Effectiveness of Next-Generation Sequencing (NGS)-Based Diagnostic Tests for Patients With Melanoma: A Peer-Reviewed Research Article*** will inform efforts to integrate personalized medicine into clinical work streams by providing evidence about the cost-effectiveness of NGS when applied to patients with melanoma.
- ***Evaluating the Clinical and Economic Value of Sequencing-Based Diagnostic Tests for Patients With Rare and Undiagnosed Diseases*** will provide evidence for sequencing platform developers, payers, and providers about the clinical and economic utility of sequencing-based diagnostics when applied to patients with rare or undiagnosed diseases.
- ***Pharmacogenomics in Clinical Guidelines: A Landscape Analysis*** will benchmark clinical progress in personalized medicine by providing a list of the circumstances in which current clinical guidelines combined with the labels of FDA-approved therapies support the use of personalized medicine strategies that tailor care based on scientific knowledge about drug-gene interactions.
- ***The Utilization of Genomic Testing in the US: A Landscape Analysis***, developed in collaboration with Illumina, Concert Genetics, and the Blue Cross Blue Shield Association, would analyze the extent to which health systems are utilizing genomic tests in various contexts related to coverage and access.
- ***Variation in the Clinical Outcomes Associated With Personalized Medicine: A Research Report*** would develop a model to compare clinical outcomes associated with the use of personalized medicine strategies in various clinical contexts and health care delivery settings.
- ***Addressing Practice Gaps in the Implementation of Genetic Testing in Cancer Care: A Research Report*** would examine barriers that are discouraging the clinical integration of genetic testing and the appropriate utilization of testing results in cancer care.