MISSION

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. 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 145 personalized therapies on the market, a number that continues to grow as the U.S. Food and Drug Administration (FDA) 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 a new U.S. Congress, government agencies, and the private sector continue to pursue personalized health care that can translate higher up-front costs into a more effective and efficient health system in 2019, PMC will leverage its credibility as an international, multi-stakeholder organization focused on:

- **Education**: To raise the profile and scope 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 engage with regulators and other policymakers to advocate for continued investments in biomedical research as well as modernized regulatory, coverage, and payment policies that support access to personalized medicine.

- **Evidence Development**: 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 and scope 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. The Coalition will elucidate personalized medicine’s value proposition to multiple audiences, resources permitting.

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: 2018 Progress Report* will demonstrate that FDA is increasingly focused on advancing personalized medicine based upon its potential to improve care for patients and the health system.

• *An Introduction to Consumer Genetic Health Testing* will help consumers understand how increasingly prevalent genetic health risk tests can inform health care decisions.

• *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.

• *Personalized Medicine 101: A Briefing for U.S. Congressional Staff* will underline the benefits of personalized medicine as a new Congress begins to consider its priorities in health care.

• *The PMC Member Update*, published as a monthly e-newsletter, analyzes how the most impactful trends in health care may affect personalized medicine.

• *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 15th 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 15th 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.

• *The PMC Education Collaborative: A Definitive Online Resource*, which PMC has presented to its members as a supplemental sponsorship opportunity, would present a comprehensive online library of information about personalized medicine and its benefits to anyone with an interest in the field.

• *PMC Representation at Prominent Academic and Industry Conferences* will help ensure that personalized medicine topics remain a focal point for the industry. In addition to accommodating ad-hoc speaking requests, PMC will again co-organize the “PMC/BIO Personalized Medicine & Diagnostics Track” at the *2019 BIO Convention* and partner with the Cambridge Healthtech Institute on key sessions at the *2019 Molecular Medicine Tri-Conference* in San Francisco.
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.
  - **Establishing a Congressional Personalized Medicine Caucus**: To help translate bipartisan enthusiasm for personalized medicine in the U.S. Congress into legislation that can advance the field, PMC will advocate for creating a Congressional Personalized Medicine Caucus.
  - **Increasing Available Research Funding**: Investments in biomedical research conducted by the National Institutes of Health (NIH) drive many of the scientific 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 2020.
  - **Setting Priorities for Patient-Centered Research in Personalized Medicine (funded by PCORI)**: To ensure that the principles of personalized medicine are incorporated into biomedical research, PMC, using an award from the Patient-Centered Outcomes Research Institute (PCORI), will 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 propose and shape legislation, guidances, policies, and major governmental initiatives to improve the regulatory landscape for targeted drugs, diagnostics, and other technologies, including those policies that relate to digital health, real-world evidence, and artificial intelligence.

- **Modernizing Coverage and Payment Policies**: To ensure that patients have access to personalized medicine, PMC helps advocate for flexible coverage policies and adequate payment rates for personalized medicine products and services. PMC will continue to work with the U.S. Centers for Medicare and Medicaid Services (CMS) and private payers to inform strategies that can document the value of and facilitate sustainable access to personalized medicine, to include advocacy for arrangements based on patient-centered assessments of value.

- **Modernizing Value Assessment Methodologies**: By influencing decisions made by stakeholders including patients, providers, payers and policymakers, value assessment frameworks (VAFs) could accelerate or undermine progress in personalized medicine. PMC will publish a commentary advocating for VAF methodologies that account for the role that diagnostics and targeted treatment strategies can play in facilitating cost-effective health care. The Coalition will also organize a webinar that will help patient advocacy groups and other stakeholders recognize the limitations of current population-based VAF methodologies and demonstrate how aligning VAFs with the principles of personalized medicine can help ensure informed decision-making at the individual and population levels.
EVIDENCE DEVELOPMENT

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 potential 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 is already convincing health systems to pursue personalized prevention and treatment strategies.

- *The Clinical and Economic Value of Next-Generation Sequencing (NGS) in Cancer Care* will help build evidence demonstrating that NGS would yield significant clinical and economic benefits in cancer care if it were fully integrated into the health care system. The study is examining the utility of NGS for patients with cancer types including lung cancer and melanoma.

- *The Integration of Personalized Medicine into U.S. Health Systems: A Landscape Analysis*, which PMC has presented to its members as a supplemental sponsorship opportunity, 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.

- *Evaluating the Clinical and Economic Value of Genomic Sequencing for Patients With Rare and Undiagnosed Diseases* will provide evidence for genomic sequencing platform developers, payers, and providers about the clinical and economic utility of sequencing when applied to all patients with rare or undiagnosed diseases.

- *Personalized Medicine in Clinical Guidelines: A Progress Report* will benchmark clinical progress in personalized medicine thus far by providing a list of the circumstances in which current clinical guidelines already include personalized medicine strategies as standard of care.

- *The Personalized Medicine Pipeline: A Trend Analysis* will define the scientific outlook for personalized medicine by examining pharmaceutical company reports of early-phase clinical research and development as well as new drug applications submitted to the U.S. Food and Drug Administration (FDA).