
A Research Program for Advancing Personalized Medicine in 2023

An In-Depth Look at Initiatives to Evaluate Progress in Health
Care, Assess Clinical and Economic Value, and Examine Clinical
Integration Strategies

President/Chairman's Letter

Dear Colleague:

Personalized medicine – linking therapies to molecular diagnostic tests to ensure that the right treatments are targeted to the right patients – is still more aspiration than reality.

Although we have made enormous progress in developing a new scientific appreciation of human heterogeneity, as evidenced by the rise in the number of personalized treatments on the market from five in 2008 to more than 300 today, significant obstacles remain. In brief, we need more evidence that personalized medicine works – that it can improve clinical outcomes while making health care more efficient and therefore less costly. We also need to understand the opportunities and challenges shaping the pace of our progress toward integrating personalized medicine into clinical workstreams.

This three-part *Research Program for Advancing Personalized Medicine in 2023* is designed to facilitate progress on each of these fronts. The projects presented in these pages focus on evaluating technological progress in personalized medicine, assessing its clinical and economic value, and examining clinical integration efforts.

We request your thoughtful consideration of these initiatives as we call on organizations from multiple sectors of the health care ecosystem to provide financial support, in addition to membership dues, for the Personalized Medicine Coalition's expanding research portfolio.

Sincerely yours,



Edward Abrahams, Ph.D.
President



Lincoln Nadauld, M.D., Ph.D.
Board Chair

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Evaluating Progress in Health Care

The first arm of PMC's research portfolio aims to establish a foundational understanding of how far personalized medicine has come while informing further advancement of the field. The Coalition's past research in this area culminated in influential reports and peer-reviewed articles titled *Understanding Genomic Testing Utilization and Coverage in the United States* (a white paper), "Comparison of FDA Table of Pharmacogenetic Associations and Clinical Pharmacogenetics Implementation Consortium Guidelines" (*Am J Health Syst Pharm.*, 2022), and PMC's now-annual analysis of FDA's newest personalized medicine-related approval decisions. In 2023, PMC will build on this work by launching a two-phased study on payer policies and perspectives on personalized medicine.

STUDY 1

Payer Policies and Perspectives on Personalized Medicine

BACKGROUND

If the health care system is to secure the full benefits of personalized medicine, it must provide appropriate access to technologies, products, and services that make getting the best treatment option to each patient possible based on individual characteristics and circumstances. To help ensure equitable access, the reimbursement system must have evidence-based coverage and payment policies that support the timely adoption of validated personalized medicine technologies, including both diagnostics and therapeutics.

Payers are increasingly considering coverage and reimbursement of personalized medicine products and services both in the U.S. and internationally. However, there remain significant challenges in establishing coverage policies and payment rates for diagnostic tests and targeted therapeutics that reflect the value of their care. As a result, many novel diagnostics are under-reimbursed or not reimbursed at all, and targeted therapies are often subject to utilization management practices such as step therapy or prior authorization. Such practices ultimately restrict patient access to needed tests and optimal care. Coverage and reimbursement policies vary widely among different payers and laboratory benefits managers, and decision-making processes are often inconsistent and not transparent. Thus, it is not clear whether the broader payer community supports the move towards personalized medicine.

A better understanding of how payers view personalized medicine will help clarify the extent to which it has informed payer policies and perspectives globally

and help identify remaining needs, which, in turn, will help product developers understand the payer landscape and the challenges they face.

OBJECTIVE

This project will involve an examination of varying policies and perspectives to capture a global picture of the coverage and reimbursement of personalized medicine services and technologies by public and private payers. The landscape analysis will include a representative sample of public and private health insurers, as well as large employers and laboratory benefit managers, and will include both quantitative and qualitative results to ensure that a global health system-wide payment picture of personalized medicine is captured. The results will be included in a report whose purpose will be to provide key personalized medicine stakeholders, including product developers, clinical laboratories, health care providers, payers, patients, and policymakers, with information about access to personalized medicine technologies as related to the payment environment, thus informing payer-related strategies that may lead to improved access to personalized medicine.

PROJECT OUTLINE

The project, to be completed in six months, will involve the development of a framework to be used to measure personalized medicine coverage and reimbursement levels by government-run and private health insurers, as well as large employers.

The framework will be based on publicly available data showing payer coverage and utilization management policies, as well as payment details. It will also include survey data highlighting payer perspectives, partnerships, and practices related to personalized medicine.

To facilitate a holistic view of the landscape of personalized medicine coverage and reimbursement, the analysis will focus on a representative sample of public and

private payers and payment manager organizations, including large and small private health insurers; national and state-run public health plans; large employers with managed employee health benefits; and laboratory benefit manager organizations.

A project steering committee consisting of PMC members across payer, provider, industry, and patient stakeholder groups will guide the project, review survey questions, track progress at various milestones, and ensure an appropriate sample of survey respondents.

CONCLUSION

Many organizations within the personalized medicine community have called for an analysis of the current landscape of payer perspectives and policies on personalized medicine in the United States and internationally. This proposal is PMC's answer to that call.

Assessing Clinical and Economic Value

The second arm of PMC's research portfolio helps establish the value proposition associated with integrating personalized medicine strategies into health care. Through its previous initiatives in this area, the Coalition has developed peer-reviewed studies titled "Cost Effectiveness of Multigene Panel Sequencing for Patients With Advanced Non-Small-Cell Lung Cancer" (*JCO Clin Cancer Inform.*, 2019) and "Cost-Effectiveness of Exome and Genome Sequencing for Children With Rare and Undiagnosed Conditions" (*Genet Med.*, 2022). In 2023, PMC will take a holistic look at the value of systemic personalized medicine integration.

STUDY 2

Improvements in Clinical Care Associated With Personalized Medicine

BACKGROUND

Integrating personalized medicine into health care delivery systems requires a shift from traditional “one-size-fits-all” practices to a more efficient approach based on understanding and addressing individual patient characteristics and circumstances, therefore requiring an investment in workforce and financial resources associated with new technologies, policies, practices and education. As providers consider integrating personalized medicine approaches into their health care delivery systems, they increasingly want evidence showing that the implementation of personalized medicine improves clinical care.

To gain a better understanding of the current landscape of personalized medicine integration, PMC coordinated a study to capture a holistic picture of the complete range of personalized medicine implementation strategies and technologies being undertaken at a representative sample of health care delivery institutions across the United States. Based on a quantitative framework that assesses progress toward personalized medicine integration at the institutional level with consideration of multiple clinical areas, institutions were assigned scores between one (minimal personalized medicine integration) and five (expansive and systematic integration). The findings show that U.S. health care organizations are widely distributed in terms of integration across clinical areas, but most are at level two or level three.

Level four and level five institutions stand out as pioneers in personalized medicine but are still not common. While many of these institutions are thought of as outstanding health systems, there is a need for evidence linking the personalized medicine approach to improved clinical care. A better understanding of the level of integration of personalized medicine associated with improved health care delivery and clinical outcomes may help justify the adoption of policy changes and investments associated with its implementation.

OBJECTIVE

This project will examine the effect of integrating personalized medicine approaches in driving improved clinical care and systemic efficiency. The extent to which a health care delivery institution has integrated personalized medicine will be based on integration scores as determined through PMC's *Integration of Personalized Medicine in U.S. Health Systems* landscape analysis. Improvements in clinical care and systemic efficiency will be considered as part of a matrix of elements contributing to the increased delivery of high-quality health care practices and services that can improve clinical outcomes and/or provide greater access to relatively higher value care, and will include both quantitative and qualitative measures.

The results will be included in a report whose purpose will be to provide key personalized medicine stakeholders — including providers, payers, clinical guideline developers, pharmaceutical and diagnostic manufacturers, patients and policymakers — with evidence of the value of integrating personalized medicine into clinical practice. This, in turn, can help inform efforts to address the most critical outstanding integration challenges.

PROJECT OUTLINE

The project, to be completed in 12 months, will involve the development of an evidence-based framework linking personalized medicine integration scores with improved clinical care, along with subsequent analysis that will help demonstrate the current value of personalized medicine integration.

A project steering committee composed of partner members of PMC's Health Care Working Group will guide the development of the value framework with the inclusion of appropriate evidence; track progress at various milestones; and ensure that results are meaningful to key audiences.

The analysis will include examination of a representative sample of U.S. health care delivery institutions including academic health centers; urban, suburban, and rural community hospital systems; and integrated payer/provider systems.

A payer advisory committee will also be formed to provide guidance especially related to assumptions about improved clinical care utilized in the value framework. The advisory committee will help ensure that the project results incorporate the perspectives of these key stakeholders, increasing the likelihood that the results will be useful in informing coverage and reimbursement processes.

Results of the study will be published in a peer-reviewed journal and become a resource for decision-makers in health care delivery, industry, and government.

CONCLUSION

The personalized medicine community needs an evidence-based analysis linking the implementation of personalized medicine to improved clinical care to help advance personalized medicine integration efforts and to make clear to health care decision-makers that they should develop policies and practices that drive personalized medicine. *Improvements in Clinical Care Associated With Personalized Medicine* is designed to address this need.

Examining Clinical Integration Strategies

Understanding that integrating personalized medicine into clinical care presents daunting challenges for many health systems, PMC has designed the third arm of its research portfolio to provide critical information and strategies to aid in the clinical implementation of personalized medicine. The Coalition's foundational publications in this area are titled "Strategies for Integrating Personalized Medicine into Healthcare Practice" (*Per Med.*, 2017), "A Quantitative Framework for Measuring Personalized Medicine Integration into US Healthcare Delivery Organizations" (*J Pers Med.*, 2021), "Clinical Utility of Genomic Testing in Cancer Care" (*JCO Precis Oncol.*, 2022), and "Impact of Clinical Practice Gaps on the Implementation of Personalized Medicine in Advanced Non-Small-Cell Lung Cancer" (*JCO Precis Oncol.*, 2022). In 2023, PMC will focus on understanding how to address inequities in research advancing personalized medicine.

STUDY 3

Addressing Disparities in Research Informing Personalized Medicine

BACKGROUND

In order for personalized medicine to be universally applicable, research and development of new treatment and prevention strategies that are tailored to the biological characteristics and circumstances of individuals must include data from all types of people, thereby capturing the diversity of patients represented within the population. However, racial and ethnic minorities, the elderly, women, and patients from disadvantaged socioeconomic groups are often underrepresented in clinical trials. The problem persists even in disease areas characterized by elevated disease incidence and mortality counts among understudied patient populations.

As electronic health records (EHRs) and other digital technologies become ubiquitous in health care settings, scientists are also increasingly conducting practice-based research and machine learning studies to help inform personalized medicine implementation strategies and policy development. Little is known about how the use of these data sources compares to more traditional research strategies. Some researchers, patients and policymakers worry that an overreliance on data built with underlying health care inequities has the potential to further exclude patients from underrepresented populations.

Health research data can fail to reflect the demographic characteristics of populations being studied due to patient mistrust and transparency concerns, outreach and education barriers, cultural barriers, overly restrictive study design eligibility criteria, cost concerns/disproportionate representation among the uninsured, language/linguistic/literacy challenges, and practical obstacles such as proximity or access to health care facilities.

The advancement of personalized medicine underlines the importance of overcoming health disparities because it anticipates targeted treatments based on a patient's individual characteristics and health needs, including variables such as the patient's race, ethnicity, age, sex, and socioeconomic status. This relies, however, on an inclusive and equitable representation of patients with these diverse characteristics and health needs in personalized medicine research and development. A better understanding of how sociocultural, behavioral, and health system factors converge and contribute to unequal participation in clinical research and differential representation in research data is therefore needed to help ensure that personalized medicine scientific advances are impactful to all patients.

OBJECTIVE

This initiative will bring together key stakeholders representing public, private, and nonprofit organizations that are working with or are part of communities that are underrepresented in health care research to comprehensively address disparities in clinical trials participation and a lack of racial, ethnic, demographic and socioeconomic equity in health data used to guide research and development as well as the delivery of personalized medicine technologies. The findings will be included in a report whose purpose will be to provide key personalized medicine stakeholders, including clinical and health outcomes researchers, information management and digital health technologies organizations, providers, pharmaceutical and diagnostic manufacturers, payers, patients, and lawmakers with insight into barriers to equitable personalized medicine research. The report will provide recommendations on how to ensure that innovations underpinning personalized medicine are accessible to all patients.

PROJECT OUTLINE

This project will involve identifying a health equity task force consisting of key stakeholders, partners, and experts that have knowledge of health research data and are working with or are part of communities that are underrepresented in health care research. A series of interviews with the task force will be conducted to elucidate inequities within health data related to current clinical research, practice, and community access. The interviews will help define barriers to the development and use of representative health data for personalized medicine research and development.

PMC, working with this health equity task force, will then conduct a comprehensive policy review, to include: an examination of trial protocols, patient engagement approaches, existing strategies to address health care data disparities, and coverage, reimbursement, and utilization policies that can lead to inequalities in access to health care. The policy review will also examine the different impacts of research practices and policies on different underrepresented populations.

Based on the policy review, a set of draft recommendations to help increase the proportion of underrepresented groups in health research and appropriately account for diverse population demographics data will be developed.

Patients and community leaders will be surveyed to get their feedback on the draft recommendations and to gauge community perceptions of their potential impact and feasibility.

A report will be published with a set of final recommendations based on the community feedback and rankings.

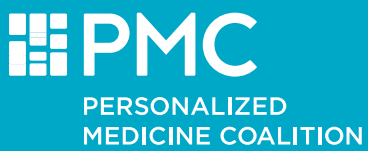
The project will involve a qualitative analysis of inequities in various forms of data used for health research, including data from clinical trials, practice-based research studies, genetic and disease-specific databases, pooled electronic health records, health insurance claims databases, and population data used in machine learning algorithms.



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 health systems.





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