Fiscal Year 2024 Senate Appropriations Committee Subcommittee on Labor, Health and Human Services, Education and Related Agencies Appropriations Testimony

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National Institutes of Health Fiscal Year 2024 Appropriations

Chairwoman Baldwin, Ranking Member Moore Capito, and distinguished members of the subcommittee, the Personalized Medicine Coalition (PMC) appreciates the opportunity to submit testimony on the National Institutes of Health (NIH) fiscal year (FY) 2024 appropriations and to highlight the importance of NIH-funded research to personalized medicine. PMC is a nonprofit education and advocacy organization comprised of more than 220 institutions from across the health care spectrum who support this growing field. We appreciate the sustained, robust funding for NIH provided by this subcommittee in recent years, which has allowed NIH to continue building the foundation of scientific knowledge underpinning personalized medicine. Continuing this momentum will be essential to support further discovery of targeted health care interventions that can improve patients' lives. As the subcommittee begins work on the FY 2024 Labor, Health and Human Services, Education and Related Agencies appropriations bill, **we request at least $50.924 billion for NIH's base program level budget. This $3.465 billion increase over the comparable FY 2023 program level will allow meaningful growth of nearly 5 percent.**

Personalized medicine, also called precision or individualized medicine, is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients. Personalized medicine promises to help detect the onset of disease, pre-empt its progression, and improve the quality, accessibility, and affordability of health care.[i] By further investing in biomedical research, Congress can help advance a brighter future where personalized medicine is one day available and accessible for all patients and health systems.

I. The Role of NIH in Personalized Medicine

Decades of NIH-funded biomedical research on the genetic and biological underpinnings of disease have contributed to the development of personalized treatments benefitting patients today. In fact, as of 2022, this research has helped inform the development of more than 300 personalized treatments[ii] and over 166,000 genetic testing products.[iii] These numbers continue to grow, with personalized medicines accounting for more than a quarter of all new drugs approved by the Food and Drug Administration (FDA) for each of the last eight years.[iv] Nearly half of new personalized treatments are approved for indications outside of oncology. Continued progress cannot be taken for granted. To ensure that scientists and innovators maintain this momentum, Congress must support policies that encourage the advancement of the field and commit to funding NIH's basic and translational research over multiple years.

II. Research Accelerating Personalized Medicine

NIH is leading much of the scientific discovery for personalized medicine, which begins with basic research that generates fundamental knowledge about the molecular basis of a disease and with translational research aimed at applying that knowledge to develop a treatment or cure. Many NIH institutes and centers are contributing research informing the development of personalized medicine approaches. A robust base budget for NIH in FY 2024 would protect the agency's foundational role in the identification and development of treatments, technologies, and tools for personalized medicine.

The *All of Us*™ Research Program, for example, was launched in 2018 to collect genetic and health information from one million volunteers as part of a decades-long research project. The *All of Us*™ Research Program will enable new discoveries over time with a goal of improving population health through the identification of risk factors and biomarkers to allow more efficient and accurate diagnosis and screening, more rational use of existing therapeutics, and the development of new treatments. As of March 2022, over 475,000 individuals have consented to participate in the program, with more than 326,000 being fully enrolled.[v] More than 80 percent of the enrolled individuals are from groups historically underrepresented in research.[vi] Ultimately, the scope of the program's impact will depend upon the extent to which it engages participants, who will be asked to share data over the long term. Additional funding for NIH will help secure the future of this promising program.

Cancer care, too, has been and will continue to be profoundly influenced by new personalized medicine approaches for detecting and treating early- and late-stage diseases. Over the past decade, personalized treatments harnessing the immune system have driven declines in mortality for lung cancer and melanoma. Targeted therapies, cell-based immunotherapies, and liquid biopsy tests are becoming the hallmarks of a new era of personalized medicine as they are integrated into the standard of care for cancer patients. Ongoing programs promise continued advances. The Cancer Moonshot, for example, aims to transform the way cancer research is conducted by fostering collaboration and data sharing. Since the Cancer Moonshot was launched in 2016, over 70 programs and consortia have been supported and more than 250 research projects have been funded.[vii] Significant infrastructure for conducting cancer research and sharing resources has been established.[viii] And at the National Cancer Institute (NCI), researchers are studying the potential of multi-cancer early detection (MCED) tests designed to find evidence of cancer wherever it occurs in the body from a simple blood draw. NCI's large national trials on MCED tests will begin enrolling participants in 2024. Additional base budget funding for NIH will help NCI sustain progress toward the Cancer Moonshot's goal of "ending cancer as we know it."

Basic and translational research also offer opportunities for personalized medicine beyond oncology, especially for rare diseases. Although individually rare, rare diseases collectively affect an estimated 25 to 30 million Americans. In recent decades, advances in genomics have helped researchers identify the molecular causes of 6,500 rare diseases. Programs at the National Institute for Advancing Translational Science (NCATS) have helped shift the scientific approach to researching rare diseases from one disease at a time to many. Pooling patients, data, experiences, and resources promises to lead to more successful clinical trials sooner for rare disease patients. More needs to be done, however, to accelerate the development of treatments for the 95 percent of rare diseases with no FDA-approved treatment; to strengthen the innovation of diagnostics to shorten the average 6.3-year long diagnostic odyssey patients face; and to lower the nearly $1 trillion annual economic burden of rare diseases.

Other patients are living with highly prevalent diseases where personalized medicine can offer better treatments or a cure. For example, the Alzheimer's Association estimates that more than six million Americans are living with Alzheimer's disease, whose genetic and molecular complexities are not fully understood. To help address the issue, NIH funded the creation of a

Precision Aging Network (PAN) that could transform the way we think about the aging brain. PAN brings together researchers from across the country to better understand how and why people experience brain aging differently, with the ultimate goal of developing more effective treatments and interventions targeted to the individual. To reduce the time between the discovery of potential drug targets and the development of new drugs, NIH continues to lead the Accelerating Medicines Partnership (AMP) for Alzheimer's disease and other initiatives. AMP has identified over 500 drug targets and maintains partnerships to enable a personalized medicine approach to researching new treatments.[ix] Other ongoing AMP projects aim to facilitate the development of gene therapies for rare diseases as well as treatments and diagnostics for type 2 diabetes, Parkinson's disease, schizophrenia, and kidney and heart diseases. A boost to the NIH base budget would go a long way toward ensuring the continued success of these programs.

Ensuring that scientific breakthroughs in personalized medicine are impactful to all patients will require the inclusive and equitable representation of patients with diverse characteristics and health needs in research and clinical trials. Multiple NIH activities are underway to improve research policies and incorporate diverse perspectives into solving scientific problems. The UNITE Initiative and the National Human Genome Research Institute (NHGRI)'s *2020 Strategic Vision*, for example, promise to play a key role in addressing disparities, as does research led by the National Institute on Minority Health and Health Disparities (NIMHD).

IV. Conclusion

PMC appreciates the opportunity to highlight NIH's importance to the continued success of personalized medicine. We believe that continued federal investment in NIH-supported research will bring us closer to a future in which every patient benefits from an individualized approach to health care.

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[i] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_The_Personalized_Medicine_Report_Opportunity_Challenges_and_the_Future.pdf
[ii] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_The_Personalized_Medicine_Report_Opportunity_Challenges_and_the_Future.pdf
[iii] https://doi.org/10.1002/ajmg.c.31881
[iv] https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/Personalized_Medicine_at_FDA_The_Scope_Significance_of_Progress_in_2021.pdf
[v] https://www.joinallofus.org/newsletters/2022/march
[vi] https://officeofbudget.od.nih.gov/pdfs/FY23/br/Overview%20of%20FY%202023%20Presidents%20Budget.pdf
[vii] https://doi.org/10.1016/j.ccell.2021.04.015
[viii] https://www.cancer.gov/research/annual-plan/directors-message
[ix] https://www.nih.gov/research-training/accelerating-medicines-partnership-amp/alzheimers-disease