Fiscal Year 2023 Senate Appropriations Committee Outside Witness Testimony
Cynthia A. Bens, Senior Vice President, Public Policy, Personalized Medicine Coalition
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Subcommittee on Labor, Health and Human Services, Education and Related Agencies
National Institutes of Health Fiscal Year 2023 Appropriations

Chairwoman Murray, Ranking Member Blunt 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) 2023 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 have come together to support this growing field. We appreciate the sustained, robust funding for NIH provided by the subcommittee in recent years, which has allowed NIH to continue building the foundation of scientific knowledge underpinning personalized medicine in the midst of unprecedented challenges. Sustaining this momentum will be essential to support further discovery of targeted health care interventions for patients with cancer as well as rare, common, and infectious diseases. As the subcommittee begins work on the FY 2023 Labor, Health and Human Services, Education and Related Agencies appropriations bill, **we request at least $49.048 billion for NIH's base program level budget. We also urge you to ensure that funds for targeted programs, like those supporting the new Advanced Research Projects Agency for Health (ARPA-H) and pandemic preparedness, supplement this request for NIH's base program level budget.**

Our funding request for FY 2023 amounts to a $4.1 billion (or nearly 8%) increase to the NIH budget, including funding for specific initiatives under the *21st Century Cures Act (Cures Act)*. This request would allow for meaningful growth above inflation in NIH's base budget and expand NIH's capacity to support progress in personalized medicine.

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 detect the onset of disease, pre-empt its progression, and improve the quality, accessibility, and affordability of health care.[i] By increasing the government's investment in science at this pivotal moment, Congress can help advance a new era of personalized medicine that promises a brighter future for 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 that patients are benefitting from today. As of 2020, this research has informed the development of more than 286 personalized treatments[ii] and over 166,703 genetic testing products.[iii] These numbers continue to grow, with personalized medicines accounting for more than a quarter of all new drugs approved

by FDA each of the past seven years and with more than half of new personalized treatments being approved for indications outside of oncology.[iv] Nearly 20 years since the historic completion of the Human Genome Project in 2003, researchers recently finished deciphering the final 8% of the roughly 3-billion-base human genome sequence that was previously impossible to decode.[v] Having a complete, gap-free reference sequence of human DNA will further improve our understanding of how genes influence human health. In recent years, scientists have also made notable progress in assessing biomarkers beyond the genome, such as proteomic and metabolic biomarkers.[vi] Harnessing the power of personalized medicine to better diagnose, treat, and prevent disease will require a continued commitment by Congress to fund NIH's basic and translational research.

II. Sustaining Basic and Translational Research for 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 institutes and centers at NIH are contributing research informing the development of personalized medicines, including the National Human Genome Research Institute (NHGRI), the National Cancer Institute (NCI), the National Institute on Aging (NIA), the National Heart, Lung and Blood Institute (NHLBI), the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), the National Center for Advancing Translational Sciences (NCATS), and the National Institute on Minority Health and Health Disparities (NIMHD). A robust base budget for NIH in FY 2023 would protect the agency's foundational role in the identification and development of treatments, technologies, and tools for personalized medicine.

Cancer care has been and will continue to be profoundly influenced by new personalized medicine approaches for detecting and treating early- and late-stage cancers. In 2021, for example, FDA approved two new chimeric antigen receptor (CAR) T-cell-based immunotherapies for patients with refractory large B-cell lymphoma and refractory multiple myeloma.[vii] These treatments work by genetically re-engineering a patient's own immune cells to combat cancer. Over the past decade, personalized treatments harnessing the immune system have also driven declines in mortality for lung cancer and melanoma. Recognizing the potential of multi-cancer early-detection tests designed to find evidence of cancer wherever it occurs in the body from a simple blood draw, NCI is also exploring large national trials to evaluate these novel tests and is already funding the collection of blood samples to serve as controls. These tests may provide less invasive testing options that could detect a patient's cancer at early stages when treatment may be more effective and less costly.

Basic and translational research also offers opportunities for personalized medicine beyond oncology, especially for rare diseases. Although individually rare, rare diseases collectively affect an estimated 25 to 30 million Americans. With advances in genomics, the molecular causes of 6,500 rare diseases have been identified — but only about 5 percent have an FDA-approved treatment. Over the past decade, programs at NCATS have helped shift the scientific approach to researching rare diseases from one disease at a time to many diseases at a time. Pooling patients, data, experiences, and resources promises to lead to more successful clinical trials sooner for rare disease patients who presently have few or no treatment and diagnostic

options. Accelerating this research can help shorten the average of six years it takes for a rare disease patient to find the correct diagnosis and lower the nearly $1 trillion annual economic burden of rare diseases.[viii]

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 6.2 million Americans are living with Alzheimer's disease.[ix] Despite increasing numbers of Alzheimer's diagnoses, researchers still need to study the genetic underpinnings of Alzheimer's disease to more fully understand its complexity. To shorten the time between the discovery of potential drug targets and the development of new drugs, the Accelerating Medicines Partnership (AMP) for Alzheimer's disease, led by NIH, has identified over 500 drug targets, and in 2021 the public-private partnership launched a second iteration to enable a personalized medicine approach to researching new treatments.[x] Other new and ongoing AMP projects aim to facilitate the development of gene therapies for rare diseases as well as treatments and diagnostics for type 2 diabetes, rheumatoid arthritis, lupus, Parkinson's disease, common metabolic diseases like kidney and heart disease, and schizophrenia.

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 initiatives at NIH to improve research policies and incorporate diverse perspectives into solving complex scientific problems — such as through the UNITE initiative, NHGRI's action agenda for a diverse genomics workforce, and the forthcoming *NIH-Wide Diversity, Equity, Inclusion, and Accessibility Strategic Plan* — will play a key role in addressing these disparities, as will the research led by NIMHD on improving minority health and understanding factors contributing to health disparities.

III. Accelerating Personalized Medicine Research

Increasing NIH's base budget will also ensure that the agency has the resources necessary to advance the longstanding aspects of its mission without de-prioritizing supplemental initiatives in personalized medicine provided for by Congress in the *Cures Act*.

The first of these initiatives made possible in part by the *Cures Act*, the *All of Us*™ Research Program, was launched in 2018 to begin collecting genetic and health information from one million volunteers as part of a decades-long research project. As of March 2022, over 475,000 individuals consented to participate, with more than 326,000 being fully enrolled.[xi] More than 80 percent of the enrolled individuals are from groups historically underrepresented in research,[xii] such as seniors, women, Hispanics and Latinos, African Americans, Asian Americans, and members of the LGBTQ community. Extensive efforts are also underway to engage American Indian and Alaska Native communities. Reaching a significant milestone, the program recently released its first dataset of nearly 100,000 whole genome sequences,[xiii] and over 1,100 research projects have been launched using the program's groundbreaking dataset. Later this year, the program also plans to begin sharing results with participants on their hereditary disease risk and medication-gene interactions. Pooling health care data across large datasets that span populations and disease areas will play a key role in advancing research for personalized medicine approaches to care.

The second initiative spurred by *the Cures Act*, the Beau Biden Cancer Moonshot, aims to transform the way cancer research is conducted by fostering collaboration and data sharing. As it enters its seventh year, the Cancer Moonshot has grown to support over 240 new research projects [xiv] and has established a significant infrastructure for conducting cancer research and sharing resources.[xv] Collaborations formed by the program include the Partnership for Accelerating Cancer Therapies (PACT), which consists of 12 pharmaceutical companies, the Foundation for NIH, and FDA working together to identify, develop, and validate biomarkers advancing the discovery of new immunotherapy treatments. This year, President Biden announced a bold new goal for the initiative of ending cancer as we know it. Funding provided by the *Cures Act* ends in FY 2023, and additional base budget funding will help NCI sustain this progress that has already been made in cancer research once the *Cures Act* funding expires.

IV. Conclusion

PMC appreciates the opportunity to highlight NIH's importance to the continued success of personalized medicine. PMC believes that basic and translational research at NIH is key to bringing us closer to a future in which every patient benefits from an individualized approach to health care. Therefore, we urge the subcommittee to appropriate at least a $49.048 billion budget to support existing centers and programs at NIH, in addition to funding Congress may provide for targeted initiatives.

[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.nih.gov/news-events/nih-research-matters/first-complete-sequence-human-genome
[vi] https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_The_Personalized_Medicine_Report_Opportunity_Challenges_and_the_Future.pdf
[vii] https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/Personalized_Medicine_at_FDA_The_Scope_Significance_of_Progress_in_2021.pdf
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[xiv] https://doi.org/10.1016/j.ccell.2021.04.015
[xv] https://www.cancer.gov/research/annual-plan/directors-message