

Fiscal Year 2023 House Appropriations Committee Subcommittee on Labor, Health and Human Services, Education and Related Agencies Appropriations Testimony

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Chair DeLauro, Ranking Member Cole 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 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. 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.**

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, 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 benefitting patients today. As of 2020, this research has informed the development of more than 286 personalized treatmentsⁱⁱ 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} 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 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.^v 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. 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. 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.

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.^{vi} 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.^{vii} 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, Parkinson's disease, schizophrenia, and kidney and heart disease.

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 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*TM Research Program, was launched in 2018 to collect 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.^{viii} More than 80 percent of the enrolled individuals are from groups historically underrepresented in research,^{ix}

such as seniors, women, Hispanics and Latinos, African Americans, Asian Americans, and members of the LGBTQ community. Reaching a significant milestone, the program recently released its first dataset of nearly 100,000 whole genome sequences,^x and over 1,100 research projects have been launched using the program's groundbreaking dataset. Later this year, the program also plans to begin returning certain health information to participants. 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^{xi} and has established a significant infrastructure for conducting cancer research and sharing resources.^{xii} 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. Additional base budget funding will help NCI sustain this progress 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 increasing the government's investment in 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.

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.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/Personalized_Medicine_at_FDA_The_Scope_Significance_of_Progress_in_2021.pdf

^{vi} <https://www.alz.org/media/Documents/alzheimers-facts-and-figures.pdf>

^{vii} <https://www.nih.gov/research-training/accelerating-medicines-partnership-amp/alzheimers-disease>

^{viii} <https://www.joinallofus.org/newsletters/2022/march>

^{ix} <https://officeofbudget.od.nih.gov/pdfs/FY23/br/Overview%20of%20FY%202023%20Presidents%20Budget.pdf>

^x <https://directorsblog.nih.gov/2022/03/29/nih-all-of-us-research-programs-first-nearly-100000-complete-human-genome-sequences-set-stage-for-new-discoveries/>

^{xi} <https://doi.org/10.1016/j.ccell.2021.04.015>

^{xii} <https://www.cancer.gov/research/annual-plan/directors-message>