

Fiscal Year 2025 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 2025 Appropriations

Chairwoman Baldwin, Ranking Member 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) 2025 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 200 institutions from across the health care spectrum who support this growing field. In recent years, the NIH has been able to continue building the foundation of scientific knowledge underpinning personalized medicine because of sustained investments made by Congress. PMC commends the subcommittee for recognizing the critical role the NIH plays in spurring discoveries that will save and improve lives, and for your ongoing attention to the need for robust funding of this agency. Continued 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 2025 Labor, Health and Human Services, Education and Related Agencies appropriations bill, **we request an appropriation of at least \$51.3 billion for NIH's foundational work, which would allow NIH's base budget to keep pace with the biomedical research and development price index and allow meaningful growth of roughly 5 percent.** In addition, as the Advanced Research Projects Agency for Health (ARPA-H) ramps up its work in targeted research areas, any funding for ARPA-H should supplement, rather than replace, further investments in NIH.

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, preempt 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 benefiting patients today. In fact, as of 2023, this research has helped inform the development of more than 300 personalized treatments and over 140,000 diagnostic products.ⁱⁱ 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) since 2015.ⁱⁱⁱ More than half of new personalized treatments are now 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 2025 would protect the agency's role in advancing personalized medicine to address rare, common, and chronic diseases.

Although individually rare, progress in data science and an increased understanding of disease genetics lead experts to agree that more than an estimated 10,000 rare diseases are affecting about 30 million people in the U.S. Most of these individuals are children. In all, nearly 10 percent of the U.S. population have a rare disease. In recent decades, advances in genomics have helped researchers identify the molecular causes of 6,500 rare diseases. Programs at the NIH's 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, experiences, and resources promises to lead to more successful clinical trials sooner for rare disease patients. In 2023, PMC noted that nearly 60 percent of the personalized medicines approved by the FDA were for rare diseases. While this progress is encouraging, more needs to be done 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.

Cancer care 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 have driven declines in mortality for some cancers and still account for more than a quarter of FDA approvals. Cancer research supported by the National Cancer Institute (NCI) played a vital role in developing targeted therapies responsible for the recent twofold decline in deaths from non-small cell lung cancer, the most common type of lung cancer. Through basic science and immunology studies, NCI also laid the foundation for the development of immune checkpoint inhibitors to enhance the body's immune response against cancer. Several of these inhibitors are now approved for treating lung cancer and have substantially improved outcomes for many people with non-small cell lung cancer. Ongoing programs promise continued advances to help researchers confront those cancers for which significant headway remains elusive, such as pancreatic cancer, glioblastoma, and certain childhood cancers.^{iv} Only high levels of sustained funding for NIH will enable NCI to support this ongoing research and pursue the next phase of the Cancer Moonshot to translate discoveries into innovative cancer prevention and treatment approaches; transform cancer screening and

diagnosis practices to save more lives; ensure rapid dissemination of standards of care to all people equitably; and enhance the diversity of the cancer research workforce.

Other patients are living with highly prevalent diseases where personalized medicine can offer better diagnosis, treatments, or a cure. A recent Congressional briefing co-hosted by the Congressional Personalized Medicine and Congressional Kidney caucuses highlighted that an estimated 37 million Americans are living with kidney diseases, including more than 800,000 with kidney failure.^v People who progress to kidney failure require either dialysis or a kidney transplant. Dialysis, the most common therapy for kidney failure, has a five-year mortality rate of 60 percent. Medicare annually spends more than \$50 billion, approximately 7 percent of all Medicare spending, to manage kidney failure. The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) funds the vast majority of federal research on kidney diseases, and NIDDK-funded scientists have produced several major breakthroughs. Geneticists have made advances in understanding the genes that cause kidney failure, and the first medications to treat certain genetic kidney diseases are now in Phase III clinical trials. NIDDK continues to support the Kidney Precision Medicine Project to pinpoint targets for novel therapies. All of this sets the stage for the advancement of personalized medicine in kidney care, which may be possible with further investment.

The NIH released an FY 2025 professional judgement budget on Alzheimer's disease and related dementias,^{vi} which impact more than six million Americans at a cost of \$321 billion to the U.S. economy.^{vii} In the last decade, NIH-funded and conducted research has allowed scientists to advance their understanding of the risk factors, genetics, and mechanisms of disease in dementia; diversify and de-risk the therapeutic pipeline for disease modifying drugs; advance drug repurposing and combination therapy development; and discover tools to detect, diagnose, and monitor dementia. Initiatives like the NIH-supported Precision Aging Network (PAN) are transforming the way we think about brain aging. PAN brings together researchers from across the country to better understand how and why people experience brain aging differently, with the goal of developing more effective treatments and interventions targeted to the individual. Increased funding for NIH is needed to allow the agency to support existing programs and to pursue research opportunities in population studies of disease mechanisms, diagnosis, assessment, and disease monitoring in order to better address the human and economic costs of Alzheimer's 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 NIH activities are underway to improve research policies and incorporate diverse perspectives into solving scientific problems. 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 April 2024, more than 790,000 individuals are participating in the program, with over 540,000 fully enrolled.^{viii} Of the enrolled individuals, 87 percent are from groups historically underrepresented in research.^{ix} *All of Us*TM looks for genetic variants in 59 genes associated with serious hereditary health conditions. It also looks for seven genes that can affect how bodies process medicines, which is information that can be used to guide which medication or dosage an individual is prescribed. Participants can receive these results. To date, approximately 3 percent

of participants who received their hereditary disease risk report have a potentially life-changing genetic variant and 89 percent of participants who receive medications reports have a result that could impact how their body processes drugs. Ultimately, *All of Us*TM 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. The program's success and future depend on additional funding for NIH. *All of Us*TM faces a funding cliff as the *21st Century Cures Act* authorizations expire in the coming year.

Realizing the promise of genomic medicine for complex common diseases requires improved methods for genomic risk prediction that can be used in all populations. A polygenic risk score (PRS) is calculated based on the total number of disease-associated genomic variants that a person harbors, and it can be used to assess a person's risk for developing certain diseases. However, at present, these risk scores are less applicable to non-European individuals due to the lack of sufficient genomic data from these populations. The Polygenic RISK MEthods in Diverse populations (PRIMED) Consortium, supported by the National Human Genome Research Institute (NHGRI), is working to improve PRS usage by studying larger numbers of non-European individuals. Multiple PRIMED sites are developing and evaluating PRS models for various common diseases, including cardiovascular diseases, diabetes, and cancer. These efforts demonstrate the benefit of using data from diverse populations to build models with better predictive capability across populations. In FY 2025 and beyond, PRIMED will continue to publish collaborative research; develop tools, pipelines, and reference panels; and disseminate recommendations to ensure PRS research and implementation are inclusive and generalizable and do not contribute to health disparities.^x

IV. Conclusion

PMC appreciates the opportunity to highlight examples of NIH's importance to the continued success of personalized medicine. Personalized medicine promises to help detect the onset of disease, pre-empt its progression, and improve the quality, accessibility, and affordability of health care. We believe that continued federal investment in NIH-supported research will bring us closer to a future in which every patient benefits from this approach.

ⁱ https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/01/strategic_plan.pdf

ⁱⁱ <https://doi.org/10.1002/ajmg.c.31881>

ⁱⁱⁱ <https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/02/report-3.pdf>

^{iv} <https://www.cancer.gov/research/leading-progress/2025-professional-judgment-budget-proposal-aag.pdf>

^v <https://youtu.be/HboDMvaeME>

^{vi} https://www.nia.nih.gov/sites/default/files/2023-07/fy25_alzheimers_bypassbudget.pdf

^{vii} <https://www.jec.senate.gov/public/index.cfm/democrats/2022/7/the-economic-costs-of-alzheimer-s-disease>

^{viii} <https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/05/slides.pdf>

^{ix} <https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/05/slides.pdf>

^x <https://www.genome.gov/about-nhgri/Budget-Financial-Information>