Chairwoman 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) 2021 appropriations. PMC is a nonprofit education and advocacy organization comprised of more than 230 institutions from across the health care spectrum. As the subcommittee begins work on the FY 2021 Labor, Health and Human Services, Education and Related Agencies appropriations bill, we ask that the NIH receive an appropriation of at least $44.7 billion in FY 2021, a $3 billion increase over the NIH’s program level funding in FY 2020. This funding level would allow for meaningful growth above inflation in the base budget, which would expand NIH’s capacity to support promising science in all disciplines. It also would ensure that funding from the Innovation Account established in the 21st Century Cures Act would supplement the NIH’s base budget, as intended, through dedicated funding for specific programs.

Personalized medicine, also called precision or individualized medicine, is an evolving field in which physicians use diagnostic tests to identify specific biological markers, often genetic, that help determine which medical treatments will work best for each patient. By combining this information with an individual’s medical records, circumstances, and values, personalized medicine allows doctors and patients to develop targeted treatment and prevention plans. Personalized health care promises to detect the onset of disease and pre-empt its progression, as well as improve the quality, accessibility, and affordability of health care.
I. The Role of NIH in Personalized Medicine

For each of the past five years, personalized medicines have accounted for a quarter or more of the new drugs approved by the U.S. Food and Drug Administration (FDA), with a record of 42 percent in 2018. In 2005, personalized medicines accounted for only 5 percent of new drug approvals. The most recent approvals address the root causes of rare diseases in many patients for whom there were no options before, such as spinal muscular atrophy, cystic fibrosis, and sickle cell disease; expand treatment options for cancer patients; and target therapies to responder populations.

As the primary federal agency conducting and supporting basic and translational research investigating the causes, treatments and cures for both common and rare diseases, NIH is leading scientific discovery for personalized medicines. Many institutes and centers at the NIH are supporting research that is 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), and the National Heart, Lung and Blood Institute (NHIBI). An increase for NIH in FY 2021 would protect its foundational role in the identification and development of personalized medicines.

II. Sustaining Basic and Translational Research

Scientific discovery begins with basic research that gathers fundamental knowledge about the genetic basis of a disease and with translational research aimed at applying that knowledge to develop a treatment or cure. Basic research has contributed to the development of more than 180 personalized medicines on the market and available for patients as of 2020.

The future of cancer care is expected to be profoundly influenced by the use of biomarkers that will guide researchers and physicians at every stage from drug development to
disease management. In 2018, 55 percent of all oncology trials involved the use of biomarkers, compared to 15 percent in 2000. According to NIH’s latest *Annual Report to the Nation on the Status of Cancer*, cancer death rates continued to decline 1.5 percent on average per year from 2001 to 2017 across all ages, genders, and racial and ethnic groups. This success can be attributed to significant progress in cancer prevention, early detection, and treatment as a result of investments in basic research.

Basic genomics research also offers opportunities beyond oncology, especially for rare diseases. Rare diseases affect an estimated 25 to 30 million Americans, and 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. In 2019, NIH awarded approximately $38 million in grants to 20 teams and a data management center to study a wide range of rare diseases. Research groups, which include scientists, clinicians, patients, families, and advocates, are collaborating on natural history studies, measuring treatment outcomes, and studying biomarkers that provide indicators of how a drug is working in patients. 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 options.

There are other people living with highly prevalent diseases that are still in need of better treatments and a cure. The Alzheimer’s Association estimates that 5.8 million Americans are living with Alzheimer’s disease. Despite increasing numbers of Alzheimer’s diagnoses, there are no treatments that can prevent or alter the course of the disease. Researchers are studying the genetic underpinnings of Alzheimer’s disease to more fully understand its complexity. The Accelerating Medicines Partnership for Alzheimer’s disease led by the NIH has identified over 500 drug targets. To build upon this progress, in 2019 the NIH launched two new research
centers focused on accelerating the discovery and development of treatments and cures for people living with Alzheimer’s.\textsuperscript{x}

The NIH is also leading efforts to develop tools and resources in gene therapy and artificial intelligence that will facilitate the identification and development of new personalized medicines for common and rare diseases. Wait times to produce vectors — or the “delivery vehicles” — in gene therapy and gene editing studies currently run one to two years due to their resource intensity.\textsuperscript{xi} Funding in FY 2021 would enable the NIH to create a consortium addressing this bottleneck to bringing new gene therapies to clinical trial for patients.

\textbf{III. Accelerating Personalized Medicine Research}

The 21st Century Cures Act (Cures Act) provided support for important initiatives that will benefit personalized medicine. The first initiative, the \textit{All of Us}™ Research Program, launched in May of 2018. \textit{All of Us} is collecting genetic and health information from one million volunteers for a decades-long research project. By mid-December 2019, over 305,000 individuals consented to participate and over 235,000 have fully enrolled. More than 80 percent of those individuals were from groups historically underrepresented in research, such as seniors, women, Hispanics and Latinos, African Americans, Asian Americans and members of the LGBTQ community.\textsuperscript{xii} This program is creating an invaluable biomedical data set that is inclusive of all Americans and will inform the development of new personalized medicines and plans to begin returning individual genetic results to participants this year.\textsuperscript{xiii}

\textit{All of Us} will continue to refine and streamline participant enrollment while focusing on retaining current participants. The program also plans to give researchers access to additional data that include participants’ genomic information, genetic propensity for disease or differential medication response, and visualization of basic electronic health record data for use in their
research to improve the diagnosis, treatment, and prevention of disease. To facilitate the use of data from *All of Us* and other cohort studies, the NHGRI plans to establish a new research program developing cutting-edge data and informatics tools for genomic research.xiv

The Cancer Moonshot is a second initiative supported by the *Cures Act*. It aims to transform the way cancer research is conducted by supporting immunotherapy networks, such as the Partnership for Accelerating Cancer Therapies (PACT). Through PACT, the NIH is collaborating with 11 pharmaceutical companies and the Foundation for NIH to identify, develop, and validate biomarkers to advance new cancer immunotherapy treatments. Improvements in immunotherapy over the past decade have driven declines in mortality from lung cancer and melanoma; however, progress in reducing rates for other cancers, including colorectal, breast, and prostate cancers, has slowed.xv These collaborations promise to discover new cancer treatments and harness the ability of the body’s immune system to fight cancer.

The *Cures Act* authorizes funding for these initiatives through the Innovation Fund. The $3 billion increase requested by PMC in FY 2021 would ensure that the $404 million authorized by the *Cures Act* this year would supplement the NIH’s base budget, as Congress intended, and thereby allow these important initiatives to continue.

**IV. Conclusion**

PMC appreciates the opportunity to highlight the NIH’s importance to the continued success of personalized medicine. The subcommittee’s support for a $3 billion increase over the NIH’s program level funding in FY 2020 will bring us closer to a future in which every patient benefits from an individualized approach to health care. PMC also commends the NIH’s leadership during the current COVID-19 pandemic, where NIH-funded basic research is critical to our understanding of how viruses express themselves across populations and individuals.