

Fiscal Year 2019 Senate Appropriations Committee Outside Witness Testimony

Submitted by
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May 29, 2018

Prepared for the
Subcommittee on Labor, Health and Human Services, Education & Related Agencies

Regarding
National Institutes of Health Fiscal Year 2019 Appropriations

Chairman Blunt, Ranking Member Murray 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) 2019 appropriations. PMC is a nonprofit education and advocacy organization comprised of more than 200 institutions from across the health care spectrum. **As the subcommittee begins work on the FY 2019 Labor, Health and Human Services, Education and Related Agencies appropriations bill, we ask that you include at least \$39.3 billion in funding for the NIH. Our request would raise NIH's base funding by \$2 billion over the final FY 2018 funding level and add \$215 million from the 21st Century Cures Act Innovation Account scheduled for NIH in FY 2019.**

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 and pre-empt the progression of disease as well as improve the quality, accessibility, and affordability of health care.¹

I. The Role of NIH in Personalized Medicine

Accounting for more than one of every four new drugs approved by the U.S. Food and Drug Administration (FDA) over the past four years,ⁱⁱ personalized medicine is a rapidly growing field. Biopharmaceutical companies nearly doubled their R & D investment in personalized medicines over five years, and expect to increase their investment by an additional third over the next five years.ⁱⁱⁱ According to the same survey, leading manufacturers also identified scientific discovery as the biggest challenge facing personalized medicine, followed closely by regulatory and reimbursement barriers.

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 basic and translational research informing the discovery of personalized treatments, including the National Human Genome Research Institute (NHGRI), the National Cancer

Institute (NCI), and the National Center for Advancing Translational Sciences (NCATS). An increase for NIH in FY 2019 would protect its foundational role in the identification and development of personalized medicines.

II. The Cures Innovation Fund: Accelerating Personalized Medicine Research

By passing the *21st Century Cures Act (The Cures Act)*, Congress acknowledged the need for NIH to accelerate basic research and provided funding for long-term initiatives, two of which will benefit personalized medicine. First, the *All of Us*TM Research Program will provide an unprecedented dataset of genetic information that promises to improve our understanding of the genetic basis of common and rare diseases. The program awarded its first four community partner awards this year to organizations well-positioned to engage and enroll communities usually underrepresented in biomedical research.^{iv} This was an initial step the *All of Us* Program took to collect genetic and health information from one million diverse volunteers. Second, the Cancer Moonshot initiative aims to make a decade's worth of cancer research progress in 5 years by transforming how cancer research is conducted. The initiative granted 142 awards, including grants in five areas of precision oncology, and established cancer research collaborations, including the Partnership for Accelerating Cancer Therapies (PACT). PACT is a public-private collaboration between the NIH and 11 biopharmaceutical companies to standardize the biological markers of cancer for new immunotherapy treatments. Immunotherapies have provided new treatment options for many patients who do not respond to other cancer therapies. Discoveries through PACT will help scientists understand why immunotherapies work for some but not all patients.

The *Cures Act* authorizes funding for these initiatives through the Cures Innovation Fund over the next 10 years for a total of \$4.8 billion; however, funding must be appropriated each year. An increase of \$215 million to the Cures Innovation Fund in FY 2019, as scheduled for NIH, would ensure these programs can continue their important research.

III. NIH Base Funding: Sustaining Basic and Translational Research

While the initiatives funded by the *Cures Act* are important for the growth of personalized medicine, 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. From 2003 to 2015, NIH lost more than 20 percent of its purchasing power.^v This loss of purchasing power, coupled with biomedical inflation, leaves NIH funding for basic and translational research short of where it needs to be to sustain the discovery and development of new personalized medicines.

Discovering New Biological Markers for Disease:

Basic research has led to the development of over 130 personalized medicines currently on the market and available for patients.^{vi} This includes novel cancer immunotherapies that harness a patient's immune system to fight cancer. This treatment is only possible thanks to the decades of basic research to understand how the immune system functions at the molecular level and the genetic characteristics of specific cancers. Basic genomics research also offers opportunities beyond oncology, especially with rare diseases. Rare diseases affect an estimated 25 to 30 million Americans, and with advances in genomics, the molecular cause of 6,500 rare diseases has been identified. However, only 500 of these rare diseases have approved treatments.^{vii}

Even though NIH's budget saw a major appropriations increase in FY 2018, at least 40 percent was designated to specific programs,^{viii} limiting the increase in funds available for basic and translational research. Reliable and consistent funding across all NIH institutes and centers will ensure basic research continues to identify new biological markers for disease.

Translating Discovery into Development:

Translational researchers require new resources and tools to bridge basic research discoveries with activities to develop treatments or cures. After decades of NIH-funded basic research, gene editing is enabling researchers to "correct" a genetic mutation causing a disease. The NIH launched the Somatic Cell Genome Editing program led by NCATS to accelerate the utilization of this technology by researchers in the development of new therapies. NIH has also recently released the PanCancer Atlas, a data set of molecular and clinical information from over 10,000 tumors representing 33 types of cancer. The project involved 150 researchers at more than two dozen institutions and was led by the NHGRI and NCI. The PanCancer Atlas provides an unparalleled resource for understanding the genetics of why, where, and how tumors arise. An increase in NIH base funding in FY 2019 will ensure translational research like this can continue for personalized medicine.

De-Risking Research and Development:

Developing a new treatment takes well over a decade; has a failure rate of more than 95 percent; and costs more than \$1 billion.^{ix} Not all discoveries lead to effective drug targets, and choosing the wrong biological target can result in costly failures late in the drug development process. The NIH's Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs invest in companies to incentivize high-risk research on new drugs and therapies.

NIH also establishes collaborations to reduce the risk of developing new personalized medicines. As a public-private partnership between the NIH, the FDA, 12 biopharmaceutical and life science companies, and 13 nonprofit organizations, the Accelerating Medicines Partnership (AMP) seeks to change the current model for the development of new diagnostics and treatments starting with Alzheimer's disease, type 2 diabetes, lupus, and Parkinson's disease. Industry and nonprofit participants only account for 26 percent of the program's funding. AMP promises to shorten timelines, cut costs, and increase the success rates of treatment development by pinpointing the right biological targets early in drug development. Robust funding will empower NIH to continue de-risking research and supporting industry through collaborations like these that have the potential to improve clinical trials success rates, including those for personalized medicines.

IV. Conclusion

PMC appreciates the opportunity to highlight the NIH's important contributions to the success of personalized medicine. The subcommittee's support for a \$2 billion increase in base funding, plus the \$215 million increase scheduled through the *Cures Act's* Innovation Account, 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/The-Personalized-Medicine-Report1.pdf>
- ⁱⁱ http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
- ⁱⁱⁱ <http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/pmc-phrma-personalized-medicine-investment-21.pdf>
- ^{iv} <https://www.nih.gov/about-nih/who-we-are/nih-director/testimony-implementation-21st-century-cures-act-progress-path-forward-medical-innovation>
- ^v <https://www.nih.gov/about-nih/who-we-are/nih-director/fiscal-year-2016-budget-request>
- ^{vi} <http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf>
- ^{vii} <https://www.nih.gov/about-nih/who-we-are/nih-director/fiscal-year-2018-budget-request>
- ^{viii} https://appropriations.house.gov/uploadedfiles/03.21.18_fy18_omnibus_-_labor_health_and_human_services_-_summary.pdf
- ^{ix} <https://www.nih.gov/research-training/accelerating-medicines-partnership-amp>