Fiscal Year 2020 House Appropriations Committee Subcommittee on Labor, Health and Human Services, Education and Related Agencies Appropriations Testimony
Cynthia A. Bens, Senior Vice President, Public Policy, Personalized Medicine Coalition
April 8, 2019

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) 2020 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 2020 Labor, Health and Human Services, Education and Related Agencies appropriations bill, **we ask that the NIH receive an appropriation of at least $41.6 billion in FY 2020, a $2.5 billion, or 6.4 percent, increase over FY 2019 program level funding.** This funding level would allow for meaningful growth above inflation in the base budget that 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 and pre-empt the progression of disease as well as improve the quality, accessibility, and affordability of health care.[i]

I. The Role of NIH in Personalized Medicine

The number of personalized medicines approved by the U.S. Food and Drug Administration (FDA) annually has increased from 5 percent of new drugs in 2005[ii] to a record 42 percent in 2018.[iii] For each of the past four years, personalized medicines have accounted for more than a quarter of new drugs approved in the United States.[iv] Biopharmaceutical companies nearly doubled their R & D investment in personalized medicines over the last five years, and expect to increase their investment by an additional third over the next five years.[v] Leading manufacturers have 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 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 2020 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 130 personalized medicines on the market and available for patients as of 2017.[vi] These include novel cancer immunotherapies that harness a patient's immune system to fight cancer.[vii] This direction of treatment was 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 cancer types. NCI-MATCH, a phase 2 clinical trial, is now investigating whether targeted therapies for people whose tumors have certain gene mutations will be effective regardless of their cancer type. Patients from more than 1,100 cancer centers and community hospitals in every state have enrolled in the trial, and 62.5 percent of the first 6,000 patients enrolled had tumors other than the four most common cancers: breast, colorectal, non-small cell lung and prostate. [viii]

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. Sickle cell disease (SCD), a rare disease that affects approximately 100,000 people in the United States, is the first "molecular disease." The molecular basis of SCD was discovered decades ago but limited symptomatic treatments are available to patients. Novel genetic approaches are now providing hope for a SCD cure within 10 years. The NIH-launched Cure Sickle Cell Initiative is advancing the most promising next-generation SCD therapies and recently reported impressive results from one experimental gene therapy.[ix]

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 diagnosis, 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. In 2018, an NIH-funded team of researchers reported that groups of genes associated with specific biological processes are "genetic hubs." These hubs are an important part of the disease

process. This and other large studies involving the NIH will continue to allow for better prioritization of genes to target for treatment.[x]

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 *All of Us*™ Research Program, launched in May of 2018. *All of Us* is collecting genetic and health information from one million volunteers for a decades-long research project. By August 15, 2018, nearly 100,000 individuals enrolled as participants. Almost 50 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.[xi] This program is creating an invaluable biomedical data set that is inclusive of all Americans and will inform the development of new personalized medicines.

This year the NIH will begin a number of *All of Us* pilot projects aimed at getting new types of data for the research community and giving health information back to participants. For the research community, we expect to see pilots related to genotyping, whole genome sequencing, lab test assays, and wearable devices. *All of Us* will also begin a pilot on the responsible return of information to participants that will include their genomic information, genetic risk for disease, medication response, and visualization of basic electronic health record data. To further support *All of Us* participants, the NIH is developing a mobile app and other educational resources about developments in personalized medicine.[xii]

The Cancer Moonshot is the second initiative supported by the *Cures Act*. It aims to transform the way cancer research is conducted. Because of funding for the Cancer Moonshot, NIH was able to issue 17 Funding Opportunity Announcements, including opportunities to

support immunotherapy networks that promise to discover new cancer treatments for adults and children.[xiii] In addition to funding research aimed at improving cancer treatment, as part of the Cancer Moonshot the NCI was able to make a resource publicly available on thousands of inherited *BRCA1* and *BRCA2* gene variants. Certain inherited variants in these genes can increase the risk of breast, ovarian, and other cancers by varying degrees, whereas others are not associated with disease. Clinicians and patients need to know whether a given variant is likely to be disease-associated and how likely a variant is to cause cancer. Until the NIH's BRCA Exchange, the available data on the inherited *BRCA* variants were not aggregated in a comprehensive way or made available to clinicians, researchers, data scientists, patients, and patient advocates.[xiv]

The *Cures Act* authorizes funding for these initiatives through the Innovation Fund. The $2.5 billion increase requested by PMC in FY 2020 would ensure that the $492 million authorized by the *Cures Act* this year would supplement the NIH's base budget as Congress intended and 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 $2.5 billion increase over the NIH's program level funding in FY 2019 will bring us closer to a future in which every patient benefits from an individualized approach to health care. PMC will gladly provide additional information on the programs described in our testimony upon request.

---

[i] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[ii] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[iii] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[iv] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[v] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/pmc-phrma-personalized-medicine-investment-21.pdf
[vi] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[vii] https://officeofbudget.od.nih.gov/pdfs/FY19/br/Overview.pdf
[viii] https://www.nih.gov/news-events/news-releases/nci-match-precision-medicine-clinical-trial-releases-new-findings-strengthens-path-forward-targeted-cancer-therapies

[ix] https://directorsblog.nih.gov/2018/12/11/accelerating-cures-in-the-genomic-age-the-sickle-cell-example/
[x] https://www.nia.nih.gov/news/data-sharing-uncovers-five-new-risk-genes-alzheimers-disease
[xi] https://www.nih.gov/about-nih/who-we-are/nih-director/testimony-prioritizing-cures-science-stewardship-national-institutes-health
[xii] https://allofus.nih.gov/sites/default/files/dish_new_year.docx
[xiii] https://www.nih.gov/about-nih/who-we-are/nih-director/testimony-21st-century-cures-implementation-updates-fda-nih
[xiv] https://www.nih.gov/news-events/news-releases/brca-exchange-aggregates-data-thousands-brca-variants-inform-understanding-cancer-risk