Fiscal Year 2022 Senate Appropriations Committee Outside Witness Testimony
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Subcommittee on Labor, Health and Human Services, Education and Related Agencies
National Institutes of Health Fiscal Year 2022 Appropriations

Chairwoman Murray, Ranking Member Blunt 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) 2022 appropriations and the importance of the agency's 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. The tragically uneven effects of the COVID-19 pandemic have underlined the importance of developing more targeted health care interventions just as groundbreaking technologies are giving us an unprecedented ability to understand the biological and environmental factors that drive disease and influence patients' responses to various treatments. As the subcommittee begins work on the FY 2022 Labor, Health and Human Services, Education and Related Agencies appropriations bill, **we strongly support the President's proposed increase in funding for NIH to $51 billion, and we request the agency receive no less than $46.1 billion for NIH's base program level budget, $3.2 billion above the comparable FY 2021 funding level.**

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 government spending on science at this pivotal moment, 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

Continued research on the genetic and biological underpinnings of disease has made it possible to develop new personalized medicine treatments for cancers as well as rare, common, and infectious diseases. This research has informed the development of more than 286 personalized treatments[ii] and over 166,703 genetic testing products[iii] available for patients in 2020. Foundational advances in genetic and genomic technologies have also paved the way for scientists' rapid response to COVID-19. The rapid progress we have seen, from mRNA vaccine development, diagnostic testing, and variant sequencing, to beginning to understand how human genomic variation influences infectivity, disease severity, vaccine efficacy, and treatment response, relies on years of personalized medicine research,[iv,v] — as well as years of diligent funding from Congress to support this research.

The widely variable effects of COVID-19 have only highlighted the need for personalized medicine to move further and faster. A $3.2 billion increase would allow for NIH's base budget to keep pace with biomedical inflation and allow meaningful growth of 5 percent. This request also includes the full $496 million NIH is scheduled to receive in FY 2022 from the Innovation Account established in the *21st Century Cures Act (Cures Act)*.

II. Sustaining Basic and Translational Research for Personalized Medicine

NIH is leading 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 the NIH are supporting research 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), the National Heart, Lung and Blood Institute (NHLBI), and the National Center for Advancing Translational Sciences (NCATS). An increase for NIH in FY 2022 would protect its foundational role in the identification and development of treatments, technologies, and tools for personalized medicine.

The future of cancer care, for example, is expected to be profoundly influenced by personalized medicine approaches for detecting and treating early- and late-stage cancers. In 2020, for example, FDA approved the first comprehensive pan-tumor liquid biopsy test for patients with advanced cancer that allows physicians to detect actionable biomarkers in patients' blood through next-generation sequencing.[vi] As soon as next year, NCI aims to launch large national trials for similar tests that are being developed to detect multiple early-stage cancers in patients' blood.[vii] These tests would provide less invasive testing options that can detect cancers 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, and in 2019, the estimated economic cost of only 379 rare diseases reached nearly $1 trillion in the U.S.[viii] Over the past decade, NIH has helped shift the scientific approach to researching rare diseases from one disease at a time to many diseases. 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 others living with highly prevalent diseases where personalized medicine can offer patients better treatments or a cure. The Alzheimer's Association estimates that 6.2 million Americans are living with Alzheimer's disease, for example.[ix] Despite increasing numbers of Alzheimer's diagnoses and FDA's recent approval of the first new Alzheimer's drug in decades, researchers are still studying 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 for Alzheimer's disease led by NIH has identified over 500 drug targets, and in 2020 launched a second iteration of the partnership to enable a personalized medicine approach to researching new treatments.[x]

Still, ensuring that the 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. Improving research policies and incorporating diverse perspectives into solving complex scientific problems, such as through NIH's UNITE initiative and NHGRI's action agenda for a diverse genomics workforce, will play a key role in addressing these disparities, in addition to research on improving minority health and understanding factors contributing to health disparities.

III. Accelerating Personalized Medicine Research

Increasing the 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 initiative, the *All of Us*™ Research Program, was launched in 2018 to begin collecting genetic and health information from one million volunteers as part of a decades-long research project. As of May 2021, over 382,000 individuals consented to participate and over 279,000 have fully enrolled.[xi] More than 80 percent of those individuals are from groups historically underrepresented in research,[xii] such as seniors, women, Hispanics and Latinos, African Americans, Asian Americans and members of the LGBTQ community. Last year, program officials met their targets to start returning individual genetic results to participants and inviting researchers to begin using the data collected.[xiii] The program also began analyzing data from its diverse participant cohort to look for patterns explaining individuals' different responses to COVID-19.[xiv] In the future, pooling health care data across large datasets will play a key role in advancing research for personalized medicine approaches to care.

The second initiative, the Beau Biden Cancer Moonshot, aims to transform the way cancer research is conducted by fostering collaboration and data sharing. Moonshot currently supports over 240 new research projects,[xv] including the Partnership for Accelerating Cancer Therapies (PACT). Through PACT, the NIH is collaborating with 12 pharmaceutical companies, the Foundation for NIH, and FDA to identify, develop, and validate biomarkers to advance the discovery of new immunotherapy treatments. Over the past decade, personalized treatments harnessing the immune system have driven declines in mortality for lung cancer and melanoma.

IV. Conclusion

PMC appreciates the opportunity to highlight the NIH's importance to the continued success of personalized medicine. As the subcommittee considers the President's proposal, we encourage the subcommittee to support at least a $3.2 billion increase for existing centers and programs, in addition to funding Congress may provide for targeted initiatives such as establishing the President's proposed Advanced Research Projects Agency for Health (ARPA-H). PMC believes that diligently funding basic and translational research at the 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://doi.org/10.1016/j.cell.2021.01.015
[v] https://doi.org/10.1038/s41586-020-2817-4
[vi] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM_at_FDA_The_Scope_Significance_of_Progress_in_2020.pdf
[vii] https://www.precisiononcologynews.com/policy-legislation/nci-director-sharpless-outlines-ideas-aggressively-lower-cancer-deaths
[viii] https://everylifefoundation.org/burden-study/
[ix] https://www.alz.org/media/Documents/alzheimers-facts-and-figures.pdf
[x] https://www.nih.gov/research-training/accelerating-medicines-partnership-amp/alzheimers-disease
[xi] https://www.joinallofus.org/newsletters/2021/may
[xii] https://doi.org/10.1016/j.cell.2021.01.015
[xiii] https://www.joinallofus.org/newsletters/2020/december
[xiv] https://www.nih.gov/news-events/news-releases/all-us-research-program-launches-covid-19-research-initiatives
[xv] https://doi.org/10.1016/j.ccell.2021.04.015