American Society of Human Genetics

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August 2, 2024

The Honorable Diana DeGette
House Energy and Commerce Committee
United States House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Larry Bucshon, MD
House Energy and Commerce Committee
United States House of Representatives
2313 Rayburn House Office Building
Washington, DC 20515

Dear Representatives DeGette and Bucshon,

The American Society of Human Genetics (ASHG), the world's largest organization for human genetics and genomics professionals, appreciates this opportunity to provide comments that build on the foundation laid out in the 21st Century Cures Act and Cures 2.0. Our approximately 8,000 members are researchers, medical geneticists, genetic counselors, and others who share the common goal of people everywhere realizing the full potential and benefits of human genetics and genomics research.

The 21st Century Cures Act changed the way that biomedical research is translated from the bench to the bedside, including the creation of innovative scientific initiatives at the National Institutes of Health (NIH) such as the *All of Us* Research Program. *All of Us* is a historic effort established by Congress to collect and study data from one million or more people living in the U.S.

<u>Striving for Health Equity and Research Cohort Diversity</u>
Shortly after the law was implemented, NIH enhanced its reporting requirements for individuals in agency-funded Phase III clinical trials based on their gender, ancestry and/or ethnic background, building on the commitment to enhance research cohort diversity exemplified by the *All of Us* program. This is of crucial importance. While human beings are 99.9 percent identical in our genetic makeup, the variation in the remaining 0.1% can influence a person's risk of disease or how the body responds to medications, stress, or environmental factors. It is imperative for participation in genetics and genomics research to reflect humanity's diversity so that all people can enjoy its benefits and so that genetic discoveries can be equitably applied in healthcare.

We were very pleased to see that proposals for a successor to Cures built upon these efforts, specifically with respect to increasing diversity in biomedical research. ASHG's commitment to diversity, equity and inclusion remains a core component of our priorities and organizational culture, and we support efforts to increase recognition and incorporation of human genetic diversity in research. This will take a concerted effort by the

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research community and research funders and must include greater engagement with individuals and communities underrepresented in research.

ASHG urges Congress in future legislation to support NIH institutes and programs that emphasize diversity and equity in research and clinical trials participation by the public. ASHG urges Congress also to provide sufficient funding for researchers to engage populations underrepresented in research. Sufficient funding to fully support the inclusion of pediatric populations in *All of Us* would greatly enhance the scientific validity and usefulness of the research resource, while also ensuring representation of individuals at all stages of life.

Robust and Sustained Funding Sources for Human Genetics and Genomics

We applaud Congress for initially authorizing the *All of Us* program in the 21st Century Cures Act and providing funding for this crucial program. As it stands, funding for the *All of Us* program will be sunsetting as soon as funds are depleted in fiscal year (FY) 2026. Given the long-term data collection required to advance precision medicine, the initial investments made in the *All of Us* program will be rendered futile without additional funds to continue the program. **ASHG urges Congress to continue to build on the successes of the 21st Century Cures Act and Cures 2.0 by establishing a robust and sustained funding source to support the *All of Us* Research Program and bolster the future of precision medicine**. **In addition, authorizing year-on-year funding increases of five percent for a new iteration of Cures would allow for meaningful growth of the programs it supports.**

A significant proportion of the Innovation Fund in Cures was implemented via the "Other Transaction" OT2 funding mechanism. Now that NIH has had several years of experience implementing programs through OT2, it would be a useful time to evaluate the value of this mechanism and its associated review process compared to other funding mechanisms, particularly for programs focused on large-scale generation, collection, and recruitment. Additionally, given the nature of appropriations, consistent annual increases are preferable to the substantial fluctuations seen in the previous Cures legislation.

To continue to spur innovation in treating rare diseases that disproportionately affect children, we propose that reauthorization of the Rare Pediatric Disease Priority Review Voucher (PRV) Program be included in the next generation of Cures legislation. The 21st Century Cures Act created the PRV program to incentivize pharmaceutical companies to develop products to diagnose, prevent, and treat rare pediatric diseases. Developing treatments for rare pediatric diseases is challenging due to the small number of affected individuals, few financial incentives, complications in conducting clinical trials with children, and delays in diagnosis among other factors. A four-year extension of the program was passed as part of the appropriations omnibus in December 2020; however, to qualify for a PRV, companies must have a rare disease designation prior to September 30, 2024. Furthermore, the U.S. Food and Drug Administration will be unable to issue PRVs after September 30, 2026, unless an extension is passed by Congress.

Harnessing Technology to Address Grand Challenges in Biomedical Research

With advances in technology, we now have the opportunity to use data science and artificial intelligence (AI) to address the grand challenges in biomedical research, including integrating genomics with other types of data and using AI to assist with designing truly personalized therapeutics. Human genetics and genomics can be most

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impactful via its integration in prevention, diagnostics, and treatment to address health challenges, particularly for cancer and rare diseases. Data sharing has been common practice in genetics and genomics research for decades, accelerating biomedical discoveries and improving human health. Supporting increasingly comprehensive repositories to house these data requires stable funding. **ASHG urges Congress to provide stable funding with regular increases to NIH to ensure that genetic and genomic data remain accessible to researchers in ways that advance science, reduce regulatory burdens, and also protect the privacy of participants and minimize the stigmatization of groups of people.**

Supporting the Human Genetics and Genomics Research Workforce

In addition, strengthening the research workforce pipeline was a key goal of Cures 2.0. This effort should continue into any new iterations of legislation to support increasing diversity of the human genetics and genomics workforce. Increasing the diversity of the biomedical research workforce also positively impacts scientific innovation, global competitiveness, and the ability to deliver new insights into human disease and health. **ASHG recognizes that investments in a diverse research workforce make a strong contribution to the U.S. economy and play a critical role in our nation's ability to maintain global competitiveness.**

Structural Reforms to NIH Require Input for Scientists

Support for NIH and the research infrastructure is critical to improving the lives of patients with a wide spectrum of diseases and disorders, many of whom depend on human genetics and genomics research for prevention, diagnosis, and treatment of disease. ASHG members are at the forefront of innovations in genomics that accelerate breakthroughs and drive our nation's economy. This research is made possible with crucial funding from NIH institutes and centers. **Any restructuring of agencies like the NIH can and should be addressed through a bipartisan and bicameral approach involving active engagement of the biomedical research community and with an open and transparent process.** ASHG looks forward to working with members on both sides of the aisle and in both the House and Senate to provide input on proposed reforms to the NIH in the weeks and months ahead.

Thank you for your ongoing support for biomedical research, and the opportunity to comment on a next-generation Cures bill. We welcome the opportunity to work with you and your staff as you further consider ways to revolutionize biomedical research. Please feel free to contact Karina Miller (kmiller@ashg.org) with any questions.

Sincerely,

Bruce D. Gelb, M.D.
President, American Society of Human Genetics