American Society of Gene + Cell Therapy

June 13, 2025

The Honorable Shelley Moore Capito
Chair
Senate Appropriations Committee
Subcommittee on Labor-HHS-Education
United States Senate
Washington, DC 20510

The Honorable Tammy Baldwin
Ranking Member
Senate Appropriations Committee
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RE: Testimony submitted by the American Society of Gene and Cell Therapy. Prepared for the Senate Appropriations Committee Subcommittee on Labor, Health and Human Services, Education, and Related Agencies, addressing FY26 Appropriations for the National Institutes of Health and Advanced Research Projects Agency for Health within the Department of Health and Human Services.

Chair Capito, Ranking Member Baldwin, and members of the Subcommittee on Labor, HHS, Education, and Related Agencies:

Thank you for the opportunity to submit testimony regarding fiscal year 2026 (FY26) appropriations for the National Institutes of Health (NIH) and the Advanced Research Projects Agency for Health (ARPA-H). The American Society of Gene and Cell Therapy (ASGCT) is a nonprofit professional membership organization that is comprised of more than 6,400 scientists, physicians, patient advocates, and other professionals working in cell and gene therapy (CGT) in settings such as universities, hospitals, and biotechnology companies.

The mission of ASGCT is to advance knowledge, awareness, and education leading to the discovery and clinical application of genetic and cellular therapies to alleviate human disease. ASGCT's strategic vision is to be a catalyst for bringing together diverse stakeholders to reshape the practice of medicine by incorporating the use of these transformative therapies. Many of ASGCT's members have spent their careers in this field performing the underlying research that has led to today's expansive pipeline of CGTs.

The Transformative Pipeline of Cell and Gene Therapies

CGTs represent one of the most significant advances in modern medicine, addressing the root causes of disease by modifying gene expression or repairing abnormal genes. These therapies typically require only a single administration and are often durable and potentially curative, providing hope for patients with diseases that previously had few or no treatment options.

The field has achieved remarkable milestones. Since the FDA's approval of the first gene therapy in 2017,[1] we now have 8 approved CAR T-cell therapies for blood cancers and 14 gene therapies for conditions including sickle cell disease and hemophilia.[2]

[1] US Food and Drug Administration. (2017). *BLA Approval - STN: BL 125646/0.* https://www.fda.gov/media/106989/download?attachment

[2] US Food and Drug Administration. (Accessed April 2025*). Approved Cellular and Gene Therapy Products*. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products

The development pipeline for CGT includes over 4,400 therapies ranging from preclinical through pre-registration stages.[3] According to one estimate, 45% of the total global burden of disease could be treated with existing biotechnologies.[4] These technologies are rapidly breaking down barriers that once seemed insurmountable, transforming theoretical possibilities into life-saving realities. One of the most striking recent breakthroughs made possible by NIH-supported research is the treatment of Baby KJ,[5] the first patient to receive a personalized CRISPR gene-editing therapy. Diagnosed with a rare, life-threatening genetic condition shortly after birth, KJ was treated with a custom therapy developed as a result of decades of federal investment in research and discovery.

While the CGT pipeline is strong, continued investments in basic, translational, and early clinical research are essential. Each genetic disease essentially requires a different therapeutic product, and different diseases require specially designed delivery vectors targeting specific organs and cell types. Limited medical and scientific knowledge exists for many rare indications, making continued research funding critical to closing knowledge gaps.

NIH: The Foundation of Scientific Innovation

ASGCT joins the research community in recommending at least $51.303 billion for the National Institutes of Health (NIH) for FY26,[6] representing a $2.4 billion increase (4.7%) from FY25 enacted levels.

Scientists at the NIH have provided foundational resources for biomedical research. For example, the National Human Genome Research Institute's (NHGRI) Human Genome Project successfully sequenced over 90% of the human genome. Additionally, scientists at the NIH Intramural Research Program[7] have contributed to major breakthroughs in treating genetic diseases such as sickle cell disease (SCD).[8,9] Reliable investment in basic, translational, and early clinical research is necessary to build the scientific underpinnings of CGTs. There remains a great scientific need to investigate the preclinical application of gene therapies to treat new diseases and improve the design of technologies, so that therapies currently in the clinic or advancing toward clinical trials have a better chance of success.

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[3] American Society of Gene & Cell Therapy & Citeline. (2025). *Gene, Cell, & RNA Therapy Landscape Report: Q1 2025 Quarterly Data Report.* https://www.asgct.org/publications/landscape-report

[4] Chui, M. et al. (2020). *The Bio Revolution: Innovations Transforming Economies, Societies, and Our Lives.* https://www.mckinsey.com/industries/life-sciences/our-insights/the-bio-revolution-innovations-transforming-economies-societies-and- our-lives.

[5] Penn Medicine & Children's Hospital of Philadelphia. (May 15, 2025). *World's first patient treated with personalized CRISPR gene editing therapy at Children's Hospital of Philadelphia.* https://www.pennmedicine.org/news/worlds-first-patient-treated-with-personalized-crispr-therapy

[6] Ad Hoc Group for Medical Research. (2025). *The Ad Hoc Group Fiscal Year 2026 Recommendation [Sign-on letter].* https://www.asgct.org/advocacy/policy-statement-landing/2025/ad-hoc-medical-research-group-nih-funding-sign-on

[7] National Institutes of Health. (Accessed April 2025). *Intramural Research Program*. https://irp.nih.gov/

[8] Duncombe, C. (2022). Discovering a Cure for Sickle-Cell Disease. *The NIH Catalyst.* https://irp.nih.gov/catalyst/28/3/discovering- a-cure-for-sickle-cell-disease

[9] MacDougall, R. (2015). *NIH Researchers Tackle Thorny Side of Gene Therapy.* https://www.genome.gov/news/news-release/NIH- researchers-tackle-thorny-side-of-gene-therapy

A vital component of NIH funding is Facilities & Administrative (F&A) cost reimbursements. These funds are neither supplemental nor insignificant.[10] They are integral to CGT research, supporting critical functions including:

- Manufacturing and Materials: Complex patient cell and vector production facilities, specialized equipment like bioreactors and chromatography systems, and the biomedical engineering staff who maintain these sophisticated apparatuses.
- Research Outputs: Research design and compliance programs, specialized disposal services for biological waste, and advanced data systems including cloud-based data management and high-performance computing resources.

CGT research requires high upfront investments in specialized staff, machinery, and biological components that generally cannot be supported by any single research grant. F&A reimbursements enable institutional efficiency and scientific advancement. Any cuts to F&A would severely curtail both institutional efficiency and the speed of scientific discovery.

ARPA-H: Accelerating Revolutionary Health Breakthroughs

ASGCT requests the Subcommittee provide at least $1.7 billion for ARPA-H, to be available through FY28.[11]

ARPA-H's unique mission within HHS focuses on revolutionary rather than incremental ideas, complementing commercial sector efforts by tackling challenges industry may consider too risky. Since its formation, ARPA-H has launched over 20 programs supporting projects led by scientists, engineers, and entrepreneurs nationwide.[12] These efforts include developing biomaterials to help joints heal themselves, rapidly manufacturing 3D tumor models, designing implantable cancer monitoring technologies, and creating therapies using electrical signals to treat metabolic disorders.

ARPA-H has activated a nationwide network of over 1,000 organizations across all fifty states, ensuring Americans in all regions can contribute to breakthrough medical innovations. The agency's entrepreneurial approach and culture of experimentation allow the most creative ideas from the CGT research community to thrive.

Economic Impact and Global Competitiveness

The federal investment in biomedical research generates substantial economic returns. Every $1 of NIH funding generates $2.56 in economic activity after one year, and each $1 of NIH basic research stimulates an additional $8.38 of industry R&D investment after eight years. Additionally, NIH funding supported 407,782 jobs across all 50 states and Washington, DC in FY24.[13]

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[10] American Society of Gene & Cell Therapy. (March 4, 2025). *Sign-on Letter to Congressional Appropriators on NIH FY26 Funding and Facilities & Administrative cost reimbursements.* https://www.asgct.org/advocacy/policy-statement-landing/2025/nih-indirect-funding-sign-on-letter

[11] Research!America. (2025). *Advanced Research Projects Agency for Health Fiscal Year 2026 Recommendation [Sign-on letter].* https://www.asgct.org/advocacy/policy-statement-landing/2025/arpah-fy26-funding-sign-on

[12] Advanced Research Projects Agency for Health. (2025). *Explore Funding: Programs.* https://arpa-h.gov/explore-funding/programs

[13] United for Medical Research. (2025). *NIH's Role in Sustaining the U.S. Economy: 2025 Update.* https://www.unitedformedicalresearch.org/annual-economic-report/

Beyond immediate economic benefits, increased NIH and ARPA-H funding enhances US global competitiveness, promotes national security, establishes viable career paths for the next generation of scientists, and generates high-quality jobs in communities nationwide.[14]

Conclusion
CGTs hold immense potential to address numerous complex diseases that currently lack effective treatments. Strong federal funding for NIH, including F&A reimbursements, along with robust support for ARPA-H, represents a critical investment in American scientific leadership, patient health, and economic prosperity.

ASGCT appreciates your attention to this critical matter. If you would like to hear more from ASGCT's member experts, please reach out to Margarita Valdez Martínez, Chief Advocacy Officer, at mvaldez@asgct.org.

Sincerely,

David Barrett
Chief Executive Officer
American Society of Gene & Cell Therapy

[14] National Security Commission on Emerging Biotechnology. (2025). *Charting the Future of Biotechnology.* https://www.biotech.senate.gov/final-report/chapters/