May 4, 2026

The Honorable Susan Collins
Chair, Senate Appropriations Committee
413 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Patty Murray
Vice Chair, Senate Appropriations Committee
154 Russell Senate Office Building
Washington, DC 20510

The Honorable Tom Cole
Chair, House Appropriations Committee
2207 Rayburn House Office Building
Washington, DC 20510

The Honorable Rosa DeLauro
Ranking Member, House Appropriations Committee
2413 Rayburn House Office Building
Washington, DC 20510

Dear Chair Collins, Vice Chair Murray, Chair Cole, and Ranking Member DeLauro,

On behalf of the undersigned organizations, we thank you for your support of the National Institutes of Health (NIH). We ask that you provide at least $51.303 billion for NIH in FY27, and that you exercise your administrative oversight authority to ensure timely disbursement of appropriated funds. NIH-funded research is critical for population health, the US economy, and maintaining our global position as the pinnacle of biomedical progress. NIH funding has supported an untold number of breakthroughs for patients, providing hope for those facing both rare and common diseases.

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 provide hope for patients with diseases that previously had few or no treatment options. The field has achieved remarkable milestones: since the US Food and Drug Administration's (FDA) approval of the first gene therapy in 2017,[1] the US now has 7 approved CAR T-cell therapies for blood cancers, 16 gene therapies for conditions including sickle cell disease and inherited vision loss, and a number of additional genetic-based medicines treating a wide range of diseases.[2] According to one estimate, 45% of the total global burden of disease could be treated with existing biotechnologies – but they require ongoing development support.[3]

The development pipeline for CGT includes over 4,100 therapies ranging from preclinical through pre-registration stages.[4] One of the breakthroughs made possible by NIH-supported research is the treatment of Baby KJ,[5,6] 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 and celebrated his 1st birthday in August 2025. Like KJ,

[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 August 2025*). Approved Cellular and Gene Therapy Products*. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products
[3] 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.*
[4] American Society of Gene & Cell Therapy & Citeline. (2025). *Gene, Cell, & RNA Therapy Landscape Report: Q4 2025 Quarterly Data Report. https://www.asgct.org/publications/landscape-report*
[5] Bhattacharaya, J. (2026). *Written Testimony: House Appropriations Hearing | March 17, 2026.* https://docs.house.gov/meetings/AP/AP07/20260317/119055/HHRG-119-AP07-Wstate-BhattacharyaJ-20260317.pdf
[6] 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*

patients who would most benefit from CGTs often have conditions that progress quickly over time. It is essential that momentum and funding be sustained in an uninterrupted fashion, so that these patients will not forever lose their opportunity to benefit from this technology.

CGT research requires multi-year commitments due to complex preparation and development processes. Researchers must be able to rely on NIH as a stable, predictable funder to successfully navigate these extended research cycles. Basic research teams continually expand upon and improve previous discoveries, utilizing the iterative scientific process to uncover genetic causes of disease and design safer viral vectors,[7] more effective gene editors,[8] or new ways to deliver CAR Ts.[9] Each advance brings with it the chance to build efficiency and reduce development costs. Translational research comes next – critical bridging studies which help advance new technologies toward clinical research. Finally, clinical trials for CGTs require years of long-term data to monitor patient safety and measure the duration of therapeutic effect.[10] When funding is unexpectedly canceled or delayed, researchers may lose years of progress and critical data, potentially delaying breakthrough treatments for diseases as varied as cancer, Huntington's disease, lupus, and pediatric rare diseases.

NIH funding also provides profound economic benefits. In FY25 the National Institutes of Health (NIH) awarded $36.58 billion in research grants to organizations in the 50 U.S. states and the District of Columbia. That funding supported nearly 400,000 jobs and nationally led to $94.15 billion in new economic activity. Additionally, every $1 of NIH basic research stimulates an additional $8.38 of industry R&D investment after 8 years.[11,12]

The undersigned organizations represent the scientists, physicians, patient advocates, and other professionals developing CGTs across the United States. CGTs have already improved the lives of thousands of US patients, their families, and communities. For that work to continue, ongoing, stable investment via NIH is imperative.

For these reasons, we again urge Congress to **protect robust NIH appropriations for FY27 and work to ensure those appropriated funds are disbursed promptly.** Thank you for your leadership to ensure continued bipartisan support for this uniquely American institution.

Sincerely,

American Society of Gene & Cell Therapy
Alport Syndrome Foundation
A Foundation Building Strength for Nemaline Myopathy
Alliance for Regenerative Medicine
American College of Medical Genetics and Genomics
American Epilepsy Society

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[7] American Society of Gene & Cell Therapy. (2024). *Viral Vectors.* https://patienteducation.asgct.org/understanding-cell-gene-therapy/viral-vectors
[8] American Society of Gene & Cell Therapy. (2025). *Gene Editing.* https://patienteducation.asgct.org/understanding-cell-gene-therapy/types-of-cell-gene-therapy/gene-editing
[9] American Society of Gene & Cell Therapy. (2024). *CAR T-Cell Therapy.* https://patienteducation.asgct.org/understanding-cell-gene-therapy/types-of-cell-gene-therapy/car-t-cell-therapy
[10] US Food and Drug Administration. (2020). *Long Term Follow-Up After Administration of Human Gene Therapy Products*. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/long-term-follow-after-administration-human-gene-therapy-products
[11] United for Medical Research. (2026). *2026 Update: NIH's Role in Sustaining the US Economy.* https://www.unitedformedicalresearch.org/annual-economic-report/
[12] National Institutes of Health. (2025). *Spurring Economic Growth.* https://www.nih.gov/about-nih/impact-nih-research/serving-society/spurring-economic-growth

American Society for Transplantation and Cellular Therapy
Angelman Syndrome Foundation
APS Type 1 Foundation
Arcanist Foundation
Association of Cancer Care Centers
ASXL Rare Research Endowment (ARRE) Foundation
Blood Cancer United
Bubba's Light, Inc.
CACNA1A Foundation
Cancer Support Community
CancerCare
Child Neurology Foundation
Children's Cardiomyopathy Foundation
Children's Hospital Association
Children's Hospital of Philadelphia
The Children's Medical Research Foundation, Inc.
Children's National Research Institute at Children's National Hospital
Children's Tumor Foundation
Cincinnati Children's Hospital
CLL Society
Coalition to Cure Calpain 3
Coalition to Cure CHD2
Cockayne Syndrome Foundation Inc
CSNK2A1 Foundation
CTNNB1 Foundation, The Gene Therapy Research Institute
Cure CMD
CURE GABA-A
Cure LBSL
Cure LGMD2i Foundation
Cure Sanfilippo Foundation
CURE SYNGAP1
Cure Tay-Sachs Foundation
CureCMT4J/Talia Duff Foundation
Cystic Fibrosis Foundation
Dana-Farber Cancer Institute
Danon Disease Foundation
Department of Genetic and Cellular Medicine and Horae Gene Therapy Center, UMass Chan Medical School
Dravet Syndrome Foundation
Dup15q Alliance
Duplication Cares
EB Research Partnership
Emily Whitehead Foundation
Epilepsy Foundation of America
EveryLife Foundation for Rare Diseases
FamilieSCN2A Foundation
Foundation for Angelman Syndrome Therapeutics (FAST)
Foundation for the Accreditation of Cellular Therapy
Fralin Biomedical Research Institute at VTC
Friedreich's Ataxia Research Alliance (FARA)
GABA-A Alliance

The Global Foundation for Peroxisomal Disorders
Global Genes
GNB1 Advocacy Group, Inc.
GRIN2B Foundation
Hannah’s Hope Fund
HealthTree Foundation
Hermansky-Pudlak Syndrome Network
Hope for HIE
Immune Deficiency Foundation
International Foundation for CDKL5 Research
International Myeloma Foundation
International Society for Cell & Gene Therapy
International Society for Stem Cell Research
The Jansen's Foundation
Kindness Over Muscular Dystrophy
Lennox-Gastaut Syndrome (LGS) Foundation
LGMD Awareness Foundation
Lupus and Allied Diseases Association, Inc.
Marshall's Mountain
The Mathew Forbes Romer Foundation
MLD Foundation
Monoamine Oxidase Deficiency Foundation
MSUD Family Support Group
Muscular Dystrophy Association
National Alliance for Caregiving
National Bleeding Disorders Foundation
National MPS Society
National Tay-Sachs & Allied Diseases Association, Inc.
NMDP (National Marrow Donor Program)
Northern California Bleeding Disorders Foundation
Organic Acidemia Association
Orphan Therapeutics Accelerator, Inc.
Phelan-McDermid Syndrome Foundation
Powell Gene Therapy Center at University of Florida
The Progeria Research Foundation
PROJECT ALIVE
Project CASK
Rare Epilepsy Network (REN)
RARE Hope
Rare Trait Hope Fund
Research Institute at Nationwide Children's Hospital
Rett Syndrome Research Trust
SCID Foundation
Sickle Cell Prodigy
Society for Immunotherapy of Cancer
SSADH Association
Tough Genes
UMass Chan Medical School
United MSD Foundation
The University of Texas Medical Branch at Galveston
University of Virginia

Usher 1F Collaborative
Usher Syndrome Coalition
Usher Syndrome Society
v-ATPase Alliance
Wilson Disease Association
Wiskott-Aldrich Foundation