January 23, 2026

The Honorable John Thune
Majority Leader
United States Senate
511 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Chuck Schumer
Democratic Leader
United States Senate
322 Hart Senate Office Building
Washington, DC 20510

Dear Majority Leader Thune and Democratic Leader Schumer,

On behalf of the undersigned organizations, we urge you to advance S.932/H.R.1262, the Mikaela Naylon Give Kids a Chance Act. Among other things, the bill would reauthorize the Rare Pediatric Disease Priority Review Voucher (PRV) program. If passed, this bill will support the development of innovative cures for pediatric diseases.

The PRV program is a proven and vital incentive for the commercial development of therapies for children who currently have few or no therapeutic options. The program has been especially successful as a pathway to encourage private investment in early-stage cell and gene therapy (CGT) programs that address serious, unmet medical needs in children. Maintaining this incentive, which requires no additional expenditure of taxpayer dollars, would help ensure that promising discoveries have a realistic path to development and approval.

Rare pediatric diseases collectively affect millions of children and families in the United States, and CGTs represent one of the most significant advances to treat them. CGTs address the root causes of disease by modifying gene expression or repairing abnormal genes. 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 nearly two dozen approved CAR T-cell therapies and gene therapies, and a number of additional genetic-based medicines, that treat a wide range of diseases.[2] The development pipeline for CGTs includes over 4,300 therapies ranging from preclinical through pre-registration stages.[3]

The PRV program has proven to be a powerful catalyst for therapeutic development. Since its creation, 63 PRVs have been awarded across 47 rare pediatric diseases. Prior to the creation of the program, only 4 of these 47 diseases had any FDA-approved treatments.[4] Of the total 569 drug and biologic products granted rare pediatric disease designation between 2013 and 2022, 160 were gene therapies and 22 were antisense oligonucleotides (ASOs).[5] Since its inception, the PRV program has helped bring to market gene therapies for devastating conditions

[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] American Society of Gene & Cell Therapy + Citeline. (2025). *Gene, Cell, & RNA Therapy Landscape Report: Q3 2025 Quarterly Data Report.* https://www.asgct.org/news-publications/landscape-report
[4] National Organization for Rare Disorders. (2025). *Impact of the Rare Pediatric Disease Priority Review Voucher Program on Drug Development From 2012 – 2025 [white paper]*. https://rarediseases.org/wp-content/uploads/2025/12/NRD-2342-PRV-Policy-Report-November-2025.pdf
[5] Mease, C., Miller, K., Fermaglich, L., Best, J., Liu, G., Torjusen, E. (2024). *Analysis of the first ten years of FDA's rare pediatric disease priority review voucher program: designations, diseases, and drug development*. Orphanet J Rare Dis. 19(86) https://pmc.ncbi.nlm.nih.gov/articles/PMC10895788/

including Duchene muscular dystrophy (DMD),[6] sickle cell disease (SCD),[7] spinal muscular atrophy (SMA),[8] epidermolysis bullosa (EB),[9] and metachromatic leukodystrophy (MLD).[10]

The PRV program has support across a wide range of stakeholders, including FDA,[11] patient advocacy organizations, academia, and therapeutic developers. The challenges facing rare pediatric disease therapeutic development require coordinated engagement across stakeholders. The PRV program is a strong example of a policy that aligns incentives to advance therapies for children, while strengthening the broader research and development ecosystem.

The undersigned organizations represent the scientists, physicians, patient advocates, and other professionals developing and committed to the advancement of CGTs. **We again urge the Senate to advance the Mikaela Naylon Give Kids a Chance Act and reauthorize the Rare Pediatric Disease Priority Review Voucher (PRV) program.**

Sincerely,

The American Society of Gene & Cell Therapy
A Foundation Building Strength for Nemaline Myopathy
Abigail Wexner Research Institute at Nationwide Children's Hospital
ADCY5.org, Inc.
ADNP Kids Research Foundation
Advanced Therapies Program at the University of Minnesota
Alliance for Regenerative Medicine
Alport Syndrome Foundation
American Cancer Society Cancer Action Network
American College of Medical Genetics and Genomics
American Epilepsy Society
American Society of Human Genetics
American Society of Transplantation & Cellular Therapy
Angelman Syndrome Foundation
Arcanist Foundation
Association for Creatine Deficiencies
Association for the Advancement of Blood and Biotherapies (AABB)
ASXL Rare Research Endowment Foundation
Barth Syndrome Foundation
Baylor College of Medicine
Biocom
Blood Cancer United (formerly the Leukemia & Lymphoma Society)

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[6] US Food and Drug Administration. (2023). *Accelerated BLA Approval - STN: BL 125781/0.* https://www.fda.gov/media/169715/download
[7] US Food and Drug Administration. (2023). *BLA Approval - STN: BL 125787/0.* https://www.fda.gov/media/174618/download
[8] US Food and Drug Administration. (2019). *BLA Approval - STN: BL 125694/0.* https://www.fda.gov/media/126130/download
[9] US Food and Drug Administration. (2023). *BLA Approval - STN: BL 125774/0.* https://www.fda.gov/media/168356/download
[10] US Food and Drug Administration. (2024). *BLA Approval - STN: BL 125758/0.* https://www.fda.gov/media/177122/download
[11] CNBC: Squawk Box. (2025) Interview: *FDA Commissioner Dr. Marty Makary on streamlining approvals for biosimilar generic drugs.* Time stamp: 6:59. https://www.cnbc.com/video/2025/10/30/fda-commissioner-dr-marty-makary-on-streamlining-approvals-for-biosimilar-generic-drugs.html

Blu Genes Foundation
CACNA1A Foundation
Canavan Foundation
Cancer Support Community
Case Western Reserve University School of Medicine
CDKL5 in Color
CGD Association of America
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 Tumor Foundation
Child's Cure Genetic Research Foundation
Cincinnati Children's Hospital
City of Hope
CMT Research Foundation
Coalition to Cure Calpain 3
Coalition to Cure CHD2
CSNK2A1 Foundation
CSNK2B Foundation
CTNNB1 Connect and Cure
Cure CLCN6
Cure CMD
Cure GM1 Foundation
Cure LGMD2i Foundation
Cure Rare Disease, Inc.
Cure Sanfilippo Foundation
Cure SMA
Cure SPG4 Foundation
CURE SYNGAP1
Cure Tay-Sachs Foundation
CureCMT4J
CureGRIN Foundation
CureSHANK
CYFIP2 Network
Cystic Fibrosis Foundation
Dana-Farber Cancer Institute
Danon Disease Foundation
Dion Foundation for Children with Rare Diseases, Inc.
DLG4 SHINE Foundation
Dravet Syndrome Foundation
Dup15q Alliance
Duplication Cares

DYNC1H1 Association
EB Research Partnership
Emily Whitehead Foundation
Emily's Entourage
Epilepsy Foundation of America
EveryLife Foundation for Rare Diseases
FAM177A1 Research Fund
FamilieSCN2A Foundation
Finding Hope for FRRS1L
Foundation for Angelman Syndrome Therapeutics
Foundation for the Accreditation of Cellular Therapy
FOXG1 Research Foundation
Friends of Cancer Research
GABA-A Alliance
Galactosemia Foundation
Gene and Cell Therapy Institute at Mass General Brigham
The Global Foundation for Peroxisomal Disorders
Global Genes
GNB1 Advocacy Group Inc
GNB1 Advocacy Group, Inc.
The Gould Syndrome Foundation
GRIN2B Foundation
GRIN2B Foundation
Hannah’s Hope Fund for Giant Axonal Neuropathy
Haystack Project
HCU Network America
Helen Diller Family Comprehensive Cancer Center, UCSF
Hermansky-Pudlak Syndrome Network
HNRNP Family Foundation
Hope for HIE
Hope in Focus
Horae Gene Therapy Center at UMass Chan
Hyper IgM Foundation
Immune Deficiency Foundation
Innovative Genomics Institute
Institute for Gene Therapies
International Fibrodysplasia Ossificans Progressiva Association
International Foundation for CDKL5 Research
International Pain Foundation
International Society for Stem Cell Research
ISCT, International Society for Cell & Gene Therapy
Jeffrey Modell Foundation
KCNQ2 Cure Alliance
Kindness Over Muscular Dystrophy

Krishnan Family Foundation
The LCC Foundation
Lennox-Gastaut Syndrome (LGS) Foundation
LGMD Awareness Foundation
LGMD2D Foundation
Li Weibo Institute for Rare Diseases Research at UMass Chan
Lupus and Allied Diseases Association, Inc.
Malan Syndrome Foundation
Marshall's Mountain
Mississippi Metabolics Foundation
MLD Foundation
Monoamine Oxidase Deficiency Foundation
MSUD Family Support Group
Muscular Dystrophy Association
National Bleeding Disorders Foundation
National MPS Society
National Organization for Rare Disorders (NORD)
National Tay-Sachs & Allied Diseases Association, Inc.
NBIA Disorders Association
NMDP (National Marrow Donor Program)
NPHP1 Family Foundation
Oregon Health & Science University
Organic Acidemia Association
Orphan Therapeutics Accelerator
Parent Project Muscular Dystrophy
Pathways for Rare and Orphan Solutions
PBD Project
Phelan-McDermid Syndrome Foundation
Plasminogen Deficiency Foundation
Primary Immune Deficiency Treatment Consortium
The Progeria Research Foundation
Project Alive
Project CASK
Raiden Science Foundation
RARE Hope
Rare Trait Hope Fund
RDH12 Fund for Sight
Research!America
Rett Syndrome Research Trust
The Sanfilippo Research Foundation
Sarcoma Foundation of America
Schinzel-Giedion Syndrome Foundation
SCID Foundation
Society for Immunotherapy of Cancer (SITC)

The Speak Foundation
SSADH Association
The Sturge-Weber Foundation
STXBP1 Foundation
Sudden Arrhythmia Death Syndrome Foundation
TESS Research Foundation for SLC13A5 Epilepsy
Texas Healthcare and Bioscience Institute
Tough Genes
TSC Alliance
UMass Chan Medical School
UMass Chan Translational Institute for Molecular Therapies
United Leukodystrophy Foundation
United MSD Foundation
University of California, San Francisco
University of Pennsylvania Perelman School of Medicine
University of Rochester
University of Utah Health
University of Wisconsin School of Medicine and Public Health
Usher 1F Collaborative
v-ATPase Alliance
Virginia Tech
Weill Cornell Medicine
Wilson Disease Association
Wiskott-Aldrich Foundation
Wylder Nation Foundation
YWHAG Research Foundation
ZMYND11 Treatment Foundation
ZTTK SON-Shine Foundation

CC:

The Honorable Bill Cassidy
Chair, Senate HELP Committee

The Honorable Bernie Sanders
Ranking Member, Senate HELP Committee