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GABA













CUREGM1





Cure KCNH1







































































Family Connection, Inc

Global Genes\*

Alter in the Bitters

Alter







STXBP1























































## September 10, 2025

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

The Honorable Chuck Schumer Minority Leader United States Senate 322 Hart Senate Office Building Washington, DC 20510 The Honorable Mike Johnson
Speaker of the House
United States House of Representatives
568 Cannon House Office Building
Washington, DC 20515

The Honorable Hakeem Jeffries Minority Leader United States House of Representatives 2267 Rayburn House Office Building Washington, DC 20515

Dear Majority Leader Thune, Minority Leader Schumer, Speaker Johnson, and Minority Leader Jeffries, Chair Guthrie, and Ranking Member Pallone,

On behalf of the 30 million Americans living with a rare disease, the undersigned 190 organizations write to express strong support for the Give Kids a Chance Act of 2025 (H.R. 1262/S.932) and urge its swift passage by Congress. This urgent legislation would reauthorize the highly effective Rare Pediatric Disease Priority Review Voucher (PRV) program for five years. As many as half of the individuals living with a rare disease are children, and this program offers a crucial incentive to develop therapies for this particularly challenging-to-study patient population living with devastating and often life-threatening rare conditions.

Nearly 70% of rare diseases start in childhood<sup>1</sup> and most have no approved treatments. The Rare Pediatric Disease PRV program, created by Congress in 2012, has been a vital catalyst for developing therapies for these vulnerable populations. Thanks to this program, treatments have reached children suffering from nearly 40 rare diseases, many of which previously had no FDA-approved options and led to severe disability or death before adulthood.<sup>2</sup>

The program's impact is accelerating – more than half of all Rare Pediatric Disease designations occurred in just the past four years,<sup>3</sup> demonstrating its growing importance in addressing urgent unmet medical needs. Yet despite this progress, over 95% of rare diseases still lack an FDA-approved therapy.

<sup>&</sup>lt;sup>1</sup> Nguengang Wakap S, Lambert DM, Olry A, Rodwell C, Gueydan C, Lanneau V, et al. Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. Eur J Hum Genet. 2020;28(2):165–73. <a href="https://www.nature.com/articles/s41431-019-0508-0">https://www.nature.com/articles/s41431-019-0508-0</a>

<sup>&</sup>lt;sup>2</sup> See: https://rarediseases.org/wp-content/uploads/2024/05/NORD PRV-white-paper FINAL.pdf

<sup>&</sup>lt;sup>3</sup> Mease, C., Miller, K. L., Fermaglich, L. J., 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. 2024;19(1):131. <a href="https://pubmed.ncbi.nlm.nih.gov/38403586/">https://pubmed.ncbi.nlm.nih.gov/38403586/</a>

The Rare Pediatric Disease PRV program's authorization expired on December 20, 2024. Without reauthorization, newly designated investigational therapies are ineligible for PRVs. Those promising therapies that received Rare Pediatric Designation before the December 20<sup>th</sup> expiration, but do not receive FDA-approval before September 30, 2026 are also ineligible for a PRV. Allowing this program to lapse not only eliminates a proven pathway for future innovation and hope, but it also creates uncertainty for scientific work currently underway that will not meet the September 30, 2026 deadline.

Importantly, the PRV program is a market-based incentive that comes at no cost to taxpayers. The data shows that the Rare Pediatric Disease PRV program is working to address unmet needs and will continue to do so for years to come when reauthorized.

We urge Congress to act quickly and pass the Give Kids a Chance Act of 2025 (H.R. 1262/S. 932) to restore this life-saving program and ensure continued progress in rare pediatric drug development. Our communities are counting on you. We look forward to working with you on this important issue.

For any questions or concerns, please contact Allison Herrity at the National Organization for Rare Disorders, at <a href="mailto:aherrity@rarediseases.org">aherrity@rarediseases.org</a> and Jamie Sullivan at the EveryLife Foundation for Rare Diseases, at <a href="mailto:jullivan@everylifefoundation.org">jullivan@everylifefoundation.org</a>.

Thank you for your leadership and commitment to improving the lives of children with rare diseases.

## Sincerely,

EveryLife Foundation for Rare Diseases

National Organization for Rare Disorders (NORD)

Adrenal Insufficiency United

Aimed Alliance
Akari Foundation

Alliance for Patient Access

Alliance to Cure Cavernous Malformation

The Alpha-1 Foundation

AMDA

American Liver Foundation
Angelman Syndrome Foundation
Aplastic Anemia and MDS International

Foundation

Association for Creatine Deficiencies

ASXL Rare Research Endowment Foundation

Avery's Hope

**Barth Syndrome Foundation** 

**BDSRA** Foundation

**BPAN WARRIORS** 

Bubba's Light, Inc.

CA Action Link for Rare Diseases (CAL RARE)

CACNA1A Foundation
Canavan Foundation

CancerCare

**CDH** International

Center for Innovation & Value Research

Child Neurology Foundation CMT Research Foundation Coalition to Cure CHD2

Congenital Hyperinsulinism International

Cooley's Anemia Foundation

CSNK2A1 Foundation

Cure CMD
CURE Epilepsy
CURE GABA-A

Cure GM1 Foundation

Cure KCNH1 Foundation Hermansky-Pudlak Syndrome Network Inc.

Cure LGMD2i Foundation Hnrnp family foundation

**Cure Lowe Foundation** Hope for HIE Cure SMA Hope in Focus

**CureARS** Hydrocephalus Association **CURED Nfp** HypoPARAthyroidism Association CureSHANK Immune Deficiency Foundation

Cyclic Vomiting Syndrome Association **INADcure Foundation** Cystic Fibrosis Research Institute Indo US Organization for Rare Diseases International Fibrodysplasia Ossificans Dana's Angels Research Trust

Progressiva (FOP) Association Developmental and Epileptic Encephalopathies -

**DEE-P Connections** International Foundation for CDKL5 Research

Dion Foundation for Children with Rare Diseases International Rett Syndrome Foundation

**Dravet Syndrome Foundation** International SCN8A Alliance **IWMF** EB Research Partnership

Eosinophilic & Rare Disease Cooperative **Jack Bear Foundation** 

**Epilepsy Advocacy Network** Joanna Sophia Foundation **Epilepsy Foundation of America** Juju and Friends CLN2 Warrior Foundation

**Epilepsy Foundation Texas** Kabuki Syndrome Foundation

**KARES Foundation** 

**Epilepsy Support Network of Orange County** KCNQ2 Cure Alliance **Epilepsy Alliance America** 

**Fabry Support & Information Group KCNT1** Epilepsy Foundation

FAM177A1 Research Fund KIF1A.ORG

Familial Dysautonomia Foundation Koolen-de Vries Syndrome Foundation

**Family Heart Foundation KPTN Alliance** 

FD/MAS Alliance KrabbeConnect

Fighting H.A.R.D. Foundation Krishnan Family Foundation

Firefly Fund Lennox-Gastaut Syndrome (LGS) Foundation flok Health LGMD Awareness Foundation, Inc

Fondazione Telethon LGMD2D Foundation

Foundation for Angelman Syndrome Little Hercules Foundation **Therapeutics** Littlest Tumor Foundation

Foundation for Prader-Willi Research Lymphoma Research Foundation

FRAXA Research Foundation Mackenzies Mission Friedreich's Ataxia Research Alliance (FARA) Melanie J Foundation

GABA-A Alliance Mississippi Metabolics Foundation

**GACI Global** MLD Foundation

Galactosemia Foundation MPS Research & Treatment Center Gaucher Community Alliance **MSUD Family Support Group** 

Gene Giraffe Project MTM-CNM Family Connection Global Genes Muscular Dystrophy Association

GNB1 Advocacy Group, Inc My Kool Brother

**Haystack Project** Myasthenia Gravis Association **HCU Network America** Myositis Support and Understanding Hemophilia Foundation of Southern California National Alliance for Caregiving

National Ataxia Foundation
National Fragile X Foundation

National Health Council National Kidney Foundation

**National MPS Society** 

National Niemann-Pick Disease Foundation

National PKU Alliance

National Tay-Sachs & Allied Diseases Association,

Inc.

**NBIA Disorders Association** 

Necrotizing Enterocolitis (NEC) Society Neev Kolte & Brave Ronil Foundation

NephCure

Noah's Hope - Hope4Bridget
NTM Info & Research, Inc.
Organic Acidemia Association
Parent Project Muscular Dystrophy
Pathways for Rare and Orphan Studies

Patient Empowerment Network

Phelan-McDermid Syndrome Foundation

Pompe Alliance Project Alive

PSC Partners Seeking a Cure

PWSA | USA - Prader-Willi Syndrome Association

Rare Epilepsy Network Rare Trait Hope Fund

RareRising

Raymond A. Wood Foundation Rett Syndrome Research Trust Sarcoidosis of Long Island SATB2 Gene Foundation

SCAD Alliance (spontaneous coronary artery

dissection)

Sickle Cell Association of Kentuckiana

South Carolina Advocates for Epilepsy (S.A.F.E.)

Spina Bifida Association

Stronger Than Sarcoidosis and Sarcoidosis of

Long Island

STXBP1 Foundation

Superior Mesenteric Artery Syndrome Research

Awareness and Support

SynGAP Research Fund dba CURE SYNGAP1
The Bonnell Foundation: Living with cystic

fibrosis

The Children's Medical Research Foundation,

Inc.

The Cute Syndrome Foundation

The Global Foundation for Peroxisomal Disorders

The GLUT1 Deficiency Foundation

The Guthy-Jackson Charitable Foundation

The Hope Project For kids The Jansens's Foundation The LAM Foundation The LCC Foundation

The Louisa Adelynn Johnson Fund for Complex

Disease

The Mended Hearts, Inc.

The National Adrenal Diseases Foundation

The Rory Belle Foundation
The Sturge-Weber Foundation

Tough Genes TSC Alliance

United Mitochondrial Disease Foundation

United MSD Foundation

**United Porphyrias Association** 

US Hereditary Angioedema Association

**USTMA Consortium and Alliance** 

Vasculitis Foundation
Wake Up Narcolepsy, Inc.
WI Rare Disease Alliance
Wilson Disease Association
Wiskott Aldrich Foundation
Wylder Nation Foundation

Yaya Foundation for 4H Leukodystrophy

**ZTTK Son-Shine Foundation**