
NORD
National Organization for Rare Disorders
EVERYLIFE FOUNDATION FOR RARE DISEASES
DYSAUTONOMIA FOUNDATION, INC
NTSAD
National Tay-Sachs & Allied Diseases Association
UNITED PORPHYRIAS ASSOCIATION
HCU NETWORK AMERICA
FOUNDATION FOR PRADER-WILLI RESEARCH
CURE EPILEPSY
LGS FOUNDATION
LENNOX-GASTAUT SYNDROME
Galactosemia Foundation
CURELGMD2i
Sarcoidosis
Myositis Support & Understanding
KrabbeConnect
epilepsy advocacy network
LITTLE HERCULES FOUNDATION
The Hope Project for Kids
CANAVAN FOUNDATION
LGMD AWARENESS FOUNDATION
iWMF
International Waldenstrom's Macroglobulinemia Foundation
IFCR
International Foundation for CDKL5 Research
FRAXA Research Foundation
SCNA ALLIANCE
BDSRA foundation
LCC Foundation
KPTN Alliance
HypoPARAthyroidism Association, Inc.
NTM Info & Research, Inc.
DEE-P CONNECTIONS
CALRARE
Hydrocephalus Association
VASCULITIS FOUNDATION
The Jansen's Foundation
Fighting H.A.R.D. Foundation
Immune Deficiency Foundation
CureAIDS
MELLIE J foundation
HAEA
US HEREDITARY ANGIOEDEMA ASSOCIATION
National MPS Society
ALPHA-1 FOUNDATION
DION FOUNDATION
nnpdf
NATIONAL NIEMANN-PICK DISEASE FOUNDATION, INC.
PSC PARTNERS SEEKING A CURE
ADRENAL INSUFFICIENCY UNITED
amda
cure SMA
AfPA
Alliance for Patient Access
MUENZER MPS
GACI Global
Patient Empowerment Network
Wiskott-Aldrich foundation
Firefly FUND
NATIONAL ALLIANCE FOR CAREGIVING
Lymphoma Research Foundation
ACD
KARES FOUNDATION
The Roxy Belle FOUNDATION
epilepsy ALLIANCE AMERICA
GNB1
pmsf
Phelan-McDermid Syndrome FOUNDATION
KCNT1 EPILEPSY
HOPE IS ON THE HORIZON
South Carolina Advocates for Epilepsy
EPILEPSY FOUNDATION
ALLIANCE TO CURE CAVERNOUS MALFORMATION
Epilepsy Support Network
BEANWARRIORS.ORG
you are not alone
AMERICAN LIVER FOUNDATION
Cure Cystinosis FOUNDATION
The Cute Syndrome FOUNDATION
NATIONAL KIDNEY FOUNDATION
flok
UNITED MSD FOUNDATION
MULTIPLE SULFATASE DEFICIENCY
MIB
My Kool Brother
LGMD2D Foundation
CMT Research Foundation
ifopa
MLD foundation
HAYSTACK PROJECT
Parent Project Muscular Dystrophy
PROJECT ALIVE
THE LAM FOUNDATION
TOUGH GENES
CDH International
NEC SOCIETY
EB RESEARCH PARTNERSHIP
KDVS
NBIA DISORDERS ASSOCIATION
WD wyder nation foundation
NR Nora Kelly & Bruce Rand Foundation
sat b2 gene foundation
TSC Alliance
GABA ALLIANCE
FAMILY HEART FOUNDATION
INADCURE FOUNDATION
CUREGM1 FOUNDATION
Cure KCNH1 Foundation
angelman SYNDROME FOUNDATION
Hemophilia Foundation of Southern California
DART
DANA'S ANGELS RESEARCH TRUST
UNITED MITOCHONDRIAL DISEASE FOUNDATION
MSUD
CVSA
THE BONNELL FOUNDATION
Barth Syndrome Foundation
WI Rare
KRISHAN FAMILY FOUNDATION
indousrare
Prader-Willi SYNDROME ASSOCIATION | USA
SAVING AND TRANSFORMING LIVES
AH CaringForRare
NADF National Adrenal Diseases Foundation
FARA Friedreich's Ataxia Research Alliance
AIMED ALLIANCE
CURED www.CuredFoundation.org
fast FOUNDATION FOR ANGELMAN SYNDROME THERAPEUTICS
PROS FOUNDATION
NATIONAL HEALTH COUNCIL
Dravet Syndrome Foundation
cure CMD
a cure is among us
Pompe Alliance
ZTTK
NephCure
FONDAZIONE Telethon
MISSISSIPPI MMF METABOLICS FOUNDATION
GLUT1 Deficiency Foundation
SCAD ALLIANCE
The Mended Hearts, Inc.
CURE LOWE
RETT SYNDROME RESEARCH TRUST
FD/MAS ALLIANCE
Organic Acidemia Association
WILSON DISEASE ASSOCIATION
SynGAP Research Fund
CCC COALITION TO CURE CHD2
NAF National Ataxia Foundation
CHI Congenital Hyperinsulinism International
MGA
GFPD GLOBAL FOUNDATION FOR PEROXISOMAL DISORDERS
HOPE for HIE
MDA Muscular Dystrophy Association
Raymond A. Wood Foundation
NPKUA national PKU alliance
International Rett Syndrome Foundation
KCNA1 FOUNDATION
YAYA FOUNDATION
CACNA1A FOUNDATION
nfxf NATIONAL FRAGILE X FOUNDATION
FSIG
Jack Bear FOUNDATION
Child Neurology FOUNDATION
Bubba's LIGHT
SPINA BIFIDA ASSOCIATION
Wake Up Narcolepsy
MACKENZIE'S MISSION
Family Connection, Inc
Global Genes
Cooley's Anemia FOUNDATION
ARRE ASXL RARE RESEARCH ENDOWMENT
cureSHANK
STXBP1 foundation
Center for Innovation & Value Research
CSNK2A1 FOUNDATION
SICKLE CELL ASSOCIATION OF KENTUCKIANA
SUPERIOR MESENTERIC ARTERY SYNDROME
CURE GABA A
Rare Rising
HOPE in FOCUS
Seeing a cure for blindness
noah's hope hope4bridget
GUTHY JACKSON Charitable Foundation
KCNQ2 Cure
EPILEPSY FOUNDATION TEXAS - HOUSTON/ DALLAS-FORT WORTH/ WEST TEXAS
The Louisa Adelynn Johnson Fund for Complex Disease
CFRI Cystic Fibrosis Research Institute
AA-MDS
Kabuki SYNDROME FOUNDATION
HPS
GCA GAUCHER COMMUNITY ALLIANCE
USTMA
littlest Tumor Foundation
CANCERcare
THE CHILDREN'S MEDICAL RESEARCH FOUNDATION, INC.
A Cure for Kirby
FAM177A1 RESEARCH FUND
REN Rare Epilepsy Network
EOSINOPHILIC & RARE DISEASE COOPERATIVE

September 10, 2025

The Honorable John Thune
Majority Leader
United States Senate
511 Dirksen 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 Chuck Schumer
Minority Leader
United States Senate
322 Hart Senate Office Building
Washington, DC 20510

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[1] 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.[2]

The program's impact is accelerating – more than half of all Rare Pediatric Disease designations occurred in just the past four years,[3] 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.

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[1] 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. https://www.nature.com/articles/s41431-019-0508-0

[2] See: https://rarediseases.org/wp-content/uploads/2024/05/NORD_PRV-white-paper_FINAL.pdf

[3] 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. https://pubmed.ncbi.nlm.nih.gov/38403586/

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 20th 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 aherrity@rarediseases.org and Jamie Sullivan at the EveryLife Foundation for Rare Diseases, at jsullivan@everylifefoundation.org.

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
Cure LGMD2i Foundation
Cure Lowe Foundation
Cure SMA
CureARS
CURED Nfp
CureSHANK
Cyclic Vomiting Syndrome Association
Cystic Fibrosis Research Institute
Dana's Angels Research Trust
Developmental and Epileptic Encephalopathies - DEE-P Connections
Dion Foundation for Children with Rare Diseases
Dravet Syndrome Foundation
EB Research Partnership
Eosinophilic & Rare Disease Cooperative
Epilepsy Advocacy Network
Epilepsy Foundation of America
Epilepsy Foundation Texas
Epilepsy Support Network of Orange County
Epilepsy Alliance America
Fabry Support & Information Group
FAM177A1 Research Fund
Familial Dysautonomia Foundation
Family Heart Foundation
FD/MAS Alliance
Fighting H.A.R.D. Foundation
Firefly Fund
flok Health
Fondazione Telethon
Foundation for Angelman Syndrome Therapeutics
Foundation for Prader-Willi Research
FRAXA Research Foundation
Friedreich's Ataxia Research Alliance (FARA)
GABA-A Alliance
GACI Global
Galactosemia Foundation
Gaucher Community Alliance
Gene Giraffe Project
Global Genes
GNB1 Advocacy Group, Inc
Haystack Project
HCU Network America
Hemophilia Foundation of Southern California
Hermansky-Pudlak Syndrome Network Inc.
Hnrnp family foundation
Hope for HIE
Hope in Focus
Hydrocephalus Association
HypoPARAthyroidism Association
Immune Deficiency Foundation
INADcure Foundation
Indo US Organization for Rare Diseases
International Fibrodysplasia Ossificans Progressiva (FOP) Association
International Foundation for CDKL5 Research
International Rett Syndrome Foundation
International SCN8A Alliance
IWMF
Jack Bear Foundation
Joanna Sophia Foundation
Juju and Friends CLN2 Warrior Foundation
Kabuki Syndrome Foundation
KARES Foundation
KCNQ2 Cure Alliance
KCNT1 Epilepsy Foundation
KIF1A.ORG
Koolen-de Vries Syndrome Foundation
KPTN Alliance
KrabbeConnect
Krishnan Family Foundation
Lennox-Gastaut Syndrome (LGS) Foundation
LGMD Awareness Foundation, Inc
LGMD2D Foundation
Little Hercules Foundation
Littlest Tumor Foundation
Lymphoma Research Foundation
Mackenzies Mission
Melanie J Foundation
Mississippi Metabolics Foundation
MLD Foundation
MPS Research & Treatment Center
MSUD Family Support Group
MTM-CNM Family Connection
Muscular Dystrophy Association
My Kool Brother
Myasthenia Gravis Association
Myositis Support and Understanding
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