
NORD
National Organization for Rare Disorders
EveryLife Foundation
cureSHANK
Cure KCNH1 Foundation
Sleep Consortium
NPKUA
PKU alliance
nac
National Alliance for Caregiving
CMTA
Charcot-Marie-Tooth Association
The Little Leg Big Heart Foundation
EB Research Partnership
Hypothalamic Hamartoma Hope
Giving Hope. Getting Answers.
MED13L Foundation
Fighting H.A.R.D. Foundation
Helping kids who Have A Rare Disease or chronic illness
Canavan Foundation
BDSRA foundation
Rett Syndrome Research Trust
CCC Coalition to Cure CHD2
Endosalpingiosis Foundation Inc
Pompe Alliance
E.W.E Foundation
Batten Disease Foundation
KrabbeConnect
Connecting Patients & Research
angelman Syndrome Foundation
American Kidney Fund
Fighting on all fronts
Cure GABA A Variants
fast
Foundation for Angelman Syndrome Therapeutics
ifopa
LGMD2D Foundation
Mellie J Foundation
NADF
National Adrenal Diseases Foundation
GLI Global Liver Institute
CDH International
Wake Up Narcolepsy
CACNA1A Foundation
HOPE in FOCUS
Seeing a cure for blindness
Epilepsy Foundation
Superior Mesenteric Artery Syndrome
Research Awareness and Support
Nephcure
Sanfilippo Children's Foundation
Sanfilippo Sud
CureARS
Tislin's Wish
Vasculitis Foundation
Early Diagnosis · Better Treatment · Improving Lives
Dup15q Alliance
United Porphyrias Association
Immune Deficiency Foundation
cure SMA
DION Foundation
For children with rare diseases
WI Rare
International Rett Syndrome Foundation
Foundation to Fight H-ABC
flok
TSC Alliance
FSIG
KCNQ2 Cure
unity. hope. strength. love.
Rare Disease Coalition
MSUD
Family Support Group
The Mended Hearts, Inc.
PMD Foundation
Pelizaeus-Merzbacher Disease
FRAXA Research Foundation
CHI
Congenital Hyperinsulinism International
When too little sugar hurts, we help.
cure CMD
a cure is among us
IFCR
Adrenal Insufficiency United
MDA
Muscular Dystrophy Association
Glut1 Deficiency Foundation
National MPS Society
Support for Families. Research for a Cure.
ALPHA-1 Foundation
UOAA
ostomy.org
Cooley's Anemia Foundation
Leading the Fight Against Thalassemia
Galactosemia Foundation
Linked for Life
CFRI Cystic Fibrosis Research Institute
Research · Education · Advocacy · Support
Dysautonomia Foundation, Inc
AA·MDS
International Foundation
Oxalosis & Hyperoxaluria Foundation
Stepping Stones to a Cure
Hydrocephalus Association
LGMD
Awareness Foundation
GBS|CIDP
Foundation International
FD/MAS Alliance
DDX3X Foundation
Kabuki Syndrome Foundation
KAT6 Foundation
Parent Project Muscular Dystrophy
Join the Fight. End Duchenne.
CSNK2A1 Foundation
CURED
Campaign Urging Research for Eosinophilic Disease
www.CuredFoundation.org
NTM
Nontuberculous Mycobacteria
NTM Info & Research, Inc.
ALGSA
MLD foundation
We C.A.R.E.
NBIA Disorders Association
From Discovery to cure
Navigating the Journey
Building Community
Investing in Research
Accelerating Cures
LGS Foundation
Lennox-Gastaut Syndrome
Shwachman-Diamond Syndrome Alliance
HCU Network America
Huntington's Disease Society of America
GDEN C.A.R.E.S.
FARA
Friedreich's Ataxia Research Alliance
NAF
National Ataxia Foundation
GABA
COMBINED
CURELGMD2i
Limb Girdle Muscular Dystrophy
SynGAP Research Fund
HPS
Hermansky-Pudlak Syndrome Network, Inc.
Organic Acidemia Association
Child Neurology Foundation
Creating a Community of Support
Jack McGovern
Coats' Disease Foundation
CNNB1
Connect and Cure
NTSAD
National Tay-Sachs & Allied Diseases Association
United Mitochondrial Disease Foundation
BrainWarriors.org
You are not alone
IWMF
International Waldenstrom Macroglobulinemia Foundation
noah's hope
hope4bridget
GCA
Gaucher Community Alliance
FAM177A1 Research Fund
Spina Bifida Association
RYR-I Foundation
www.ryr1.org
KDVS
Koolen-de Vries Syndrome Foundation
Alport Syndrome Foundation
Hope | Action | Support
AH
#CaringForRare
Avery's Hope
sat b2
gene foundation
UNITEDMSD Foundation
Multiple Sulfatase Deficiency
INADCURE Foundation
Fighting Infantile Neuroaxonal Dystrophy
Global Genes
Allies in Rare Disease
Fondazione Telethon
CUREGM1 Foundation
Eosinophilic & Rare Disease Cooperative
Cure Mito Foundation
The Bonnell Foundation: Living with Cystic Fibrosis
Live · Breathe · Inspire
Roadmap to CF™ | thebonnellfoundation.org
Project Alive
ARRE
ASXL Rare Research Endowment
Acromegaly Community
AIMED Alliance
APBD
Adult Polyglucosan Body Disease Research Foundation
Autism Science Foundation
BARE
Bubba's Light
Krishnan Family HV Foundation
CalRare
California Action Link for Rare Diseases
Center for Innovation & Value Research
CONQUER MG
Conquer Myasthenia Gravis
Cure Lowe
CURE
cureSHANK
CVSA
The Jansen's Foundation
DART
Dana's Angels Research Trust
debra
because the cost of doing nothing is too great
Dravet Syndrome Foundation
www.dravetfoundation.org
Dreamsickle Kids Foundation
Family Heart Foundation
Foundation for Prader-Willi Research
Juju and Friends CLN2 Warrior Foundation
UCI HD-CARE
Huntington's Disease Community Advocacy, Research & Education
Little Hercules Foundation
Lung Transplant Foundation
Mississippi MMF Metabolics Foundation
Li-Fraumeni Syndrome Association
LFSA
Family Connection, Inc
Resources • Research • Relationships
mg
Myasthenia Gravis Foundation of America
Myositis Support & Understanding
NE1 Collaborative
NAPPA
National Alliance for PANS/PANDAS Action
nfxf
National Fragile X Foundation
Patient Powered National Health Council
Mission:Cure
National Kidney Foundation
Never Quit & Brave Road Foundation
Partnership to Fight Chronic Disease
PROS Foundation
Petronille Healthy Society
PTEN
Hamartoma Tumor Syndrome Foundation
RNE
Rare New England
Rare Rising
Raymond A. Wood Foundation
SCAD Alliance
Sickle Cell Association of Kentucky
sisters' hope Foundation
Taylor's tale
Team Telomere
U.R. Our Hope
Tough Genes
MED13L Foundation
The Louisa Adelynn Johnson Fund
for Complex Disease
LAM Foundation
A Breath of Hope
GFPD
Bluefield Project
Curing FTD
AFTD
Hemophilia Foundation of Southern California
For all bleeding disorders
ZTTK
SON-SHINE Foundation
Wilson Disease Association
Undiagnosed Diseases Network Foundation
Advocates for Medically Fragile Kids NC

December 4, 2024

The Honorable Chuck Schumer
Majority Leader
United States Senate
322 Hart Senate Office Building
Washington, D.C. 20510

The Honorable Mike Johnson
Speaker of the House
United States House of Representatives
568 Cannon House Office Building
Washington, D.C. 20515

The Honorable Mitch McConnell
Minority Leader
United States Senate
317 Russell Senate Office Building
Washington, D.C. 20510

The Honorable Hakeem Jeffries
Minority Leader
United States House of Representatives
2433 Rayburn House Office Building
Washington, D.C. 20515

Dear Majority Leader Schumer, and Minority Leader McConnell, Speaker Johnson, Minority Leader Jeffries,

As you work to finalize legislation for consideration before the end of the 118th Congress, the undersigned 213 organizations urge you to pass or include within any larger bill the provisions of the Creating Hope Reauthorization Act (H.R. 7384/S. 4583), which would reauthorize the highly effective Rare Pediatric Disease Priority Review Voucher (PRV) program for at least five years. Reauthorizing the Rare Pediatric Disease PRV program has broad bipartisan support, including approval by the full House of Representatives in September as part of the amended Give Kids a Chance Act (H.R. 3433). The current authorization is set to expire on December 20th, and a timely, clean and long-term reauthorization is critical to maintaining this important incentive which has effectively spurred drug development to help children living with rare diseases.

Since its creation by Congress in 2012, the Rare Pediatric Disease PRV program has helped bring to market therapies for children affected by 39 rare diseases.[1] Without treatment, many of these diseases lead to death or debilitating illness before the children reach adulthood, and only three of these rare diseases had a safe and effective FDA-approved therapy on the market before the program began.[2] Additionally, more than half of all Rare Pediatric Disease PRV designations occurred in the last four years,[3] showing the program is fostering robust drug development where significant unmet therapeutic needs currently exist.

Developing drugs for rare pediatric diseases often presents unique challenges, including small patient populations and difficulties conducting pediatric clinical trials. The incentive established by the Rare Pediatric Disease PRV program is simple and according to the Congressional Budget Office (CBO) score[4]

[1] See: https://rarediseases.org/wp-content/uploads/2024/05/NORD_PRV-white-paper_FINAL.pdf
[2] Ibid.
[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 Journal of Rare Diseases. https://link.springer.com/epdf/10.1186/s13023-024-03097
x03097x?sharing_token=tVsdcxtCuGoLKGG18G02G_BpE1tBhCbnbw3BuzI2ROyCDnBKl_41BmSn3a_5qrzjgrL
XsufvRX0wtQEn
ALK9Za3v_5zjNTa3quYxLJ0LC4dnFV94TbHqovQ6Vq5sRWu7_u2v1C7h16jaeLChSswkyx4eSqy_KycTNie1
qfGSM
[4] https://www.cbo.gov/system/files/2024-09/suspensions_week_of_9_23_2024_1.pdf

would not have any effect on direct federal spending: if a product manufacturer develops an FDA-approved therapy to treat a rare pediatric disease, the company earns a transferable priority review voucher – and the right to a more expeditious FDA review timeline – that can be used for a subsequent product application or can be sold and transferred to another company. If the Rare Pediatric Disease PRV program is not reauthorized by Congress, a key incentive that has effectively helped bring treatments and cures to kids and their families will end. We cannot afford to let this happen.

As noted, a five-year extension of the Rare Pediatric Disease PRV program passed unanimously by the House of Representatives and has garnered significant bipartisan support. It is also widely supported by the rare disease patient community, with nearly 200 patient organizations signing on to a letter of support for the bill this summer. We urge that you build upon the work done to date and pass a timely, clean and long-term reauthorization to ensure promising science can be translated into treatments and hope for children and families affected by rare diseases.

Thank you for considering this request and please don't hesitate to reach out to Jamie Sullivan at the EveryLife Foundation for Rare Diseases, at jsullivan@everylifefoundation.org and Hayley Mason, Policy Analyst with the National Organization for Rare Disorders, at HMason@rarediseases.org with any questions.

Sincerely,

EveryLife Foundation for Rare Diseases
National Organization for Rare Disorders
Acromegaly Community Inc.
Adrenal Insufficiency United
Advocates for Medically Fragile Kids NC
Aimed Alliance
Aislinn's Wish Foundation
Alagille Syndrome Alliance
Alpha-1 Foundation
Alport Syndrome Foundation
AMDA
American Kidney Fund
Angelman Syndrome Foundation
APBDRFoundation
Aplastic Anemia and MDS International Foundation
ASXL Rare Research Endowment
Autism Science Foundation
Avery's Hope
BARE Inc
Barth Syndrome Foundation
BDSRA Foundation
BPAN WARRIORS
Bubba's Light
CA Action Link for Rare Diseases (Cal Rare)
CACNA1A Foundation
Canavan Foundation
CDH International
Center for Innovation & Value Research
Charcot Marie Tooth Research Foundation
Child Neurology Foundation
Chondrosarcoma CS Foundation, Inc.
Coalition to Cure Calpain 3
Coalition to Cure CHD2
COMBINEDBrain
Congenital Hyperinsulinism International
Conquer MG
Cooley's Anemia Foundation
CSNK2A1 FOUNDATION
CTNNB1 Connect and Cure
Cure CMD
CURE GABA-A
Cure GM1 Foundation
Cure KCNH1 Foundation
Cure Lowe Foundation
Cure Mito Foundation
Cure Sanfilippo Foundation
Cure SMA
CureARS
CURED Nfp (Campaign Urging Research for Eosinophilic Diseases)
CureLGMD2i

CureSHANK
Cyclic Vomiting Syndrome Association
Cystic Fibrosis Research Institute
Dana's Angels Research Trust
debra of America
Dion Foundation for Children with Rare Diseases
Dravet Syndrome Foundation
Dreamsickle Kids Foundation, Inc.
Dup15q Alliance
EB Research Partnership
End AxD
Endosalpingiosis Foundation INC
Eosinophilic & Rare Disease Cooperative
Epilepsy Foundation of America
Fabry Support & Information Group
FAM177A1 RESEARCH FUND
Familial Dysautonomia Foundation
Family Heart Foundation
FD/MAS Alliance
Fighting H.A.R.D. Foundation
flok Health
Fondazione Telethon
Foundation for Angelman Syndrome Therapeutics (FAST)
Foundation for Prader-Willi Research
Foundation to Fight H-abc
FRAXA Research Foundation
Friedreich's Ataxia Research Alliance (FARA)
GABA-A Alliance
Galactosemia Foundation
Gaucher Community Alliance
GBS|CIDP Foundation International
Gene Giraffe Project
Global Genes
Global Liver Institute
Glut1 Deficiency Foundation
Haystack Project
HCU Network America
HD-CARE - Huntington's Disease Community Advocacy & Education
Hemophilia Foundation of Southern California
Hermansky-Pudlak Syndrome Network
Hope for Hypothalamic Hamartomas
Hope in Focus
Huntington's Disease Society of America
Hydrocephalus Association
HypoPARAthyroidism Association
Immune Deficiency Foundation
INADcure Foundation
Indo US Organization for Rare Diseases (IndoUSrare)
International Fibrodysplasia Ossificans Progressiva (FOP) Association
International Foundation for CDKL5 Research
International Rett Syndrome Foundation
International Waldenstrom's Macroglobulinemia Foundation
Jack McGovern Coats' Disease Foundation
Jansen's Foundation
Jordan's Guardian Angels
Juju and Friends CLN2 Warrior Foundation
Kabuki Syndrome Foundation
KCNQ2 Cure Alliance
Koolen-de Vries Syndrome Foundation
Krabbe Connect
Krishnan Family Foundation
Lambert Eaton LEMS Family Association
Lennox-Gastaut Syndrome (LGS) Foundation
Leukodystrophy Newborn Screening Action Network
LGMD Awareness Foundation, Inc
LGMD2D Foundation
Li-Fraumeni Syndrome Association (LFS Association)
Little Hercules Foundation
Lung Transplant Foundation
MECP2 Duplication Foundation
Mellie J Foundation
Mission: Cure
Mississippi Metabolics Foundation
MitoAction
MLD Foundation
MSUD Family Support Group
MTM-CNM Family Connection
Muscular Dystrophy Association
Myasthenia Gravis Association
Myositis Support and Understanding
N=1Collaborative
National Alliance for Caregiving
National Alliance for PANS/PANDAS Action
National Ataxia Foundation
National Eosinophilia Myalgia Syndrome Network
National Fragile X Foundation

National Health Council
National Kidney Foundation
National MPS Society
National Tay-Sachs & Allied Diseases Association
NBIA Disorders Association
Necrotizing Enterocolitis (NEC) Society
Neev Kolte & Brave Ronil Foundation
NephCure
Noah's Hope - Hope4Bridget
NTM Info & Research, Inc.
NW Rare Disease Coalition
Ogden CARES
Organic Acidemia Association
Parent Project Muscular Dystrophy
Partnership to Fight Chronic Disease
Pathways for Rare and Orphan Solutions
Petronille Healthy Society
PMD Foundation
Pompe Alliance
Project Alive
PTEN Hamartoma Tumor Syndrome Foundation
PWSA | USA - Prader-Willi Syndrome Association
Rare New England
Rare Trait Hope Fund
RareRising
Raymond A. Wood Foundation
Rett Syndrome Research Trust
Sanfilippo Children's Foundation
SANFILIPPO SUD
SATB2 Gene Foundation
SCAD Alliance
SHINE Syndrome Foundation
Shwachman-Diamond Syndrome Alliance Inc
Sickle cell association of Kentuckiana
Sisters Hope Foundation
Sleep Consortium
SMS Research Foundation
Spina Bifida Association
Stronger Than Sarcoidosis
Superior Mesenteric Artery Syndrome Research Awareness and Support
Supporters of Families with Sickle Cell Disease, Inc.
SynGAP Research Fund, DBA cureSYNGAP1
Taylor's Tale
Team Telomere
The Association for Frontotemporal Degeneration
The Bluefield Project to Cure FTD
The Bonnell Foundation: Living with cystic fibrosis
The Children’s Medical Research Foundation, Inc.
The DDX3X Foundation
The E.WE Foundation
The Global Foundation for Peroxisomal Disorders
THE KAT6 FOUNDATION INC
The LAM Foundation
The Little Legs Big Heart Foundation
The Louisa Adelynn Johnson Fund for Complex Disease
The MED13L Foundation Inc.
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The National Adrenal Diseases Foundation
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The Oxalosis and Hyperoxaluria Foundation
The RYR-1 Foundation
The Akari Foundation
Tough Genes
TSC Alliance
U.R. Our Hope
Undiagnosed Diseases Network Foundation
United Mitochondrial Disease Foundation
United MSD Foundation
United Ostomy Associations of America, Inc.
United Porphyrias Association
Uriel E. Owens Sickle Cell Disease Association of the Midwest
Vasculitis Foundation
Wake Up Narcolepsy, Inc.
Wilson Disease Association
Wisconsin Rare Disease Alliance
Wylder Nation Foundation
ZTTK SON-Shine Foundation

Cc: The Honorable Bernie Sanders, Chairman, Senate Committee on Health, Education, Labor and Pensions

The Honorable Bill Cassidy, Ranking Member, Senate Committee on Health, Education, Labor and Pensions
The Honorable Cathy McMorris Rodgers, Chair, House Committee on Energy and Commerce
The Honorable Frank Pallone, Ranking Member, House Committee on Energy and Commerce
The Honorable Robert Casey, Lead Sponsor, Creating Hope Reauthorization Act
The Honorable Markwayne Mullin, Lead Cosponsor, Creating Hope Reauthorization Act
The Honorable Sherrod Brown, Lead Cosponsor, Creating Hope Reauthorization Act
The Honorable Susan Collins, Lead Cosponsor, Creating Hope Reauthorization Act
The Honorable Michael McCaul, Lead Sponsor, Creating Hope Reauthorization Act
The Honorable Anna Eshoo, Lead Cosponsor, Creating Hope Reauthorization Act
The Honorable Gus Bilirakis, Lead Cosponsor, Creating Hope Reauthorization Act
The Honorable Nanette Barragan, Lead Cosponsor, Creating Hope Reauthorization Act
The Honorable Lori Trahan, Lead Cosponsor, Creating Hope Reauthorization Act
The Honorable Michael Burgess, Lead Cosponsor, Creating Hope Reauthorization Act