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July 22, 2024

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

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

The Honorable Bernie Sanders
Chairman
Committee on Health, Education, Labor & Pensions
United States Senate
322 Senate Dirksen Office Building
Washington, D.C. 20510

The Honorable Bill Cassidy
Ranking Member
Committee on Health, Education, Labor & Pensions
United States Senate
455 Senate Dirksen Office Building
Washington, D.C. 20510

Dear Senate Majority Leader Chuck Schumer, Senate Minority Leader McConnell, Chairman Sanders, and Ranking Member Cassidy,

On behalf of the 30 million Americans living with a rare disease, the undersigned 191 organizations write in support of the Creating Hope Reauthorization Act of 2024 and urge the Senate's swift passage of this critical legislation before the Rare Pediatric Disease Priority Review Voucher program's current authorization expires September 30, 2024. 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.

Since its creation by Congress in 2012, the Rare Pediatric Disease (RPD) Priority Review Voucher (PRV) program has helped spur rare disease drug development in pediatric populations and brought therapies to market for children affected by almost 40 rare diseases.[1] Many of these diseases lead to death or debilitating illness before the children reach adulthood, and almost none had any safe and effective FDA-approved therapies on the market before the program began. Additionally, more than half of all RPD PRV designations occurred in the last four years,[2] showing the program is fostering drug development where significant unmet therapeutic needs currently exist.

With more than 95% of rare diseases still lacking an FDA approved therapy, the RPD PRV program is important to our patient communities and a source of hope for the future development of safe and effective

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

[2] 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 ofRareof Rare Diseases. https://link.springer.com/epdf/10.1186/s13023-024-03097
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treatments. This program's authorization ends on September 30, 2024, and without a timely reauthorization, FDA will no longer be allowed to initiate the process necessary to issue new rare pediatric disease PRVs.

Therefore, we urge swift passage by the Senate of the Creating Hope Reauthorization Act to avoid a lapse in this critical program's authorization. We look forward to working with you on this important issue. For any questions or concerns, please contact the National Organization for Rare Disorders' Karin Hoelzer, Senior Director of Policy and Regulatory Affairs, at khoelzer@rarediseases.org or Hayley Mason, Policy Analyst, at hmason@rarediseases.org. Thank you for your consideration.

Sincerely,

National Organization for Rare Disorders
3q29 Foundation
Abetalipoproteinemia and Related Disorder Foundation
ADCY5.org
Adrenal Insufficiency United
Advocates for Medically Fragile Kids NC
Aicardi Goutieres Syndrome Advocacy Association (AGSAA)
Aislinn's Wish Foundation
Akari Foundation
Alpha-1 Foundation
Alport Syndrome Foundation
Alternating Hemiplegia of Childhood Foundation
AMDA
American Kidney Fund
American Porphyria Foundation
Angelman Syndrome Foundation
Aplastic Anemia & MDS International Foundation
ASXL Rare Research Endowment Foundation
Avery's Hope
Barth Syndrome Foundation
BDSRA Foundation
Born a Hero, Research Foundation
CACNA1A Foundation
Canavan Foundation
CDH International
Charcot Marie Tooth Association
Child Neurology Foundation
Chondrosarcoma Foundation
CMT Research Foundation
Coalition to Cure Calpain 3
Coalition to Cure CHD2
COMBINEDBrain, Inc.
Congenital Hyperinsulinism International
Cooley's Anemia Foundation
Creutzfeldt-Jakob Disease Foundation, Inc.
CSNK2A1 Foundation
CTNNB1 Connect and Cure
Cure CMD
Cure GABA-A
Cure GM1 Foundation
Cure KCNH1 Foundation
Cure MECP2 Duplication Syndrome
Cure Mito Foundation
Cure SMA
CureARS
CURED Nfp (Campaign Urging Research for Eosinophilic Disease
CureGRIN Foundation
CureLGMD2i Foundation
CureSHANK
Cystic Fibrosis Research Institute
Desmoid Tumor Research Foundation
Dup15q Alliance
Elise's Corner
End AxD
Eosinophilic & Rare Disease Cooperative (ERDC)
Everylife Foundation for Rare Diseases
FAM177A1 Research Fund
Familial Dysautonomia Foundation
Fighting H.A.R.D. Foundation
flok Health
Foundation for Angelman Syndrome Therapeutics (FAST)
Foundation to Fight H-abc
FRAXA Research Foundation
Friedreich's Ataxia Research Alliance (FARA)
GABA-A Alliance
Gaucher Community Alliance
GBS|CIDP Foundation International
Global Liver Institute
Glut1 Deficiency Foundation
GRIN2B Foundation
HCMA
HCU Network America

Hemophilia Federation of America
Hemophilia Foundation of Southern California
Hereditary Angioedema Association
Heterotaxy Connection
Histiocytosis Association, Inc.
Hope For Hypothalamic Hamartomas
Hope in Focus
Hydrocephalus Association
Hyper IgM Foundation
HypoPARAthyroidism Association
Immune Deficiency Foundation
INADcure Foundation
Indo US Organization for Rare Diseases
International FOXP1 Foundation
International Rett Syndrome Foundation
Jack McGovern Coats' Disease Foundation
Kabuki Syndrome Foundation
KCNQ2 Cure Alliance
KIF1A.org
Koolen-de Vries Syndrome Foundation
KrabbeConnect
Lennox-Gastaut Syndrome (LGS) Foundation
LGDA
LGMD Awareness Foundation
LGMD2D Foundation
Malan Syndrome Foundation
Marshall-Smith Syndrome Organization of the USA
MECP2 Duplication Syndrome
MED13L
MED13L Foundation
Mellie J Foundation
MitoAction
MLD Foundation
Moebius Syndrome Foundation
MSUD Family Support Group
Mucolipidosis Type IV Foundation
Muscular Dystrophy Association
National Alliance for Caregiving
National Ataxia Foundation
National Bleeding Disorders Foundation
National Eosinophilia Myalgia Syndrome Network
National MALS Foundation
National MPS Society
National Niemann-Pick Disease Foundation
National PKU Alliance
National Tay-Sachs & Allied Diseases Association
NBIA Disorders Association
Necrotizing Enterocolitis (NEC) Society
NephCure
NF Northeast
Noah's Hope
NTM Info & Research, Inc.
NW Rare Disease Coalition
Ogden CARES
Organic Acidemia Association
Parent Project Muscular Dystrophy
PCD Foundation
Pediatric Retinal Research Foundation
Pheo Para Alliance
PMD Foundation
PRISMS, Inc. (Parents and Researchers Interested in Smith-Magenis Syndrome)
Project 8p Foundation
Project Alive
Pulmonary Hypertension Association
PWSA | USA
Rare Disease Innovations Institute
Rare Disease Renegades
Rare Trait Hope Fund
Rare Village Foundation
RASopathies Network
Rein in Sarcoma
Rett Syndrome Research Trust
Sanfilippo Children's Foundation
SANFILIPPO SUD
SATB2 Gene Foundation
SHINE Syndrome Foundation
Shwachman-Diamond Syndrome Alliance
Sleep Consortium
SMS Research Foundation
Spina Bifida Association
Stevens-Johnson Syndrome Foundation
STXBP1 Foundation
Superior Mesenteric Artery Syndrome Research Awareness and Support
SynGAP Research Fund
TESS Research Foundation for SLC13A5 Epilepsy
The After Organization Inc
The Bonnell Foundation: Living with cystic fibrosis
The Caring Board
The Children's Medical Research Foundation, Inc.
The DDX3X Foundation
The Dion Foundation for Children with Rare Disease
The E.WE Foundation
The Ehlers-Danlos Society

The Healing NET Foundation
The Jansen's Foundation
The KAT6A Foundation
The Little Legs Big Heart Foundation
The Mast Cell Disease Society
The Mended Hearts, Inc.
The National Adrenal Diseases Foundation
The Oley Foundation
The Oxalosis and Hyperoxaluria Foundation
The Progeria Research Foundation
The RYR-1 Foundation
The Speak Foundation
The Sudden Unexplained Death in Childhood Foundation
Thrive with PK
TSC Alliance
United Leukodystrophy Foundation
United Mitochondrial Disease Foundation
United MSD Foundation
United Ostomy Associations of America, Inc.
United Porphyrias Association
Upequity
Vasculitis Foundation
Wake Up Narcolepsy, Inc.
Wisconsin Rare Disease Alliance
Yaya Foundation for 4H Leukodystrophy
Yellow for Yiannis IRF2BPL Foundation