June 24, 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

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WASHINGTON, DC 20036
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rarediseases.org ▪ orphan@rarediseases.org

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

The Honorable Bill Cassidy
Ranking Member
Committee on Health, Education, Labor & Pensions
United States Senate
428 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 132 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 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
ADCY5.org
Adrenal Insufficiency United
Advocates for Medically Fragile Kids NC
Aicardi Goutieres Syndrome Advocacy Association (AGSAA)
Aislinn's Wish Foundation

[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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Alpha-1 Foundation
AMDA
American Kidney Fund
Angelman Syndrome Foundation
Avery's Hope
Barth Syndrome Foundation
BDSRA Foundation
Born a Hero, Research Foundation
CACNA1A Foundation
Canavan Foundation
Child Neurology Foundation
Chondrosarcoma Foundation
Coalition to Cure Calpain 3
Congenital Hyperinsulinism International
Cooley's Anemia Foundation
Creutzfeldt-Jakob Disease Foundation, Inc.
CSNK2A1 Foundation
CTNNB1 Connect and Cure
Cure CMD
Cure GM1 Foundation
Cure MECP2 Duplication Syndrome
Cure SMA
CURED Nfp (Campaign Urging Research for Eosinophilic Disease)
CureLGMD2i Foundation
Cystic Fibrosis Research Institute
Desmoid Tumor Research Foundation
Elise's Corner
End AxD
Eosinophilic & Rare Disease Cooperative (ERDC)
EveryLife Foundation for Rare Diseases
Familial Dysautonomia Foundation
Fighting H.A.R.D. Foundation
Foundation for Angelman Syndrome Therapeutics (FAST)
Foundation to Fight H-abc
Friedreich's Ataxia Research Alliance (FARA)
GABA-A Alliance
Gaucher Community Alliance
GBS|CIDP Foundation International
Global Liver Institute
HCMA
HCU Network America
Hemophilia Federation of America
Hereditary Angioedema Association
Histiocytosis Association, Inc.
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
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
Mellie J Foundation
MitoAction
MLD Foundation
Moebius Syndrome Foundation
Muscular Dystrophy Association
National Ataxia Foundation
National Bleeding Disorders Foundation
National MALS Foundation
National MPS Society
National Niemann-Pick Disease Foundation
National PKU Alliance
National Tay-Sachs & Allied Diseases Association
Necrotizing Enterocolitis (NEC) Society
NF Northeast
Noah's Hope
NW Rare Disease Coalition
Organic Acidemia Association
Parent Project Muscular Dystrophy
Pheo Para Alliance
PMD Foundation
Project 8p Foundation
Project Alive
Pulmonary Hypertension Association
PWSA | USA
Rare Disease Innovations Institute
Rare Disease Renegades
Rein in Sarcoma
Sanfilippo Children's Foundation
SANFILIPPO SUD
SATB2 Gene Foundation
SHINE Syndrome Foundation
Sleep Consortium
Stevens-Johnson Syndrome Foundation

STXBP1 Foundation
TESS Research Foundation for SLC13A5 Epilepsy
The Akari Foundation
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 Diseases
The E.WE Foundation
The Jansen's Foundation
The Little Legs Big Heart Foundation
The Mast Cell Disease Society
The National Adrenal Diseases Foundation
The Oley Foundation
The Oxalosis and Hyperoxaluria Foundation
The Progeria Foundation
The RYR-1 Foundation
The Speak 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
Wake Up Narcolepsy, Inc.
Wisconsin Rare Disease Alliance
Yaya Foundation for 4H Leukodystrophy