November 19, 2025

The Honorable John Thune
Majority Leader
U.S. Senate
Washington, D.C. 20510

The Honorable Mike Johnson
Speaker
U.S. House of Representatives
Washington, D.C. 20515

The Honorable Chuck Schumer
Minority Leader
U.S. Senate
Washington, D.C. 20510

The Honorable Hakeem Jeffries
Minority Leader
U.S. House of Representatives
Washington, D.C. 20515

Dear Leader Thune, Leader Schumer, Speaker Johnson, and Leader Jeffries:

The 117 undersigned organizations, representing a diverse group of stakeholders in American healthcare, have come together to **urge Congress to include two crucial pieces of legislation in any year-end package - 1) *Give Kids a Chance Act* (S. 932 /H.R. 1262); and 2) *Accelerating Kids' Access to Care Act* (S. 752 / H.R. 1509)**. These bipartisan, bicameral bills represent hope for the rare disease community and will meaningfully improve access to life-changing and life-saving treatments.

Give Kids a Chance Act

The Rare Pediatric Disease Priority Review Voucher (RPD PRV) Program has received broad, bipartisan and bicameral support since its inception in 2012. In September, the *Give Kids a Chance Act* passed unanimously out of the House Energy & Commerce Committee. In the previous Congress, the program passed unanimously through the full House of Representatives and was also included in the bipartisan health care package at the end of last year. **We urge Congress to reauthorize the RPD PRV Program by passing the *Give Kids a Chance Act* this year.**

Last year's expiration of the RPD PRV Program is already negatively impacting decisions about rare disease therapies in earlier stages of development and is discouraging investment in pediatric products, threatening treatments for patients in the years ahead. It takes years and considerable capital to bring new rare disease therapies to market, and companies need predictability to make business decisions, plan future research & development, and attract investors. On average, the development timeline for rare disease treatments is 10-15 years.[1] The *Give Kids a Chance Act* ensures the PRV incentive is preserved for future patients.

The RPD PRV Program was established more than a decade ago to provide crucial incentives for pharmaceutical and biotech companies to develop new therapies for rare conditions. RPD PRVs make it possible for companies to invest in these products - including potentially curative cell and gene therapies -which address critical, unmet medical needs. The program has no cost to taxpayers, and since 2012 more than 60 PRVs have been awarded across 40 different pediatric diseases. Simply put, the RPD PRV Program supports faster access to cures for children with rare diseases. Failure to reauthorize the RPD PRV Program hurts the pipeline for new treatments.

Accelerating Kids' Access to Care Act

Unfortunately, even when Medicaid covers complex care patients face barriers receiving treatment. One well-documented challenge is the need to travel out-of-state to receive care, which is often the case for patients receiving cell and gene therapies or other specialized care. Often there are a limited number of hospitals or qualified treatment centers, concentrated in limited geographic areas. When providers treat out-of-state Medicaid patients, they must be credentialed by the patients' home state Medicaid program – a process that can be lengthy, time consuming, and administratively complex. This process can take months, and this regulatory burden can

cause dangerous delays in care - when patients don't have time to waste. Delayed care can lead to worse outcomes - or even death, in some cases.

The *Accelerating Kids' Access to Care Act* would streamline this process for specialty providers caring for children with complex medical needs, while protecting programmatic safeguards. The legislation enjoys strong bipartisan support, sponsored by more than 135 Members of Congress, and it is endorsed by more than 200 organizations. **We urge Congress to pass the *Accelerating Kids' Access to Care Act* to reduce harmful treatment delays for children on Medicaid who must travel out-of-state.**

Our diverse organizations recognize the need to incentivize innovation, find sustainable financing mechanisms for new treatments, and alleviate administrative barriers to bring treatments to rare disease patients. We have come together to urge you to include the aforementioned bills in any year-end legislative package.

Your leadership in advancing these crucial policies is greatly appreciated, and we stand ready to support in whatever way we can. If you have questions, please feel free to contact Erica Cischke at ecischke@alliancerm.org.

Sincerely,

Alliance for Regenerative Medicine

Abigail Wexner Research Institute, Nationwide Children's Hospital

Acromegaly Community Inc.

Advocates for Responsible Care

AiArthritis

Akari Foundation

American Behcet's Disease Association (ABDA)

American Kidney Fund

American Society for Transplantation & Cellular Therapy

American Transplant Foundation

Angelman Syndrome Foundation

Association of Pediatric Hematology/Oncology Nurses

Best Day Ever Foundation

Blood Cancer United (formerly The Leukemia & Lymphoma Society)

Bubba's Light, Inc.

CACNA1A Foundation

California Life Sciences

Canavan Research Foundation

Cancer Support Community

Caregiver Action Network

CARES Foundation

CDG CARE

Cedars-Sinai

Charlie's Cure

Children's Cardiomyopathy Foundation

Chondrosarcoma Foundation

Choroideremia Research Foundation

CLOVES Syndrome Community

Coalition for Hemophilia B

Cory Heidaran Charitable Foundation (CHCF)

CTNNB1 Connect and Cure

Cure CMD

Cure Sanfilippo Foundation

Cure SMA

CureDuchenne

CureLGMD2i Foundation

CureSHANK

Danon Disease Foundation

debra of America

Dravet Syndrome Foundation

Dreamsickle Kids Foundation Inc.

EB Research Partnership

Emily Whitehead Foundation

EveryLife Foundation for Rare Diseases

Fighting H.A.R.D. Foundation

Foundation for Angelman Syndrome Therapeutics

Foundation for Fighting Blindness

Foundation for Prader-Willi Research

Galactosemia Foundation

Gaucher Community Alliance

Genetic Alliance

Global Genes

Global Liver Institute

Good Days

Immune Deficiency Foundation

INADcure Foundation

Infusion Access Foundation

Innovative Genomics Institute

Institute for Gene Therapies

International Rett Syndrome Foundation

Lennox-Gastaut Syndrome (LGS) Foundation

Little Hercules Foundation

Lupus and Allied Diseases Association, Inc.

Lymphedema Advocacy Group

Lymphoma Research Foundation

M-CM Network

MEPAN Foundation

MLD Foundation

Monoamine Oxidase Deficiency Foundation

MSUD Family Support Group

Muenzer MPS Research & Treatment Center

National Ataxia Foundation

National Bleeding Disorders Foundation

National Leiomyosarcoma Foundation / Sarcoma Coalition

National MPS Society

National Organization for Rare Disorders (NORD)

National Patient Advocate Foundation

National PKU Alliance

National Scleroderma Foundation

NBIA Disorders Association

NF Northeast

Noah's Hope - Hope4Bridget Foundation

Organic Acidemia Association

Parent Project Muscular Dystrophy

PBD Project

Phelan-McDermid Syndrome Foundation

Project Alive

PXE International

Rare Access Action Project

Rare Mamas

Rett Syndrome Research Trust

RUNX1 Research Program

SATB2 Gene Foundation

SETBP1 Society

Share & Care Cockayne Syndrome Network, Inc.

Speak Foundation

STXBP1 Foundation

Sudden Arrhythmia Death Syndromes Foundation

Taylor's Tale

The Dion Foundation for Children with Rare Disease

The E.WE Foundation

The Global Foundation for Peroxisomal Disorders

The LAM Foundation

The LCC Foundation

The Louisa Adelynn Johnson Fund for Complex Disease

The National Adrenal Diseases Foundation

The Oxalosis and Hyperoxaluria Foundation

Tourette Association of America

Transplant Families

TSC Alliance

Turner Syndrome Society of the United States

United Leukodystrophy Foundation

United Mitochondrial Disease Foundation

United MSD Foundation

US Hereditary Angioedema Association

Usher 1F Collaborative

WashU Center of Regenerative Medicine

Wilson Disease Association

Yaya Foundation for 4H Leukodystrophy

[1] https://pmc.ncbi.nlm.nih.gov/articles/PMC10752072/#:~:text=Treatments%20for%20rare%20diseases%2C%20however,much%20as%202.6%20billion%20dollars.