November 13, 2024

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

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

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

The Honorable Mitch McConnell
Minority Leader
U.S. Senate
Washington, D.C. 20510

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

The 137 undersigned organizations, representing a diverse group of stakeholders in American healthcare, have come together to urge Congress to pass three crucial pieces of legislation: reauthorization of the *Rare Pediatric Disease Priority Review Voucher Program*, the *Medicaid Value-Based Payments for Patients (MVP) Act* (S. 4204 / H.R. 2666), and the *Accelerating Kids' Access to Care Act* (S. 2372 / H.R. 4758) before the end of the year. These bipartisan, bicameral bills represent hope for the rare disease community and will meaningfully improve access to life-changing and lifesaving treatments.

The Rare Pediatric Disease (RPD) Priority Review Voucher (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. If the program is discontinued, the pipeline of potential cures could languish. RPD PRVs have enabled the development of more than 50 new rare disease treatments since its inception, at no cost to U.S. taxpayers. **The FDA's authority to issue RPD PRVs expires on December 20, 2024, and we urge Congress to reauthorize it for at least five years to support continued innovation.**

The pipeline of cancer and rare disease treatments is growing, but our healthcare system is struggling to keep pace. Innovative new therapies often require a significant upfront investment but can yield years, decades, or even a lifetime of benefit. Value-based payment arrangements (VBAs) that tie drug reimbursement to outcomes rather than volume mitigate uncertainty about the long-term durability of high-cost, high-value therapies and give payers additional tools to manage their budgets. Medicaid programs and commercial payers' interest in VBAs is growing, particularly for cell and gene therapies. As evidence, the Center for Medicare and Medicaid Innovation recently unveiled the Cell and Gene Therapy Access Model to support the adoption of VBAs for Sickle Cell gene therapies. However, existing laws and regulations present barriers to more widespread uptake of VBAs. The bipartisan MVP Act provides clarity for manufacturers entering into VBAs and ensures that such arrangements do not inappropriately distort Medicaid prices. The bill also requires a GAO study on the effectiveness of VBAs on patient access and overall health system costs. VBAs enable payers to overcome cost barriers and cover breakthrough treatments. **We urge Congress to pass the MVP Act to ensure that patients have timely access to new therapies.**

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 centers of excellence, 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. 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. **We urge Congress to pass this commonsense bill 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 end-of-year legislative package.

Your leadership in advancing these crucial policies is greatly appreciated, and we stand ready to support in whatever way we can.

Sincerely,

Alliance for Regenerative Medicine
AADC Family Network
Acromegaly Community
Advocates for Responsible Care
AKARI FOUNDATION
Alliance to Cure Cavernous Malformation
Alpha-1 Foundation
American Behcet's Disease Association
American Kidney Fund
Angelman Syndrome Foundation
Association of Pediatric Hematology/Oncology Nurses
Autoimmune Association
Barth Syndrome Foundation
BCM Families Foundation
BDSRA Foundation
Best Day Ever Foundation
CACNA1A Foundation
California Life Sciences
Canavan Foundation
CDG CARE
Charcot-Marie-Tooth Association (CMTA)
Charlie's Cure
chILD Foundation
Children's Sickle Cell Foundation, Inc.
Institute for Gene Therapies
Chondrosarcoma Foundation
Cleveland Clinic
CLOVES Syndrome Community
Cockayne Syndrome Network, Share and Care
CARES Foundation
Cooley's Anemia Foundation
CSNK2A1 Foundation
CTNNB1 Connect and Cure
Cure CMD
Cure LBSL
Cure Sanfilippo Foundation
Cure SMA
CureDuchenne
CureSHANK
debra of America
Dion Foundation for Children with Rare Diseases
Dravet Syndrome Foundation
Dreamsickle Kids Foundation
EveryLife Foundation
FAM177A1 RESEARCH FUND
FamilieSCN2A Foundation
Fighting Blindness Foundation
Fighting H.A.R.D. Foundation

FND Hope

Foundation for Angelman Syndrome Therapeutics (FAST)

Foundation to Fight H-abc

Friedreich's Ataxia Research Alliance (FARA)

Galactosemia Foundation

Gaucher Community Alliance

Genetic Alliance

Global Liver Institute

Good Days

Help 4 HD International

Hunter's Hope Foundation

Hydrocephalus Association

Hypertrophic Cardiomyopathy Association

Immune Deficiency Foundation

Infusion Access Foundation

Innovative Genomics Institute

International Foundation for Autoimmune and Autoinflammatory Arthritis

International Rett Syndrome Foundation

Lennox-Gastaut Syndrome (LGS) Foundation

Leukodystrophy Newborn Screening Action Network

Little Hercules Foundation

Lowe Syndrome Association

Lupus and Allied Diseases Association, Inc.

Lymphedema Advocacy Group

Malan Syndrome Foundation

Mission MSA

Mitochondrial Medicine Society

MLD Foundation

MSUD Family Support Group

Muenzer MPS Research & Treatment Center

National Ataxia Foundation

National Gaucher Foundation

National MPS Society

National Organization for Rare Disorders

National Patient Advocate Foundation

National PKU Alliance

National Scleroderma Foundation

NBIA Disorders Association

Neurofibromatosis Network

Neurofibromatosis Northeast

Noah's Hope - Hope4Bridget Foundation

NTM Info & Research

Organic Acidemia Association

Parent Project Muscular Dystrophy

Phelan-McDermid Syndrome Foundation

Pheo Para Alliance

Project Alive

Rare Mamas

Rein in Sarcoma

Rett Syndrome Research Trust

RUNX1 Research Program

SADS Foundation | Sudden Arrhythmia Death Syndromes

SATB2 Gene Foundation

SETBP1 Society

Shwachman-Diamond Syndrome Foundation

Soft Bones

STXBP1 Foundation

Taylor's Tale

The Bonnell Foundation: Living with Cystic Fibrosis

The Canavan Research Foundation

The Champ Foundation

The CMT Research Foundation

The Coalition for Hemophilia B

The Ehlers-Danlos Society

The Foundation for Prader-Willi Research

The Global Foundation for Peroxisomal Disorders

The LAM Foundation

The LCC Foundation

The Leukemia & Lymphoma Society

The Matthew and Andrew Akin Foundation

The National Adrenal Diseases Foundation

The NOTA (Network of Tyrosinemia Advocates)

The Oxalosis and Hyperoxaluria Foundation

Tourette Association of America

TSC Alliance

Tulane University

Turner Syndrome Society of the United States

United Leukodystrophy Foundation

United Mitochondrial Disease Foundation

United MSD Foundation

United Porphyrias Association

University of Wisconsin-Madison School of Medicine and Public Health

University of Iowa Carver College of Medicine

Usher Syndrome Coalition

UW Health Kids

Vasculitis Foundation

Washington University School of Medicine

Wilson Disease Association

Yaya Foundation for 4H Leukodystrophy