Rare Disease Company Coalition

BY ELECTRONIC DELIVERY

December 18, 2024

The Honorable Charles 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, DC 20510

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

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

RE: Rare Disease Company Coalition Urges Passage of Continuing Resolution to Reauthorize Rare Pediatric Disease Priority Review Voucher Program

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

On behalf of the Rare Disease Company Coalition (RDCC) and the 1 in 10 Americans living with a rare disease, I thank you for the inclusion of the reauthorization of the rare pediatric disease priority review voucher (PRV) program in the continuing resolution (CR). Without reauthorization by December 20, 2024, the rare pediatric disease PRV program will begin to sunset, and hope for millions of children may vanish. Therefore, we urge the swift passage of this critical legislation.

The rare pediatric disease PRV program is a proven policy that has benefitted hundreds of thousands of patients living with a rare disease.[1] Over the past 12 years, the program has been an innovation-driving and cost-effective policy that spurs research & development (R&D) in rare pediatric diseases.

In the United States, a rare disease is defined as a condition that affects fewer than 200,000 people.[2] Approximately 30 million Americans are affected by one of over 10,000 rare diseases,[3] and only 5% of those rare diseases have a Food and Drug Administration (FDA)-approved treatment.[4] Further complicating this unmet need, rare disease drug development is extraordinarily challenging. Rare diseases are characterized by small patient populations, complex and variable disease presentation, limited natural

[1] Rare Disease Company Coalition. Impact of the Priority Review Voucher Program on Rare Pediatric Disease Drug Development. May 2024.
[2] Orphan Drug Act, Public Law 414, U.S. Statutes at Large 96 (1982): 2049-2066.
[3] National Center for Advancing Translational Sciences (NCATS). Delivering Hope for Rare Diseases. January 2023.
[4] Fermaglich LJ, Miller KL. A comprehensive study of the rare diseases and conditions targeted by orphan drug designations and approvals over the forty years of the Orphan Drug Act. Orphanet J Rare Dis. 2023 June 3;18(1):163

history, slow disease progression with often irreversible symptoms, and a lack of defined endpoints and biomarkers.

The unique challenges of rare disease drug development are further amplified in pediatric populations, and there remains a pressing need to invest in rare pediatric disease R&D. One in two patients living with a rare disease are children – and a third of those children will not live to see their fifth birthday.[5] Smart policy is needed to ensure that these children have access to innovative therapies that can slow, stop, or even reverse the progression of their disease or condition.

The 26 companies represented in the RDCC have spent a collective 530 years dedicated to rare disease innovation. Our member companies invest, on average, nearly 60 percent of annual expenditures on R&D – over $17 billion annually – to ensure more rare disease patients have access to new and innovative treatments.[6] RDCC members have brought more than 50 rare disease treatments to market, and have over 200 programs in our development pipelines, many of which would be the first ever FDA-approved therapy for their respective patient communities.[7]

The rare pediatric disease PRV program enables rare disease companies to provide hope to children living with a rare disease while reinvesting critical dollars into additional R&D for rare disease treatments. The program has little to zero cost to taxpayers and remains a critical incentive to attract investment into rare disease drug development.

The reauthorization included in the continuing resolution would protect the rare disease ecosystem by providing stability to innovators, encourage investment in rare disease in R&D, and, most importantly, benefit patients by advancing drug development for rare pediatric diseases.

The RDCC urges lawmakers to act quickly and pass the continuing resolution to ensure that this critical program is reauthorized prior to its expiration on December 20.

If you have any questions or would like to discuss further, please contact me at stacey@rarecoalition.com.

Signed,

Stacey Frisk
Executive Director
Rare Disease Company Coalition

[5] Global Genes. Rare Disease Facts.
[6] Rare Disease Company Coalition (RDCC). 2024 Outlook. May, 2024.
[7] Ibid.