Rare Disease Company Coalition

BY ELECTRONIC DELIVERY

February 6, 2025

The Honorable John Thune
Majority Leader
United States Senate
511 Dirksen Senate Office Building
Washington, D.C. 20510

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

The Honorable Chuck Schumer
Minority Leader
United States Senate
322 Hart Senate Office Building
Washington, DC 20510

The Honorable Hakeem Jeffries
Minority Leader
United States House of Representatives
2267 Rayburn House Office Building
Washington, D.C. 20515

RE: Rare Disease Biotech Innovators Urge Immediate Restoration of Rare Pediatric Disease Priority Review Voucher (PRV) Program

Dear Majority Leader Thune, Minority Leader Schumer, Speaker Johnson, and Minority Leader Jeffries,

On behalf of the rare disease companies we represent, and the 1 in 10 Americans living with a rare disease, we urge you to support the swift reauthorization of the rare pediatric disease priority review voucher (PRV) program. The program began to lapse on December 20, 2024 and without urgent Congressional action, hope for millions of children may vanish for good.

Reauthorization of the program would ensure that the rare pediatric disease PRV program will continue to benefit hundreds of thousands of patients living with a rare disease. The program has received broad, bipartisan, and bicameral support since its inception in 2012. Most recently in the 118th Congress, the program enjoyed unanimous passage through the House of Representatives and was included in the health title of the end-of-year package. Further, the Food and Drug Administration (FDA) has supported the reauthorization of the program via inclusion in their anomalies list. A diverse array of stakeholders, from Congress to the Administration, from patient advocates to academia, and from the investor community to rare disease innovators, understand that the PRV program is a proven 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.[1] Approximately 30 million Americans are affected by one of the over 10,000 rare diseases,[2] and only 5% of those rare diseases have an FDA-approved treatment.[3] Further complicating this unmet need, rare disease drug development is extraordinarily challenging. Rare diseases are characterized by small patient

[1] Orphan Drug Act, Public Law 414, U.S. Statutes at Large 96 (1982): 2049-2066.

[2] National Center for Advancing Translational Sciences (NCATS). Delivering Hope for Rare Diseases. January 2023.

[3] 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

populations, complex and variable disease presentation, limited natural 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 2 patients diagnosed with a rare disease are children – and a third of those children will not live to see their fifth birthday.[4] 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.

We have an intimate appreciation of the challenges of drug development, as well as the tremendous potential of scientific advances to transform the lives of people living with rare diseases. Across the 27 companies represented in the Rare Disease Company Coalition (RDCC), we have spent a collective 530 years dedicated to rare disease innovation. On average, our companies invest nearly 60 percent of annual revenue on R&D – over $17 billion annually – to ensure more rare disease patients have access to new and innovative treatments.[5] 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.[6]

The rare pediatric disease PRV program enables companies like ours 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.

Programs like the rare pediatric disease PRV are crucial to mitigating the risks associated with investment in the rare disease space. Without tools like the PRV program, investors may view rare disease drug development as too risky – and for small and emerging companies that rely on capital markets for funding, that could make the difference between bringing a product to market and shuttering a program. Research shows that many rare disease companies are struggling to survive in the current policy and funding ecosystems,[7] and policies like the PRV program are necessary to attract investors that are fundamental to rare disease drug development.

A recent study has shown that the PRV program is an effective and proven policy that spurs new rare disease treatments and cures. Innovation driven by the program has benefited over 200,000 rare disease patients across 47 unique indications.[8] And this innovation is meeting previously unmet needs. More than 90 percent of all PRVs were awarded to therapies for indications with no approved therapy on the market.[9] Furthermore, uptake of the program is still expanding, as more than half of all vouchers were

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[4] Global Genes. Rare Disease Facts.

[5] Rare Disease Company Coalition (RDCC). 2024 Outlook. May, 2024.

[6] Ibid.

[7] Masia, Neal. Health Capital Group. Rare Disease Companies in the Public Markets: Challenging Performance Against a Backdrop of Policy Uncertainty. October 2023.

[8] Rare Disease Company Coalition (RDCC). Impact of the Priority Review Voucher Program on Rare Pediatric Disease Drug Development. May 2024.

[9] Ibid.

granted in the last four years alone.[10] Rare disease treatments take longer than prevalent disease treatments to move from bench to bedside. On average, the development timeline for rare disease treatments is 15 years.[11,12] As the program has only been operational for 12 years, its true impact is still being realized.

Immediate restoration of the rare pediatric disease PRV program is critical to achieving our important mission to address the unmet medical needs of the rare disease community. Every minute the PRV program remains expired jeopardizes investment in critical innovations that could transform the lives of children living with rare, often fatal and progressively debilitating, diseases.

As leaders in the rare disease community, we urge policymakers to prioritize the reauthorization of this critical program as swiftly as possible.

If you have any questions or would like to discuss further, please contact Stacey Frisk, Executive Director of the Rare Disease Company Coalition at stacey@rarecoalition.com.

Signed,

[10] Ibid.

[11] Brown DG, Wobst HJ, Kapoor A, Kenna LA, Southall N. Clinical development times for innovative drugs. Nat Rev Drug Discov. 2022 Nov;21(11):793-794.

[12] HHS ASPE. Examination of Clinical Trial Costs and Barriers for Drug Development.

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Cc: The Honorable Bill Cassidy, Chairman, Senate Committee on Health, Education, Labor and Pensions
The Honorable Bernie Sanders, Ranking Member, Senate Committee on Health, Education, Labor and Pensions
The Honorable Brett Guthrie, Chair, House Committee on Energy & Commerce
The Honorable Frank Pallone, Ranking Member, House Committee on Energy & Commerce