Rare Disease Company Coalition

BY ELECTRONIC DELIVERY

September 12, 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 Biotech Innovators Urge Timely Reauthorization of Rare Pediatric Disease Priority Review Voucher (PRV) Program

Dear Majority Leader Schumer, Minority Leader McConnell, 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 passage of S.4583/H.R. 7384, the Creating Hope Reauthorization Act of 2024. The rare pediatric disease priority review voucher (PRV) program will begin to sunset after September 30, 2024, and without Congressional action, hope for millions of children may vanish. In the event that reauthorization legislation is not enacted in a timely manner, an extension via the continuing resolution as referenced in FDA's Authorization Issues[1] would allow the program to continue, representing an important stopgap measure that would prevent a destabilization of the rare disease drug development ecosystem.

The Creating Hope Reauthorization Act would reauthorize the rare pediatric disease priority review voucher (PRV) program, a proven policy that has benefitted hundreds of thousands of patients living with a rare disease. 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 the over 10,000 rare diseases,[3] and only 5%

[1] Office of Management and Budget. 2025 CR Authorization Issues. August 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.

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 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 research & development. One in 2 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.

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 25 companies represented in the Rare Disease Company Coalition (RDCC), we have spent a collective 530 years dedicated to rare disease innovation. Our 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 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 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,[8] 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

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[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

[5] Global Genes. Rare Disease Facts.

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

[7] Ibid.

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

patients across 47 unique indications.[9] 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.[10] Furthermore, uptake of the program is still expanding, as more than half of all vouchers were granted in the last four years alone.[11] 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.[12,13] As the program has only been operational for 12 years, its true impact is still being realized.

Continuity 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. However, per the current provisions in the law,[14] the PRV program will begin to sunset after September 30. After that date, eligibility to receive a voucher is limited to products that have received rare pediatric disease designation, placing this critical program out of reach for many rare disease treatments. Importantly, FDA notes in its update to sponsors[15] that the Agency has limited resources available, and given the surge in requests expected a few months prior to the deadline "meeting all review timelines may not be feasible." Failure to reauthorize this critical program on time would result in reduced incentives for the discovery of new treatments, jeopardize programs already in development, and destabilize investment in new treatments, resulting in fewer treatments and cures for people living with rare diseases.

A long-term extension of the program, as proposed in the Creating Hope Reauthorization Act, would protect the rare disease ecosystem by providing stability to innovators, encourage investment in rare disease in R&D, and ultimately benefit patients by advancing drug development for rare pediatric diseases.

As leaders in the rare disease community, we urge Congressional leadership to act quickly and ensure that this critical program is reauthorized prior to its expiration on September 30.

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,

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[9] Rare Disease Company Coalition (RDCC). Impact of the Priority Review Voucher Program on Rare Pediatric Disease Drug Development. May 2024.
[10] Ibid.
[11] Ibid.
[12] 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.
[13] HHS ASPE. Examination of Clinical Trial Costs and Barriers for Drug Development.
[14] 21 USC 360ff: Priority review to encourage treatments for rare pediatric diseases
[15] Food and Drug Administration. Rare Pediatric Disease Designation and Priority Review Voucher Programs:Information for product sponsors.

Rare Disease Company Coalition

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