BY ELECTRONIC DELIVERY

November 12, 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 by prioritizing the passage of S.932/H.R. 1262, *the Give Kids a Chance Act* before the end of the year**. The authorization for the PRV program expired last December, and without urgent Congressional action, hope for millions of children may vanish for good.

Last year's expiration is already having a significant negative impact on companies making decisions regarding their early-stage pipelines and is discouraging investment in pediatric products. It takes years to bring new rare disease therapies to market, and companies need predictability to make business decisions, plan future research & development (R&D), and attract investors.

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. Last month, the *Give Kids a Chance Act* passed unanimously through the House Energy & Commerce Committee. In the 118th Congress, the program passed unanimously through the full House of Representatives. A diverse array of stakeholders, from policymakers to patient advocates, from innovators to investors, understand that the PRV program is a proven innovation-driving and cost-effective policy that spurs 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 percent 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 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, including cell and gene therapies, that can slow, stop, or even reverse the progression of their disease or condition.

As leaders in rare disease innovation, our ability to deliver first-in-class therapies for rare pediatric diseases would not be possible without the incentives provided in the Orphan Drug Act supplemented by the Rare Pediatric Disease PRV program. Notably, many of these medicines are the first-ever FDA-approved therapy for their respective patient communities

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 and ensuring that approved therapies are available for all patients in need. **The program has 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,[5] 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.[6] 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

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[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

[4] Global Genes. Rare Disease Facts.

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

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

market.[7] Furthermore, uptake of the program is still expanding, as more than half of all vouchers were granted in the last four years alone.[8] 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.[9,10] 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 Congress to prioritize the reauthorization of this critical program by passing S.932/H.R. 1262, *the Give Kids a Chance Act*, before the end of the year.

Signed,

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[7] Ibid.

[8] Ibid.

[9] 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.

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

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