Rare Disease Company Coalition

Impact Report: Lapse of the Rare Pediatric Disease Priority Review Voucher Program

December 2025

Introduction

The rare pediatric disease (RPD) priority review voucher (PRV) program encourages the development of treatments for rare pediatric diseases. To date, it has addressed unmet need across more than 50 distinct indications and benefited more than 200,000 people living with a rare disease. However, the program lapsed on December 20, 2024. Restoration of this critical incentive is necessary to spur investment in and development of new rare disease treatments.

Program Impact

The rare pediatric disease priority review voucher program **encourages the development** of treatments for rare pediatric diseases, which are associated with **urgent, unmet medical need.**

People with rare pediatric diseases have **limited or no treatment options** due to the **complexities** involved in **developing and delivering** new therapies.

The rare pediatric disease PRV program has led to new innovations that **benefit over 200,000 rare disease patients** and address unmet medical needs across **50+ rare pediatric indications.**

More than 90 percent of all priority review vouchers were awarded to therapies for indications with no approved therapy on the market.

The rare pediatric disease PRV program is a **proven tool** for spurring research & development (R&D) investment for rare pediatric diseases and can help attract critical funding for **small, emerging biopharmaceutical companies.**

Key Findings

1. There are **over 200** sunset-impacted assets at risk of not receiving a PRV unless the program is reinstated.

85% of surveyed biotech executives reported PRV to be a significant factor in the decision to pursue the development of a RPD assets.

2. **Nearly 40** of those sunset-impacted therapies are likely to come to market.

Since the PRV program lapsed, **35%** of respondents have cancelled or delayed programs in their pipeline.

3. There is **over $4 billion** in lost reinvestment capacity if the program is not reinstated.

50% of surveyed biotech executives expect further difficulties in accessing capital after the program lapse.

Unmet Need & Challenges in Rare Pediatric Disease Drug Development

Rare diseases present a global health crisis and have an outsized impact in pediatric populations. Approximately 10% of the US population is affected by one of the over 10,000 rare diseases,[1] and only 5% of those rare diseases have a Food and Drug Administration (FDA)-approved treatment.[2] Half of all patients diagnosed with a rare disease are children, and 70% of rare genetic disorders manifest during childhood.[3] Three out of ten children with a rare disease will not survive beyond the age of five.[4] There is tremendous unmet need and at the same time, rare pediatric disease drug development is extraordinarily challenging. These challenges include small patient populations, complex and variable diseases with limited natural history, slow disease progression with often irreversible symptoms, and a lack of defined endpoints and biomarkers.

The impact of rare diseases extends beyond health implications. Rare disease patients incur substantially higher medical costs compared to those with non-rare conditions, placing a heavy financial strain on affected people, their families and health care systems.[6] However, the availability of treatments mitigates the economic impact. Recent studies reveal that the per-patient-per-year (PPPY) costs associated with rare diseases far exceed those of high-prevalence diseases (e.g., diabetes, cardiovascular disease).[7] Recent studies have shown that in the United States alone, the costs related to 373 rare diseases range from $1 trillion dollars to $2.2 trillion annually, and the societal responsibility for all known rare diseases may reach an astonishing $7.2 trillion to $8.6 trillion dollars per year. [8, 9] Regulatory and commercial hurdles add further uncertainty to rare disease drug development.

1 in 10 Americans live with a rare disease

10,000 known rare diseases. 95% have no approved treatment

1 in 2 patients diagnosed with a rare disease are children

30% of children with a rare disease don't see their 5th birthday

Regulators grapple with disease heterogeneity, limited natural history data, and a lack of regulatory precedent. Longer development timelines (orphan drug development can take up to 15.1 years [10, 11]) and higher costs (clinical development of orphan drugs can cost up to $291 million dollars[12]) exacerbate the challenges. Commercially, small market sizes pose high risks for biopharmaceutical companies and investors. These challenges are further compounded when working with pediatric populations.

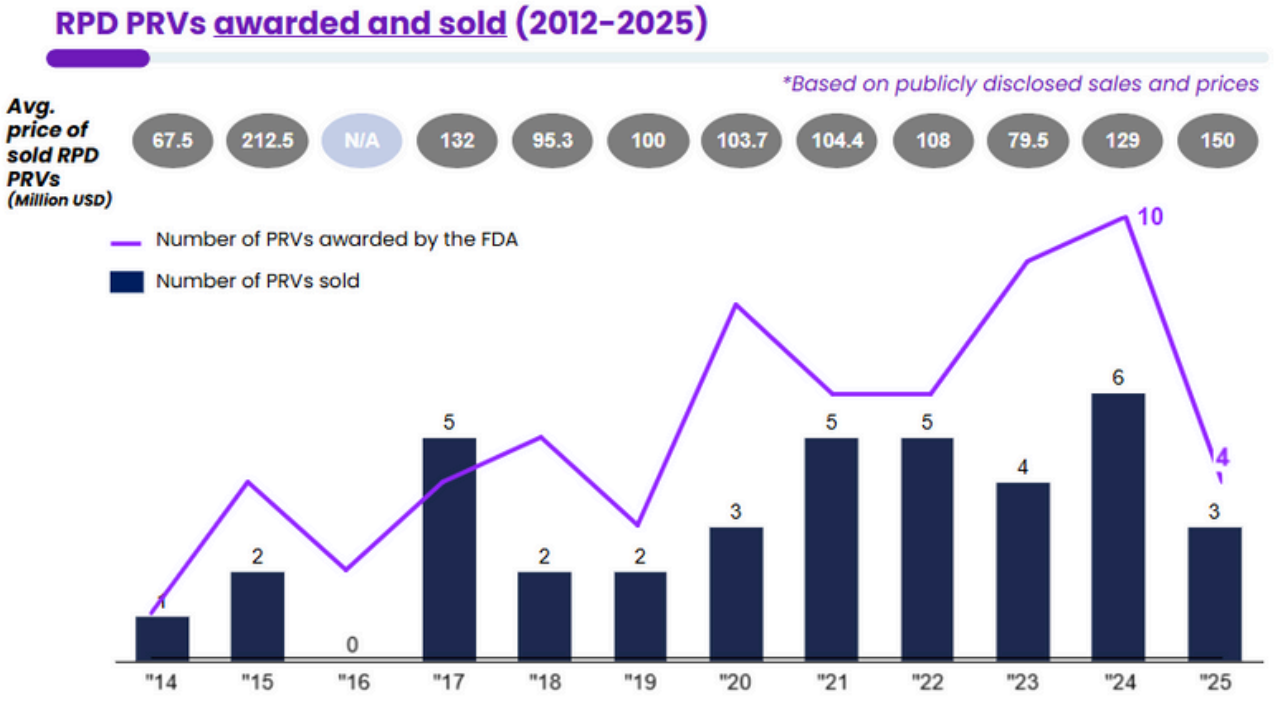
Given these substantial obstacles, it is critical to encourage rare disease drug development. Landmark policies like the Orphan Drug Act of 1983 have been incredibly successful in transforming the rare disease ecosystem and spurring innovation. However, recent trends indicate that the United States' robust incentive ecosystem is in jeopardy. It is imperative to preserve key incentives like the rare pediatric disease PRV program to encourage innovation and provide treatment options for people living with a rare disease.

History of the rare pediatric disease priority review voucher program and its utilization

The rare pediatric disease PRV program was created in 2012 and subsequently reauthorized with bipartisan support in 2016 and 2020. The program encourages drug development for rare pediatric diseases by expediting the regulatory process for qualifying products.

Companies can obtain a PRV by initiating a development program for a rare pediatric disease and submitting a PRV request to the FDA during New Drug Application (NDA) or Biologics License Application (BLA) submission. If a sponsor's drug or biologic receives approval for a rare pediatric disease and the PRV request is granted, a PRV is awarded. Sponsors can redeem vouchers for priority review (reducing the review time from 10 months to 6 months) of a future NDA or BLA submitted to the FDA, or transfer or sell to another biopharmaceutical company to expedite review of another drug or biologic.

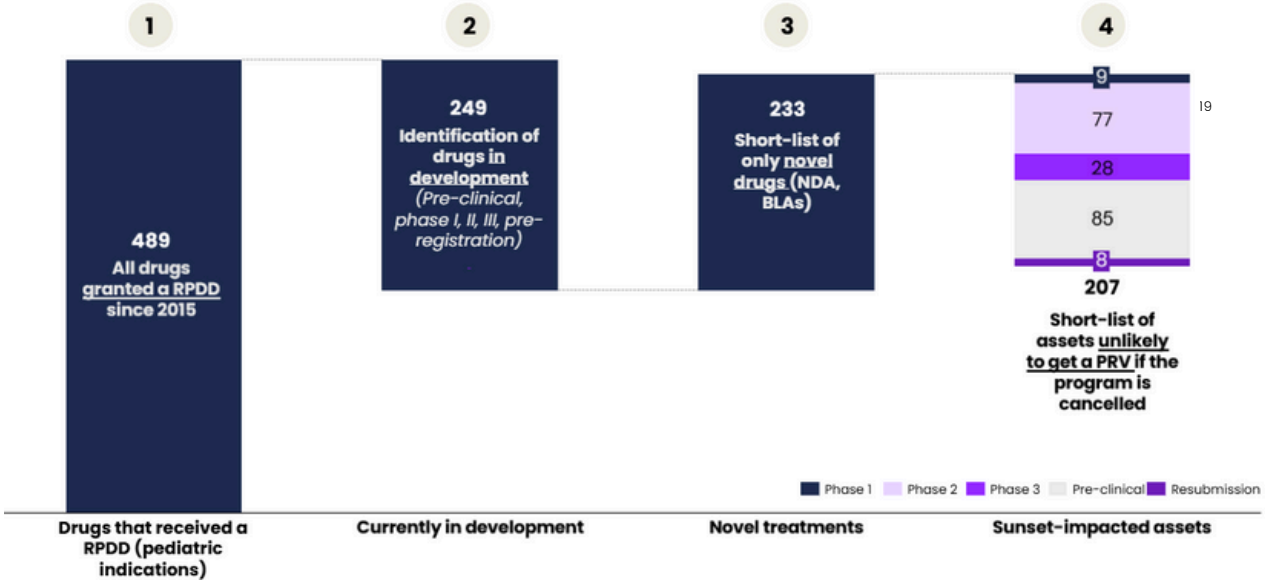
While the program's primary goal remains incentivizing the development of treatments for rare pediatric diseases, the program also speeds the approval of additional therapies, which could be indicated for higher-prevalence diseases, granting more patients quicker access to innovative treatments.[13]



As of December 2025, up to 63 RPD PRVs have been awarded across 47 rare pediatric indications. PRV grants were concentrated in 2024, likely due to the designation deadline in December 2024. 2024 saw the highest number of PRVs sold, as sponsors and buyers rushed to capitalize before the program ended.

Quantitative Impact of the RPD PRV Program Lapse

We identified drugs in development for rare pediatric diseases that are at risk of not receiving a PRV, and then quantified the impact of this lost opportunity. We defined asset profiles by reviewing investigational drugs for rare pediatric diseases that are at risk of not receiving a PRV. To identify drugs which are at risk of not getting a PRV, we short-listed those novel drugs in development for an RPD.



There are ~ 207 drugs in development for a RPD, which are at risk of not receiving a PRV unless the program is reinstated. Of the 207 drugs at risk, 38 are likely to reach the market, based on benchmark probabilities of success by development stage. We split the set of drugs in development that are at risk of not receiving a PRV by development stage and applied benchmark probabilities of success (PoS) based on these drugs' development stage.

38
Number of drugs at risk that are likely to eventually make it to market

×

$105M
Average price of a PRV assuming that the program gets reinstated

=

$4B
Final 'lost' reinvestment capacity due to the PRV lapse

The true value loss from the PRV program lapse is even greater, as PRV proceeds are typically reinvested into additional R&D programs that might not otherwise receive funding. This reinvestment fuels the development of future therapies. Without it, fewer drugs will reach the market, fewer patients will benefit from innovative treatments, and companies will miss out on potential sales from these new products.

Qualitative Impact of the RPD PRV Program Lapse

Interviews were conducted with 8 experts impacted or affected by the program lapse, including biotech executives & investors. The goal of conducting expert interviews was to gather case studies and directional insights on the real-world impact of the PRV lapse. Additionally, a survey was sent to RPD-focused biotechnology companies to understand the impact of the program lapse on pipeline, financial planning & capital access for the industry. The goal of the survey is to better understand the program lapse impact on pipeline, financial planning and capital access for biotech companies.

Impact on Patients & Innovation: The program lapse is already stifling innovation & reducing the availability of new therapies for patients with highest unmet needs.

Impact on Patients

- The lapse of the PRV program has resulted in the delay or discontinuation of multiple rare disease drug development programs, reducing the likelihood that new therapies will reach patients with high unmet needs.
- Patients with rare conditions are particularly affected, as programs targeting these diseases often lack alternative funding sources and are at risk of being permanently shelved.
- PRV incentives are critical for providing hope and therapies to these patients, who often face long diagnostic journeys and have no treatment options.

Impact on Innovation

- Advances in rare diseases often originate in small biotech companies. The PRV program helps bridge the gap to commercialization for these high-risk, low-commercial-return indications.
- The program also serves as a non-dilutive funding mechanism, enabling small and mid-sized biotech companies to pursue rare disease drug development that would otherwise be economically unviable.
- With the lapse of the program, financial risk and uncertainty have increased, resulting in the de-prioritization of early-stage rare disease assets within company pipelines.

"If the PRV program ceases to exist, it's going to mean fewer medicines for those who are most vulnerable."
- Mid-size biotech executive

"Patients with the rarest conditions will lose access to potential treatments."
- Founder of small biotech focused on rare pediatric drugs

"Programs for diseases with tiny patient populations (sometimes as little as 15 known patients) are not financially viable and will be abandoned without PRV support."
- Founder of small biotech focused on rare pediatric drugs

"Dozens of programs for rare diseases will not move forward unless PRV is renewed."
-Small single-asset biotech executive

Impact on pipeline decisions : 85% of surveyed biotech executives reported PRV to be a significant factor in the decision to pursue the development of a RPD assets

> **The lapse of the PRV program means that many rare disease programs, often the only hope for children and families, will be discontinued or never started.**
> *- Investor in the rare disease biotech space*

Relevance of PRV in Pipeline Decisions

71% rated the PRV as highly significant in the decision to pursue development of an RPD asset.

46% of cases cited the loss of the PRV financial incentive as an influence on pipeline decisions.

Impact of the Program Lapse on Pipeline Decisions

35% of respondents have cancelled or delayed programs in their pipeline since the program lapsed.

14% have been forced to accelerate programs to meet the program sunset deadline.

> **All of our funding went away in December 2024 with the lapse of the PRV program. We were about to go bankrupt as a company.**
> -Small biotech company executive

"Before the lapse, many investors explicitly cited the potential PRV as a key part of the value proposition and were more willing to fund a single asset, late-stage pediatric rare disease company."
- Mid-size biotech executive

"We slowed down a program, delayed progress on another one and cancelled and returned several programs for rare diseases"
- Small, rare disease-focused biotech executive

Impact on Valuation & Financing: The PRV program lapse can especially impact smaller biotech companies, where most innovation occurs, leading to lower valuations.

Impact on company valuation

Smaller and early-stage companies

- The lapse of the PRV program has **significantly reduced** valuations for small & early-stage biotech by removing a key source of non-dilutive capital, making them less attractive to investors
- Investors view these companies as **higher risk**, leading to lower valuation multiples
- For smaller companies that are highly dependent on PRVs, the program lapse **threatens their viability** and may lead to discontinuation of clinical development or company closure

Larger, diversified, later-stage companies

- The valuation impact on larger, diversified companies **is less significant due to broader pipelines & alternative capital sources**
- However, the loss of PRV incentives can **still influence the prioritization of certain rare programs** within their portfolios.

Impact on ability to secure capital

Smaller and early-stage companies

- These companies face **greater challenges in raising capital,** as investors are less willing to provide equity financing without the liquidity and risk mitigation offered by PRVs
- Many of these companies must rely on charitable or foundation-based funding, which is **typically insufficient for sustained R&D**

Larger, diversified, later-stage companies

- Larger companies can be **more resilient** to the lapse as their broader portfolio & pipeline mix reduce their dependence on the PRV
- These companies may hold later-stage assets, so their **overall risk profile is less affected by the lapse** as investors remain focused on these programs and near-term PRV opportunities
- PRV remains the **only option to secure funding** without giving up equity or diluting ownership

"Impact on valuation is pronounced for single asset companies that depend on the PRV. For other companies we don't see as much valuation swing."
- Healthcare investor with experience in rare disease financing

"From investments we were considering, a lot came off the table if we cannot contemplate the PRV as a return mechanism."
- Healthcare investor with experience in rare disease financing

"For earlier stage companies, the PRV is the only thing that's going to get them there"
- Mid-size biotech executive

"All of the programs in our pipeline, they're not getting funded at all because there's just no PRV around."
- Small ultra-rare disease biotech founder

Impact on Valuation & Financing: ~50% of surveyed biotech executives expect further difficulties in accessing capital after the program lapse.

Perceived Financial Value of the PRV	**71%** of respondents attribute a financial value of $100-150M to the PRV.
	21% of respondents believe the PRV to have a value greater than $150M even before the program lapse.
Impact of the Program Lapse on Financial Planning	**23%** of the respondents expect a reduction in budget allocation for RDP assets because of the lapse
	23% anticipate that investments will be reprioritized and will focus on other sources of business
Impact of the Program Lapse on Ability to Access Capital	**46%** of respondents reported an increased difficulty in accessing capital since the program lapsed
	32% report that the lapse is making it **significantly** more difficult to access capital

"Single asset, late-stage RPD focused-companies that are no longer eligible or less likely to earn a PRV have suffered a $75–$150M reduction in value."
- Medium size multi-asset biotech executive

""Financing terms are now materially more onerous with higher implied cost of capital, deeper discounts, and more heavily structured or dilutive proposals"
- Small single-asset biotech executive

"As a single asset company… raising capital has become extremely challenging, to the point that we may be unable to complete our Phase 3 trial without highly unfavorable financing"
-Small single-asset biotech executive

The Rare Disease Company Coalition urges Congress to:

1. Pass the Mikaela Naylon Give Kids a Chance Act to restore the PRV program through 2029.
2. Pass legislation to permanently reauthorize the PRV program before the next sunset period.

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Rare Disease Company Coalition

We are dedicated to being a reliable and trusted resource for our leaders in Congress and the Administration, and a productive partner to rare disease-minded industry, academic and patient groups, by educating on the issues and opportunities that affect rare disease companies and advancing our shared mission to improve the lives of people living with rare diseases.

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