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Congress Extends Rare Pediatric Disease Priority Review Voucher Program Through 2029

On February 3, Congress enacted the Consolidated Appropriations Act, 2026 (CAA). Among its health policy provisions, Section 6604 extends the Rare Pediatric Disease Priority Review Voucher (PRV) program through September 30, 2029, preserving a regulatory incentive to encourage development of therapies for children with rare diseases.

The CAA also includes other provisions relevant to the pharmaceutical industry that we have previously written on. Section 6605 restores FDA's longtime interpretation of indication-specific orphan drug exclusivity, reversing the impact of the *Catalyst Pharm., Inc. v. Becerra* decision (see here). In addition, Section 6703 introduces new formulation-transparency requirements that give abbreviated new drug application applicants greater certainty about inactive-ingredient sameness (see here).

Background and Purpose of the Rare Pediatric Disease PRV Program

Congress created the rare pediatric disease PRV program in 2012 as part of the FDA Safety and Innovation Act to encourage the development of treatments for serious or life-threatening diseases affecting small pediatric populations.

Under the program, a sponsor that obtains FDA approval of a drug or biologic for a qualifying rare pediatric disease may receive a transferable voucher entitling the holder to priority review of a future marketing application. Section 529(a)(3) of the Federal Food, Drug, and Cosmetic Act (FD&C Act) defines a "rare pediatric disease" as a serious or life-threatening condition that primarily affects individuals from birth through 18 and qualifies as a "rare disease or condition." That term is defined in Section 526 of the FD&C Act as one affecting fewer than 200,000 people in the U.S., or

if more prevalent, one for which there is no reasonable expectation that development costs will be recovered through U.S. sales. Priority review shortens FDA's review timeline from the standard 10 months to about six months. Because faster review can significantly accelerate time to market and translate to earlier sales, vouchers have substantial economic value and have sold for more than $100 million in private transactions.

Sponsors may seek rare pediatric disease designation during development to help establish that a drug or biologic is intended to treat or prevent a qualifying condition. Although designation is not required to receive a voucher, FDA encourages sponsors to request it before submitting a marketing application, and applicants must show that the drug or biologic meets the statutory eligibility criteria.

The program was designed to address a persistent market failure.

Many rare pediatric diseases affect only a few thousand patients worldwide, making traditional return-on-investment models difficult to justify. By attaching a transferable asset to approval of a pediatric therapy, Congress sought to encourage companies to pursue research that might otherwise be financially infeasible. Conditions such as spinal muscular atrophy, sickle cell disease, Batten disease, and autosomal recessive polycystic kidney disease illustrate the types of unmet needs the program aims to address.

Impact of the Program to Date

Since its creation, the Rare Pediatric Disease PRV program has played a meaningful role in pediatric drug development.

As of late 2025, FDA had awarded more than 60 vouchers covering dozens of rare pediatric diseases, many of which previously lacked any FDA-approved therapies. Industry observers credit the program with accelerating development timelines, attracting investment into small-patient-population research, and generating financing through secondary voucher sales. Faster review can bring products to market sooner, improving commercial viability, while the ability to sell a voucher provides an important source of capital for emerging biotechnology companies.

Section 6604: Extension and Technical Clarifications

Section 6604 of the CAA makes two principal statutory changes.

First, it replaces the prior sunset provision with a new deadline of September 30, 2029. FDA may continue to award vouchers for qualifying approvals until that date. The statute does not impose a separate deadline for obtaining rare pediatric disease designation, which preserves flexibility for sponsors whose development timelines extend several years.

Second, the amendment clarifies that the priority review voucher user fee is due upon submission of the marketing application that redeems the voucher, while other applicable user fees continue to follow standard FDA timing.

GAO Study to Evaluate the Program's Effectiveness

In addition to extending the program, Congress directed the Government Accountability Office (GAO) to conduct a comprehensive study of effectiveness of rare pediatric disease PRVs.

The GAO must examine, among other issues, the types of drugs or biologics that earned vouchers, whether those approvals addressed unmet medical needs, how vouchers have been transferred and used, and whether the program has influenced drug development decisions, investment in rare pediatric therapies, or FDA review priorities.

The GAO must report its findings to Congress within five years of enactment. The study's findings may help inform future legislative decisions about whether to extend, modify, or replace the program after the current authorization period.

Practical Takeaways for Sponsors

The CAA's extension restores predictability for companies developing therapies for rare pediatric diseases. Sponsors can incorporate the potential value of a priority review voucher into portfolio planning, licensing negotiations, and financing strategies. For companies currently in development, the 2029 sunset provides a clear window for evaluating whether their programs may qualify.

At the same time, the mandated GAO review means that the program will be examined in the coming years. We will continue to monitor the GAO study, as its findings may guide how Congress approaches the program's future.

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