CAPITOL STREET

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Rare Disease Voucher Update

PRV Program Ends Sept. 30, Reauthorization May Slip (Senate Holdup)

Relevant Companies

Our Take & Next Up

The rare pediatric disease priority voucher program is set to expire on September 30, and we think reauthorization may slip and could take place at the end of the year. The program has been reauthorized since 2012 and has not lapsed. We see reauthorization by September 30 or before the end of the year. Reauthorization bills have been introduced in both chambers and made progress largely in the House. However, Senate passage is being slowed by lawmaker (e.g., Bernie Sanders, VT) concerns that the voucher program does not actually incentivize rare disease innovation as vouchers are often bought and can be used for non-rare therapeutics. The authority to award rare pediatric disease designations expires September 30, but the FDA's authority to grant priority vouchers does not end until September 2026.

Key Points

Priority review vouchers (PRVs) began as an incentive to support the development of drugs for neglected tropical diseases that was expanded in 2012 to include rare pediatric diseases (RPD PRVs). Companies upon approval for a rare pediatric disease therapeutic can be awarded an RPD PRV, which allows for priority review for products that would otherwise not qualify. The voucher is transferable and can be redeemed to receive priority review for a different product. The company may also sell the voucher to another sponsor.

The program is set to end after September 30 without reauthorization. The program was last reauthorized in 2020 and in 2016. There are two dates to keep in mind. If the FDA cannot grant a rare pediatric disease designation by September 30, 2024, that drug cannot be eligible for a voucher as the FDA will no longer be allowed to initiate the process. For drugs that already have a rare pediatric disease designation, the FDA will not be allowed to award any rare pediatric disease priority review vouchers upon approval after September 30, 2026.

The disease indication with the most RPD PRVs awarded is duchenne muscular dystrophy (Sarepta, ITF therapeutics, NS Pharma, Marathon Pharma, among others). Other common indications for RPD PRVs include neuroblastoma (YMAB, AZN), spinal muscular atrophy (Genentech, BIIB, NVS) and sickle cell disease

(VRTX & CRSP, PFE). The program is critically important for small biotechs with a significant number of PRVs awarded to smaller companies in the rare disease space.

Reauthorization (to 2030) passed out of the House E&C Health Subcommittee (Chair Guthrie, R-KY) in May; Senate reauthorization was introduced in June but it has stalled in the upper chamber. Both versions aim to extend the program to September 30, 2030. House E&C (Chair McMorris Rodgers, R-WA) committee vote was scheduled for June 27 but cancelled. The *Creating Hope Reauthorization Act i*s being led by Reps. Mike McCaul (R-TX), Anna Eshoo (D-CA), Gus Bilirakis (R-FL), Nanette Barragán (D-CA), Lori Trahan (D-MA), and Michael Burgess (R-TX) in the House. In the Senate, lead co-sponsors are Senators Bob Casey (D-PA), Chairman of the HELP Subcommittee on Children and Families, Markwayne Mullin (R-OK), Sherrod Brown (D-OH) and Susan Collins (R-ME).

We believe that the PRV program will be reauthorized as costs are minimal & lawmakers praise the program as having no cost to taxpayers. In 2020, CBO estimated that a 4-year reauthorization would cost just $12 M over a 5-year period for additional staffing.

Skepticism over whether the program actually incentivizes new rare disease research may impact reauthorization in the Senate. In the past, Sen. Sanders (I-VT) expressed concerns with the way the vouchers are used to reward companies that already have the resources to develop rare disease drugs. A GAO report in 2020 reported that the available research on PRV programs found they had little or no effect on drug development. However, the same report noted that all 7 drug sponsors (BMRN, JNJ, Medicines Development for Global Health, NVS, Sanofi, SIGA, and RARE) interviewed said that PRVs were a factor in drug development decisions.

PRVs are often bought by companies to accelerate their review times. Voucher holders can sell the PRV an unlimited number of times (but must notify FDA each time). Sponsors can redeem the PRV for any product, including rare and non-rare diseases. According to NORD, 29 of the 53 awarded RPD PRVs have been sold, and others have transferred ownership as part of company mergers or acquisitions. The same research by National Organization for Rare Disorders (NORD) found that 23 RPD PRVs have been redeemed for priority review of 21 different drugs, just 6 were redeemed for drugs that treat rare diseases. Products that used a RPD PRV include (but are not limited to):

- Cosentyx (NVS)
- Descovy (GILD)
- Imjudo (AZN)
- Ultomiris (AZN)
- Rinvoq (ABBV)
- Skyrizi (ABBV)
- Tyvaso DPI (UTHR)
- Vabysmo (Genentech)
- Vyvgart Hytrulo (Argenx).

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