The Creating Hope Reauthorization Act

U.S. Senators Bob Casey and Markwayne Mullin

Rare diseases, such as pediatric cancers, affect significant numbers of Americans. An estimated one in ten people in the United States, half of whom are children, have a rare disease according to the National Organization for Rare Disorders. Only one in 20 of these rare diseases have a Food and Drug Administration (FDA)-approved treatment. Despite this significant unmet medical need, private companies seldom pursue new therapies for rare diseases because it requires making an investment in treatments that will likely not recoup the high costs associated with their research, development, marketing, and distribution. Developing products for children is particularly challenging because of the difficulties associated with conducting clinical trials in this population.

Senator Casey and Senator Mullin introduced the *Creating Hope Reauthorization Act* to extend the current FDA pediatric rare disease priority review voucher program. The legislation extends the program through September 30, 2030, a longer period of time than previous reauthorizations. This change will provide greater stability to innovators, encourage investment from key biomedical stakeholders, and spur innovation in rare and neglected diseases that disproportionately impact children.

Additional Background

In 2012, the Food and Drug Administration Safety and Innovation Act (FDASIA) created the Rare Pediatric Disease (RPD) Priority Review Voucher (PRV) program. This program established an incentive for pharmaceutical companies to develop novel treatments for rare pediatric diseases by providing them with a priority review voucher that entitles the company to receive a "priority" 6-month review of another new drug application that would otherwise be reviewed under FDA's standard 10-month review clock. Shaving off four months or more in review time can lead to earlier market entry. And since the vouchers can be sold, they are worth millions of dollars to companies seeking faster market entry for their new drugs. Vouchers have sold for as much as $395 million.

The RPD PRV program has been reauthorized with bipartisan support twice since its inception, in 2016 and 2020. Since 2012, this program has awarded 53 vouchers for 39 rare pediatric diseases that have led to innovations benefitting over 200,000 patients. Thirty-six of the rare pediatric diseases had no previously approved therapies on the market at the time of approval. Given that this program is set to expire on September 30, 2024, Casey and Mullin are working to have the program extended until September 30, 2030.