



2025

# ADVOCACY

## Rare Pediatric Disease Priority Review Voucher

Request your support to reauthorize the Priority Review Vouchers (PRV). The purpose of PRV's is to expedite the development and promotion of drugs in the rare pediatric diseases. Developing drugs for rare pediatric diseases is particularly challenging due to the small populations affected, difficulties associated with conducting clinical trials for children, delays in diagnosis, and more. A majority of PRVs have gone to rare pediatric disease drugs, meaning the program has a large impact on the rare disease community.

It is vital to reauthorize the Priority Review Voucher program, which provides incentives to pharmaceutical companies for developing treatment for pediatric rare diseases. Companies can either use the voucher (or incentive) to accelerate the approval of another drug they are developing, or they can sell it to another pharmaceutical company.

## Accelerating Kids' Access to Care Act

Currently, if a patient needs to see a provider outside of their state the provider must become a part of the patient's state's Medicaid program, which is a long-time consuming process. This bill would make a new, faster process for doctors to enroll in another state's Medicaid program by:

- Establishing a federal streamlined screening and enrollment pathway
  - Voluntary, meaning providers are not required to do this
  - If providers opt to be screened through this pathway, they will be eligible to enroll in other state's Medicaid programs for a 5-year period, assuming they remain in good standing
- Eligible providers must present the most limited risk of fraud and abuse, be enrolled in Medicare or their home state Medicaid program, and not be excluded from participating in any federal health programs

## Rare Disease Congressional Caucus

Request your support by joining the bipartisan and bicameral Rare Disease Congressional Caucus to promote awareness of rare disease issues. One in ten Americans with a rare disease diagnosis need your support to overcome barriers.

## DeafBlind Congressional Caucus

Request your support by joining the NEW DeafBlind Congressional Caucus to highlight and amplify the priorities of the deafblind community. Only 4 more members are needed to officially be established.

## Upcoming Bills Being Introduced

### 1. The Helen Keller Education Act

### 2. The Cogswell-Macy Act

Representative Morgan McGarvey (KY) plans to introduce the above bills in March 2025. Both bills would provide what is needed for individuals with deafblindness to access their education.