Pediatric
Brain Tumor
FOUNDATION

Data for Pediatric Brain Cancer Act

Introduced by Representatives Ami Bera, M.D. (D-CA-06) and Mike Kelly (R-PA-16)

Atypical teratoid/rhabdoid tumors (AT/RT) are a rare and aggressive form of cancer that affect the brain and spinal cord of very young children. Despite intense therapy, only about 50 percent of children with AT/RT will survive. Those who do will have serious long-term side effects from these treatments. New treatment options are desperately needed; however, the gold-standard for randomized clinical trials is challenging in rare populations due to the limited patient pool and geographical spread, among other factors.

The Data for Pediatric Brain Cancer Act (H.R. 6288) would establish a first-of-its-kind external control cohort for clinical trial design. External control cohorts can be used to conduct non-randomized trials by comparing the experimental treatment under investigation against data collected from previous patient populations, thereby allowing all patients enrolled in the trial to access these new treatments and limiting the number of patients receiving the current standard of care. Additionally, because more patients are receiving the experimental therapy, trial timelines are shortened as fewer patients are required to enroll in the study. The major barrier to the use of external control cohorts is a lack of the sufficiently detailed patient databases necessary for use in clinical trials. While the legislation focuses on AT/RT, this pilot program aims to demonstrate how trials using external control cohorts will greatly benefit research into other rare diseases.

The Data for Pediatric Brain Cancer Act would help identify potential uses of real-world data registries as external control cohorts for pediatric brain tumor clinical trial design, by:

- Developing and maintaining a registry of real-world data for children with AT/RT
- Considering new and innovative approaches and technology for data collection, integration, and analysis
- Continuing and expanding activities, which may include existing data collection activities, to establish real-world database infrastructure
- Providing support for data integration, bioinformatics, and statistical analysis

To learn more about how you can support this important legislation, please email Mike Henry, the Pediatric Brain Tumor Foundation's Director of Advocacy, at mhenry@curethekids.org.