AUTHENTICATED
U.S. GOVERNMENT
INFORMATION
GPO

I

118TH CONGRESS
1ST SESSION

H. R. 6288

To amend the Federal Food, Drug, and Cosmetic Act to provide for a Pediatric Brain Tumor Real-World Data Registry Program, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

NOVEMBER 8, 2023

Mr. BERA (for himself and Mr. KELLY of Pennsylvania) introduced the following bill; which was referred to the Committee on Energy and Commerce

---

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to provide for a Pediatric Brain Tumor Real-World Data Registry Program, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Data for Pediatric Brain Cancer Act of 2023''.

SEC. 2. FINDINGS.

Congress finds the following:

(1) Randomized clinical trials require the use of control groups to serve as the baseline for determining the effectiveness of a study treatment.

(2) Because of the rarity of some diseases, it is often hard to find enough study participants in a sufficient amount of time, making randomized clinical trials for these treatments infeasible. Couple this with the fact that the current standard of care for many rare diseases is ineffective (often leading to death), making administering the current standard of care unethical.

(3) In these cases, data reflecting patient treatment in routine clinical practice can be used to develop external control groups for single-arm trials. Clinical trials using external control cohorts can provide valuable benchmark results on potential comparator treatment efficacy.

(4) The Food and Drug Administration (in this section referred to as the ''FDA'') has distributed draft guidance on this issue commenting on considerations for the use of real-world data for the design and conduct of externally controlled trials.

(5) Through collaboration with community advocates and industry partners with input from the FDA, researchers focused on atypical teratoid

rhabdoid tumor (in this section referred to as ''ATRT''), an ultra-rare and poor prognosis pediatric brain tumor, have taken the lead in the design of real-world data sets.

(6) Because of the advancements in the research, ATRT has presented itself as the ideal candidate to pilot the development of real-world data sets for use in external control cohorts.

SEC. 3. PEDIATRIC BRAIN TUMOR REAL-WORLD DATA REGISTRY PROGRAM.

Chapter X of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 391 et seq.) is amended by adding at the end the following:

''SEC. 1015. PEDIATRIC BRAIN TUMOR REAL-WORLD DATA REGISTRY PROGRAM.

''(a) IN GENERAL.—The Secretary shall carry out a program, to be known as the Pediatric Brain Tumor Real-World Data Registry Program, to strengthen and expand activities related to the collection, sharing, and use of real-world data for children with brain tumors.

''(b) REQUIREMENTS.—In carrying out the program under this section, the Secretary shall—

''(1) develop and maintain (or support the development and maintenance of) a registry of real-

world data for children with atypical teratoid rhabdoid tumors;

''(2) consider new and innovative approaches and technology for data collection, integration, and analysis;

''(3) continue and expand activities, which may include existing data collection activities, to establish real-world database infrastructure;

''(4) provide support for data integration, bioinformatics, and statistical analyses; and

''(5) identify potential uses of real-world data registries as external control cohorts for pediatric brain tumor clinical trial design.

''(c) COLLABORATION AND CONSULTATION.—In carrying out the program under this section, the Secretary shall collaborate and consult, as appropriate, with public and private entities, including relevant departments and agencies, academic institutions, and industry.

''(d) GRANTS, CONTRACTS, AND COOPERATIVE AGREEMENTS.—

''(1) AWARDS.—In carrying out the program under this section, the Secretary shall award grants, contracts, and cooperative agreements to academic institutions and other entities with relevant expertise

in pediatric neuro-oncology or the collection, integration, and analysis of real-word data.

"(2) APPLICATION.—

"(A) IN GENERAL.—To seek an award under paragraph (1), an entity described in such paragraph shall submit to the Secretary an application at such time, in such manner, and containing such information as the Secretary may require.

"(B) CONTENTS.—An application under subparagraph (A) shall include a description of how the applicant will partner, as applicable, with academic institutions or a consortium of academic institutions that have relevant expertise, such as expertise in pediatric neuro-oncology, clinical research, or the application of bioinformatics or statistics.

"(e) AUTHORIZATION OF APPROPRIATIONS.—For carrying out this section, there are authorized to be appropriated $2,000,000 for fiscal year 2025.".

○