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(Original Signature of Member)

118TH CONGRESS
1ST SESSION

H. R. _____

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

Mr. BERA introduced the following bill; which was referred to the Committee
on _____

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.

1   *Be it enacted by the Senate and House of Representa-*

2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4   This Act may be cited as the "Data for Pediatric

5 Brain Cancer Act of 2023".

6 **SEC. 2. FINDINGS.**

7   Congress finds the following:

1   (1) Randomized clinical trials require the use of
2 control groups to serve as the baseline for deter-
3 mining the effectiveness of a study treatment.

4   (2) Because of the rarity of some diseases, it is
5 often hard to find enough study participants in a
6 sufficient amount of time, making randomized clin-
7 ical trials for these treatments infeasible. Couple this
8 with the fact that the current standard of care for
9 many rare diseases is ineffective (often leading to
10 death), making administering the current standard
11 of care unethical.

12   (3) In these cases, data reflecting patient treat-
13 ment in routine clinical practice can be used to de-
14 velop external control groups for single-arm trials.
15 Clinical trials using external control cohorts can pro-
16 vide valuable benchmark results on potential com-
17 parator treatment efficacy.

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20 draft guidance on this issue commenting on consid-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

23   (5) Through collaboration with community ad-
24 vocates and industry partners with input from the
25 FDA, researchers focused on atypical teratoid

1 rhabdoid tumor (in this section referred to as

2 "ATRT"), an ultra-rare and poor prognosis pedi-

3 atric brain tumor, have taken the lead in the design

4 of real-world data sets.

5 (6) Because of the advancements in the re-

6 search, ATRT has presented itself as the ideal can-

7 didate to pilot the development of real-world data

8 sets for use in external control cohorts.

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

11 Chapter X of the Federal Food, Drug, and Cosmetic

12 Act (21 U.S.C. 391 et seq.) is amended by adding at the

13 end the following:

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

16 "(a) IN GENERAL.—The Secretary shall carry out a

17 program, to be known as the Pediatric Brain Tumor Real-

18 World Data Registry Program, to strengthen and expand

19 activities related to the collection, sharing, and use of real-

20 world data for children with brain tumors.

21 "(b) REQUIREMENTS.—In carrying out the program

22 under this section, the Secretary shall—

23 "(1) develop and maintain (or support the de-

24 velopment and maintenance of) a registry of real-

4

1 world data for children with atypical teratoid

2 rhabdoid tumors;

3 "(2) consider new and innovative approaches

4 and technology for data collection, integration, and

5 analysis;

6 "(3) continue and expand activities, which may

7 include existing data collection activities, to establish

8 real-world database infrastructure;

9 "(4) provide support for data integration,

10 bioinformatics, and statistical analyses; and

11 "(5) identify potential uses of real-world data

12 registries as external control cohorts for pediatric

13 brain tumor clinical trial design.

14 "(c) COLLABORATION AND CONSULTATION.—In car-

15 rying out the program under this section, the Secretary

16 shall collaborate and consult, as appropriate, with public

17 and private entities, including relevant departments and

18 agencies, academic institutions, and industry.

19 "(d) GRANTS, CONTRACTS, AND COOPERATIVE

20 AGREEMENTS.—

21 "(1) AWARDS.—In carrying out the program

22 under this section, the Secretary shall award grants,

23 contracts, and cooperative agreements to academic

24 institutions and other entities with relevant expertise

1 in pediatric neuro-oncology or the collection, integra-

2 tion, and analysis of real-word data.

3   "(2) APPLICATION.—

4   "(A) IN GENERAL.—To seek an award

5 under paragraph (1), an entity described in

6 such paragraph shall submit to the Secretary

7 an application at such time, in such manner,

8 and containing such information as the Sec-

9 retary may require.

10   "(B) CONTENTS.—An application under

11 subparagraph (A) shall include a description of

12 how the applicant will partner, as applicable,

13 with academic institutions or a consortium of

14 academic institutions that have relevant exper-

15 tise, such as expertise in pediatric neuro-oncol-

16 ogy, clinical research, or the application of

17 bioinformatics or statistics.

18   "(e) AUTHORIZATION OF APPROPRIATIONS.—For

19 carrying out this section, there are authorized to be appro-

20 priated $2,000,000 for fiscal year 2025.".