

115TH CONGRESS
1ST SESSION

S. 456

To amend the Federal Food, Drug, and Cosmetic Act to establish a program to increase the development of new drugs to treat pediatric cancers, and for other purposes.

IN THE SENATE OF THE UNITED STATES

FEBRUARY 27, 2017

Mr. BENNET (for himself, Mr. RUBIO, Mr. VAN HOLLEN, and Mr. GARDNER) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to establish a program to increase the development of new drugs to treat pediatric cancers, 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 “Research to Accelerate
5 Cures and Equity for Children Act” or the “RACE for
6 Children Act”.

1 **SEC. 2. DRUG DEVELOPMENT FOR PEDIATRIC CANCER.**

2 (a) MOLECULAR TARGETS REGARDING CANCER
3 DRUGS.—Section 505B of the Federal Food, Drug, and
4 Cosmetic Act (21 U.S.C. 355c) is amended—

5 (1) in subsection (a)(2)(A)(i) by striking “prod-
6 uct for the claimed indications in all relevant pedi-
7 atric subpopulations; and” and inserting “product in
8 all relevant pediatric subpopulations—

9 “(I) for the claimed indications;
10 or”;

11 “(II) for a pediatric cancer indi-
12 cation, if the drug is intended for the
13 treatment of an adult cancer and is
14 directed at a molecular target consid-
15 ered to be germane to the growth and
16 progression of such pediatric cancer;
17 and”;

18 (2) in subsection (b)(1)—

19 (A) by amending subparagraph (A)(i) to
20 read as follows:

21 “(A)(i) the drug or biological product is
22 used for a substantial number of pediatric pa-
23 tients—

24 “(I) for the labeled indications; or

25 “(II) for a pediatric cancer indication,
26 if the drug is intended for the treatment of

1 an adult cancer and is directed at a molec-
2 ular target considered to be germane to
3 the growth and progression of such pedi-
4 atric cancer; and”; and

5 (B) by amending subparagraph (B) to read
6 as follows:

7 “(B) there is reason to believe that the
8 drug or biological product would represent a
9 meaningful therapeutic benefit over existing
10 therapies for pediatric patients—

11 “(i) for one or more of the claimed in-
12 dications; or

13 “(ii) for a pediatric cancer indication,
14 if the drug is intended for the treatment of
15 an adult cancer and is directed at a molec-
16 ular target considered to be germane to
17 the growth and progression of such pedi-
18 atric cancer; or”; and

19 (3) by amending paragraph (2) of subsection
20 (c) to read as follows:

21 “(2) the drug or biological product is in a class
22 of products, is for an indication, or is directed at a
23 specific molecular target in an adult cancer and such
24 molecular target is germane to the growth or pro-

gression of cancer in a pediatric cancer, for which there is need for additional options.”.

(b) EARLY MEETING ON PEDIATRIC STUDY PLAN.—

(1) IN GENERAL.—Clause (i) of section 505B(e)(2)(C) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(e)(2)(C)) is amended to read as follows:

“(i) shall meet with the applicant—

“(I) if requested by the applicant with respect to a drug that is intended to treat a serious or life-threatening disease or condition, to discuss preparation of the initial pediatric study plan, not later than the end-of-Phase 1 meeting (as such term is used in section 312.47(b) of title 21, Code of Federal Regulations, or successor regulations) or within 30 days of receipt of such request, whichever is later;

“(II) to discuss the initial pediatric study plan as soon as practicable, but not later than 90 calendar days after the receipt of such plan under subparagraph (A); and

1 “(III) to discuss any scientific or
 2 operational challenges that may be the
 3 basis of a deferral under subsection
 4 (a)(3) or a full or partial waiver under
 5 subsection (a)(4);”.

6 (2) CONFORMING CHANGES.—Section 505B(e)
 7 of the Federal Food, Drug, and Cosmetic Act (21
 8 U.S.C. 355c(e)) is amended—

9 (A) in the heading of paragraph (2), by
 10 striking “MEETING” and inserting “MEETINGS”;

11 (B) in the heading of paragraph (2)(C), by
 12 striking “MEETING” and inserting “MEET-
 13 INGS”;

14 (C) in clauses (ii) and (iii) of paragraph
 15 (2)(C), by striking “no meeting” each place it
 16 appears and inserting “no meeting under clause
 17 (i)(II)”; and

18 (D) in paragraph (3) by striking “meeting
 19 under paragraph (2)(C)(i)” and inserting
 20 “meeting under paragraph (2)(C)(i)(II)”.

21 (c) ORPHAN DRUGS.—Section 505B(k) of the Fed-
 22 eral Food, Drug, and Cosmetic Act (21 U.S.C. 355c(k))
 23 is amended by inserting “except in the case of a drug or
 24 biological product that is intended for the treatment of
 25 an adult cancer and is directed at a molecular target con-

1 sidered to be germane to the growth and progression of
2 a pediatric cancer,” after “regulation,”.

3 (d) GUIDANCE.—Not later than 1 year after the date
4 of enactment of this Act, the Secretary of Health and
5 Human Services, acting through the Commissioner of
6 Food and Drugs, shall issue guidance on the implementa-
7 tion of the amendments to section 505B of the Federal
8 Food, Drug, and Cosmetic Act (21 U.S.C. 355c) made by
9 this section, including—

10 (1) study designs;

11 (2) molecular targets considered to be germane
12 to the growth and progression present in one or
13 more cancers in pediatric populations that may be
14 appropriate for assessment under such section 505B,
15 as so amended; and

16 (3) considerations for implementation of such
17 section 505B, as so amended, and waivers of the re-
18 quirements of such section 505B with regard to mo-
19 lecular targets for which several drugs may be under
20 investigation.

21 (e) APPLICABILITY.—This section and the amend-
22 ments made by this section apply with respect to applica-
23 tions for a drug submitted under section 505 of the Fed-
24 eral Food, Drug, or Cosmetic Act (21 U.S.C. 355) or sec-
25 tion 351 of the Public Health Service Act (42 U.S.C. 262)

1 on or after the date that is 18 months after the date of
2 enactment of this Act.

3 (f) REPORT TO CONGRESS.—Section 508(b) of the
4 FDA Safety and Innovation Act (21 U.S.C. 355c–1(b))
5 is amended—

6 (1) in paragraph (10), by striking “; and” and
7 inserting “;”; and

8 (2) by striking paragraph (11) and inserting
9 the following:

10 “(11) an assessment of the impact of the
11 amendments to such section 505B made by the
12 RACE for Children Act on pediatric labeling of
13 drugs and pediatric labeling of molecularly targeted
14 drugs for the treatment of cancer;

15 “(12) an assessment of the efforts of the Sec-
16 retary to implement the plan developed under sec-
17 tion 505C–1 of the Federal Food, Drug, and Cos-
18 metic Act, regarding earlier submission of pediatric
19 studies under sections 505A and 505B, including—

20 “(A) the average length of time after the
21 approval of an application under section
22 505(b)(1) of the Federal Food, Drug, and Cos-
23 metic Act (21 U.S.C. 355(b)(1)) before studies
24 conducted pursuant to such sections 505A or

1 505B are completed, submitted, and incor-
2 porated into labeling;

3 “(B) the average length of time after the
4 receipt of a proposed pediatric study request be-
5 fore the Secretary responds to such request;

6 “(C) the average length of time after the
7 submission of a proposed pediatric study re-
8 quest before the Secretary issues a written re-
9 quest for such studies;

10 “(D) the number of written requests issued
11 for each investigational new drug prior to the
12 submission of an application under section
13 505(b)(1) of the Federal Food, Drug, and Cos-
14 metic Act; and

15 “(E) the average number, and range of
16 numbers, of amendments to written requests
17 issued;

18 “(13) a list of sponsors of applications or hold-
19 ers of approved applications who received exclusivity
20 under such section 505A after receiving a letter
21 issued under such section 505B(d)(1) and before the
22 studies referred to in such letter were completed and
23 submitted; and

1 “(14) a list of assessments required under sub-
 2 section (a)(2)(A)(i)(II), and (b)(1)(B)(ii) of section
 3 505B.”.

4 (g) RULE OF CONSTRUCTION.—Nothing in this sec-
 5 tion, including the amendments made by this section, shall
 6 limit the authority of the Secretary of Health and Human
 7 Services to issue written requests under section 505A of
 8 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
 9 355a).

10 **SEC. 3. IMPROVING THE TIMELINESS OF PEDIATRIC STUD-**
 11 **IES.**

12 (a) INFORMING INTERNAL REVIEW COMMITTEE.—
 13 Section 505A(f) of the Federal Food, Drug, and Cosmetic
 14 Act (21 U.S.C. 355a(f)) is amended by adding at the end
 15 the following:

16 “(7) INFORMING INTERNAL REVIEW COM-
 17 MITTEE.—The Secretary shall provide to the com-
 18 mittee referred to in paragraph (1) any response
 19 issued to an applicant or holder with respect to a
 20 proposed pediatric study request.”.

21 (b) ACTION ON SUBMISSIONS.—Section 505A(d) of
 22 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
 23 355a(d)) is amended—

24 (1) by redesignating paragraphs (3) through
 25 (5) as paragraphs (4) through (6), respectively; and

1 (2) by inserting after paragraph (2) the fol-
2 lowing:

3 “(3) ACTION ON SUBMISSIONS.—The Secretary
4 shall review and act upon a submission of a pro-
5 posed pediatric study request or a sponsor’s pro-
6 posed amendment to a written request for pediatric
7 studies within 120 days of the submission.”.

8 (c) STUDY.—The Secretary of Health and Human
9 Services, acting through the internal review committee es-
10 tablished under section 505C of the Federal Food, Drug,
11 and Cosmetic Act (21 U.S.C. 355d) shall, not later than
12 one year after the date of enactment of this Act, develop
13 and implement a plan to achieve, when appropriate, earlier
14 submission of pediatric studies under section 505A of the
15 Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355a).
16 Such plan shall include recommendations to achieve—

17 (1) earlier discussion of proposed pediatric
18 study requests and written requests with sponsors,
19 and if appropriate, at the meeting required under
20 section 505B(e)(2)(C) of the Federal Food, Drug,
21 and Cosmetic Act (21 U.S.C. 355c(e)(2)(C)), as
22 amended by section 2;

23 (2) earlier issuance of written requests for a pe-
24 diatric study under such section 505A, including for
25 investigational new drugs prior to the submission of

1 an application under section 505(b)(1) of the Fed-
2 eral Food, Drug, and Cosmetic Act (21 U.S.C.
3 355(b)(1)); and

4 (3) shorter timelines, when appropriate, for the
5 completion of studies pursuant to a written request
6 under such section 505A.

7 **SEC. 4. NEONATOLOGY EXPERTISE.**

8 Section 6(d) of the Best Pharmaceuticals for Chil-
9 dren Act (21 U.S.C. 393a(d)) is amended by striking “For
10 the 5-year period beginning on the date of enactment of
11 this subsection, at” and inserting “At”.

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