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

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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.

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 ''Research to Accelerate Cures and Equity for Children Act'' or the ''RACE for Children Act''.

SEC. 2. DRUG DEVELOPMENT FOR PEDIATRIC CANCER.

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

(1) in subsection (a)(2)(A)(i) by striking ''product for the claimed indications in all relevant pediatric subpopulations; and'' and inserting ''product in all relevant pediatric subpopulations—

''(I) for the claimed indications; or'';

''(II) for a pediatric cancer indication, if the drug is intended for the treatment of an adult cancer and is directed at a molecular target considered to be germane to the growth and progression of such pediatric cancer; and'';

(2) in subsection (b)(1)—

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

''(A)(i) the drug or biological product is used for a substantial number of pediatric patients—

''(I) for the labeled indications; or

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

an adult cancer and is directed at a molecular target considered to be germane to the growth and progression of such pediatric cancer; and''; and

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

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

''(i) for one or more of the claimed indications; or

''(ii) for a pediatric cancer indication, if the drug is intended for the treatment of an adult cancer and is directed at a molecular target considered to be germane to the growth and progression of such pediatric cancer; or''; and

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

''(2) the drug or biological product is in a class of products, is for an indication, or is directed at a specific molecular target in an adult cancer and such 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

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

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

(A) in the heading of paragraph (2), by striking "MEETING" and inserting "MEETINGS";

(B) in the heading of paragraph (2)(C), by striking "MEETING" and inserting "MEETINGS";

(C) in clauses (ii) and (iii) of paragraph (2)(C), by striking "no meeting" each place it appears and inserting "no meeting under clause (i)(II)"; and

(D) in paragraph (3) by striking "meeting under paragraph (2)(C)(i)" and inserting "meeting under paragraph (2)(C)(i)(II)".

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

sidered to be germane to the growth and progression of a pediatric cancer,'' after ''regulation,''.

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

(1) study designs;

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

(3) considerations for implementation of such section 505B, as so amended, and waivers of the requirements of such section 505B with regard to molecular targets for which several drugs may be under investigation.

(e) APPLICABILITY.—This section and the amendments made by this section apply with respect to applications for a drug submitted under section 505 of the Federal Food, Drug, or Cosmetic Act (21 U.S.C. 355) or section 351 of the Public Health Service Act (42 U.S.C. 262)

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

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

(1) in paragraph (10), by striking ''; and'' and inserting '';''; and

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

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

''(12) an assessment of the efforts of the Secretary to implement the plan developed under section 505C–1 of the Federal Food, Drug, and Cosmetic Act, regarding earlier submission of pediatric studies under sections 505A and 505B, including—

''(A) the average length of time after the approval of an application under section 505(b)(1) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(b)(1)) before studies conducted pursuant to such sections 505A or

505B are completed, submitted, and incorporated into labeling;

''(B) the average length of time after the receipt of a proposed pediatric study request before the Secretary responds to such request;

''(C) the average length of time after the submission of a proposed pediatric study request before the Secretary issues a written request for such studies;

''(D) the number of written requests issued for each investigational new drug prior to the submission of an application under section 505(b)(1) of the Federal Food, Drug, and Cosmetic Act; and

''(E) the average number, and range of numbers, of amendments to written requests issued;

''(13) a list of sponsors of applications or holders of approved applications who received exclusivity under such section 505A after receiving a letter issued under such section 505B(d)(1) and before the studies referred to in such letter were completed and submitted; and

''(14) a list of assessments required under subsection (a)(2)(A)(i)(II), and (b)(1)(B)(ii) of section 505B.''.

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

SEC. 3. IMPROVING THE TIMELINESS OF PEDIATRIC STUDIES.

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

''(7) INFORMING INTERNAL REVIEW COMMITTEE.—The Secretary shall provide to the committee referred to in paragraph (1) any response issued to an applicant or holder with respect to a proposed pediatric study request.''.

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

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

(2) by inserting after paragraph (2) the following:

''(3) ACTION ON SUBMISSIONS.—The Secretary shall review and act upon a submission of a proposed pediatric study request or a sponsor's proposed amendment to a written request for pediatric studies within 120 days of the submission.''.

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

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

(2) earlier issuance of written requests for a pediatric study under such section 505A, including for investigational new drugs prior to the submission of

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

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

SEC. 4. NEONATOLOGY EXPERTISE.

Section 6(d) of the Best Pharmaceuticals for Children Act (21 U.S.C. 393a(d)) is amended by striking ''For the 5-year period beginning on the date of enactment of this subsection, at'' and inserting ''At''.

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