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117TH CONGRESS
1ST SESSION

H. R. 5416

To amend the Federal Food, Drug, and Cosmetic Act to establish additional authorities of the Food and Drug Administration regarding the conduct of pediatric investigations of molecularly targeted drugs to treat cancer, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

SEPTEMBER 29, 2021

Mr. BUTTERFIELD (for himself, Mr. MCCAUL, and Ms. WILD) introduced the following bill; which was referred to the Committee on Energy and Commerce

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A BILL

To amend the Federal Food, Drug, and Cosmetic Act to establish additional authorities of the Food and Drug Administration regarding the conduct of pediatric investigations of molecularly targeted drugs to treat cancer, 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 "Give Kids a Chance Act".

SEC. 2. RESEARCH INTO PEDIATRIC USES OF DRUGS; ADDITIONAL AUTHORITIES OF FOOD AND DRUG ADMINISTRATION REGARDING MOLECULARLY TARGETED CANCER DRUGS.

(a) IN GENERAL.—

(1) AUTHORITY REGARDING INVESTIGATION OF COMBINATION OF ACTIVE INGREDIENTS.—Section 505B(a)(3) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)(3)) is amended—

(A) by redesignating subparagraphs (B) and (C) as subparagraphs (C) and (D), respectively; and

(B) by striking subparagraph (A) and inserting the following:

"(A) IN GENERAL.—For purposes of paragraph (1)(B), the investigation described in this paragraph is (as determined by the Secretary) a molecularly targeted pediatric cancer investigation of—

"(i) the drug or biological product for which the application referred to in such paragraph is submitted; or

"(ii) the active ingredient or ingredients of such drug or biological product in combination with—

"(I) an active ingredient of a drug or biological product for which there is in effect an exemption for investigational use under section 505(i) that was submitted by the person submitting the application referred to in paragraph (1)(B); or

"(II) an active ingredient of a drug for which an approval under section 505 is in effect, or an active ingredient of a biological product for which an approval under section 351 of the Public Health Service Act is in effect.

"(B) ADDITIONAL REQUIREMENTS.—

"(i) DESIGN OF INVESTIGATION.—A molecularly targeted pediatric cancer investigation referred to in subparagraph (A) shall be designed to yield clinically meaningful pediatric study data, gathered using appropriate formulations for each age group for which the study is required, regarding dosing, safety, and preliminary efficacy.

"(ii) PURPOSE OF INVESTIGATION.—The purpose of a molecularly targeted pediatric cancer investigation referred to in subparagraph (A) shall be—

"(I) in the case of such an investigation conducted with respect to a drug or biological product referred to in clause (i) of such subparagraph, to inform potential pediatric labeling of the drug or biological product involved; and

"(II) in the case of such an investigation conducted with respect to a combination of active ingredients described to in clause (ii) of such subparagraph, to assist in determining the relevance of its molecular target to the growth or progression of a pediatric cancer.

"(iii) PRECLINICAL DATA.—The reports on an investigation required in paragraph (1)(B) shall include the results of all preclinical studies on which the decision to conduct such investigation was based.

"(iv) RULE OF CONSTRUCTION REGARDING INACTIVE INGREDIENTS.—With respect to a combination of active ingredients referred to in subparagraph (A)(ii), such subparagraph may not be construed as addressing the use of inactive ingredients with such combination. For purposes of such subparagraph, the Secretary may establish such requirements with respect to inactive ingredients as the Secretary determines to be appropriate.".

(2) CLARIFYING APPLICABILITY OF CERTAIN PROVISIONS.—Section 505B(a)(3) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)(3)), as amended by paragraph (1), is amended by adding at the end the following:

"(E) INTERNAL COMMITTEE REVIEW; LABELING CHANGES; DISSEMINATION OF INFORMATION; ADVERSE EVENTS; SCOPE OF AUTHORITY.—Subsections (f) through (j) shall apply with respect to investigations described in this paragraph to the same extent and in the same manner as such subsections apply with respect to the assessments required under paragraph (1)(A), except that subsection (g) does not

apply with respect to an investigation referred to in subparagraph (A)(ii).''.

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

(A) in paragraph (3)(C), as redesignated by paragraph (1)(A) of this subsection, by striking ''investigations described in this paragraph'' and inserting ''investigations referred to in subparagraph (A)(i)'';

(B) in paragraph (3)(D), as redesignated by paragraph (1)(A) of this subsection, by striking ''the assessments under paragraph (2)(B)'' and inserting ''the assessments required under paragraph (1)(A)''; and

(C) in paragraph (5)(D), by inserting before the period at the end the following: '', except this subparagraph is not applicable to a drug or biological product that is the subject of an investigation referred to in paragraph (3)(A)(ii)''.

(b) AUTHORITY REGARDING PRECLINICAL STUDIES.—Section 505B(a)(1) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)(1)), as amended by sub-

section (b)(1), is further amended by adding at the end the following:

"(D) PRECLINICAL STUDIES GENERALLY.—

"(i) IN GENERAL.—With respect to an application for an exemption for investigational use under section 505(i) for a drug or biological product that is intended for the treatment of an adult cancer, the Secretary may require, as a condition of permitting the exemption to go into effect, that the sponsor involved enter into an agreement with the Secretary to conduct not more than two preclinical studies of the drug or biological product in order to assist in determining the relevance of its molecular target to the growth or progression of a pediatric cancer.

"(ii) TIMEFRAME FOR PRECLINICAL STUDIES.—With respect to the drug or biological product involved, an agreement under clause (i) for a preclinical study shall specify the date by which an initial plan for the study will be submitted to the Secretary. The results of the preclinical

study shall be submitted to the Secretary in accordance with a timeframe to which the Secretary and the sponsor involved have agreed.''.

(c) APPLICABILITY.—The amendments made by this section apply with respect to any application under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)), any application under section 505 of such Act (21 U.S.C. 355), and any application under section 351(a) of the Public Health Service Act (42 U.S.C. 262), that is submitted on or after the expiration of the 3-year period beginning on the date of the enactment of this Act.

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