

117TH CONGRESS  
2D SESSION

# S. 4215

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.

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## IN THE SENATE OF THE UNITED STATES

MAY 12, 2022

Mr. RUBIO (for himself and Mr. BENNET) 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 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.

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 “Give Kids a Chance  
5 Act of 2022”.

1 **SEC. 2. RESEARCH INTO PEDIATRIC USES OF DRUGS; ADDI-**  
2 **TIONAL AUTHORITIES OF FOOD AND DRUG**  
3 **ADMINISTRATION REGARDING MOLECU-**  
4 **LARLY TARGETED CANCER DRUGS.**

5 (a) IN GENERAL.—

6 (1) ADDITIONAL ACTIVE INGREDIENT FOR AP-  
7 PPLICATION DRUG; LIMITATION REGARDING NOVEL-  
8 COMBINATION APPLICATION DRUG.—Section  
9 505B(a)(3) of the Federal Food, Drug, and Cos-  
10 metic Act (21 U.S.C. 355c(a)(3)) is amended—

11 (A) by redesignating subparagraphs (B)  
12 and (C) as subparagraphs (C) and (D), respec-  
13 tively; and

14 (B) by striking subparagraph (A) and in-  
15 serting the following:

16 “(A) IN GENERAL.—For purposes of para-  
17 graph (1)(B), the investigation described in this  
18 paragraph is (as determined by the Secretary)  
19 a molecularly targeted pediatric cancer inves-  
20 tigation of—

21 “(i) the drug or biological product for  
22 which the application referred to in such  
23 paragraph is submitted; or

24 “(ii) such drug or biological product  
25 in combination with—

1           “(I) an active ingredient of a  
2 drug for which an approved applica-  
3 tion under section 505(j) is in effect  
4 or an active ingredient of a biological  
5 product for which an approved appli-  
6 cation under section 351(k) of the  
7 Public Health Service Act is in effect,  
8 which drug or biological product is de-  
9 termined by the Secretary to be the  
10 standard of care for treating a pedi-  
11 atric cancer;

12           “(II) an active ingredient of a  
13 drug for which an approved applica-  
14 tion under section 505(b) is in effect  
15 to treat an adult cancer, or an active  
16 ingredient of a biological product for  
17 which an approved application under  
18 section 351(a) of the Public Health  
19 Service Act is in effect to treat an  
20 adult cancer, which approved applica-  
21 tion is held by the same person sub-  
22 mitting the application; or

23           “(III) an active ingredient of a  
24 drug or biological product for which  
25 there is in effect an exemption for in-

1                   vestigational use under section 505(i),  
2                   which drug or biological product is  
3                   under such exemption being studied  
4                   jointly by the person submitting the  
5                   application referred to in paragraph  
6                   (1)(B) and by another person pursu-  
7                   ant to an agreement between such  
8                   persons.

9                   “(B) ADDITIONAL REQUIREMENTS.—

10                   “(i) DESIGN OF INVESTIGATION.—A  
11                   molecularly targeted pediatric cancer inves-  
12                   tigation referred to in subparagraph (A)  
13                   shall be designed to yield clinically mean-  
14                   ingful pediatric study data, gathered using  
15                   appropriate formulations for each age  
16                   group for which the study is required, re-  
17                   garding dosing, safety, and preliminary ef-  
18                   ficacy to inform potential pediatric label-  
19                   ing.

20                   “(ii) LIMITATION.—Studies described  
21                   in subparagraph (A)(ii) may be required  
22                   only if the drug or biological product for  
23                   which the application referred to in para-  
24                   graph (1)(B) contains either—

1                   “(I) a single new active ingre-  
2                   dient; or

3                   “(II) more than one active ingre-  
4                   dient, if an application for the com-  
5                   bination of active ingredients has not  
6                   previously been approved but each ac-  
7                   tive ingredient has been previously ap-  
8                   proved to treat an adult cancer.

9                   “(iii) PRECLINICAL DATA.—The Sec-  
10                  retary may require that reports on an in-  
11                  vestigation required pursuant to paragraph  
12                  (1)(B) include the results of all preclinical  
13                  studies on which the decision to conduct  
14                  such investigation was based.

15                  “(iv) RULE OF CONSTRUCTION RE-  
16                  GARDING INACTIVE INGREDIENTS.—With  
17                  respect to a combination of active ingredi-  
18                  ents referred to in subparagraph (A)(ii),  
19                  such subparagraph shall not be construed  
20                  as addressing the use of inactive ingredi-  
21                  ents with such combination.”.

22                  (2) CONFORMING AMENDMENTS.—Section  
23                  505B(a) of the Federal Food, Drug, and Cosmetic  
24                  Act (21 U.S.C. 355c(a)) is amended—

1 (A) in paragraph (3)(C), as redesignated  
2 by paragraph (1)(A) of this subsection, by  
3 striking “investigations described in this para-  
4 graph” and inserting “investigations referred to  
5 in subparagraph (A)(i)”; and

6 (B) in paragraph (3)(D), as redesignated  
7 by paragraph (1)(A) of this subsection, by  
8 striking “the assessments under paragraph  
9 (2)(B)” and inserting “the assessments re-  
10 quired under paragraph (1)(A)”.

11 (b) AUTHORITY REGARDING PRECLINICAL STUD-  
12 IES.—Section 505B(a)(1) of the Federal Food, Drug, and  
13 Cosmetic Act (21 U.S.C. 355c(a)(1)) is amended by add-  
14 ing at the end the following:

15 “(C) PRECLINICAL STUDIES GEN-  
16 ERALLY.—

17 “(i) IN GENERAL.—With respect to a  
18 submission for an exemption for investiga-  
19 tional use under section 505(i) for a drug  
20 or biological product that is intended for  
21 the treatment of an adult cancer, the Sec-  
22 retary may require, as a condition of per-  
23 mitting the exemption to go into effect,  
24 that the sponsor involved enter into an  
25 agreement with the Secretary to conduct

1 not more than 2 preclinical studies of the  
2 drug or biological product in order to as-  
3 sist in determining the relevance of its mo-  
4 lecular target to the growth or progression  
5 of a pediatric cancer.

6 “(ii) TIMEFRAME FOR PRECLINICAL  
7 STUDIES.—With respect to the drug or bi-  
8 ological product involved, an agreement  
9 under clause (i) for a preclinical study  
10 shall specify the date by which an initial  
11 plan for the study will be submitted to the  
12 Secretary except that the Secretary may  
13 not require the submission of such plan  
14 any earlier than 90 days after the exemp-  
15 tion referred to in clause (i) goes into ef-  
16 fect. The results of the preclinical study  
17 shall be submitted to the Secretary in ac-  
18 cordance with a timeframe to which the  
19 Secretary and the sponsor involved have  
20 agreed. Such timeframe shall provide for  
21 deferrals equivalent to deferrals under  
22 paragraphs (4) and (5).

23 “(iii) USE OF PRECLINICAL STUDY  
24 RESULTS.—The Secretary may not use the  
25 results of the preclinical studies under

1 clause (i) to require additional clinical  
2 studies under subparagraph (B) other than  
3 the pediatric cancer studies specified in the  
4 agreement under clause (i).”.

5 (c) APPLICABILITY.—The amendments made by this  
6 section apply with respect to any submission under section  
7 505(i) of the Federal Food, Drug, and Cosmetic Act (21  
8 U.S.C. 355(i)), any application under section 505 of such  
9 Act (21 U.S.C. 355), and any application under section  
10 351(a) of the Public Health Service Act (42 U.S.C. 262),  
11 that is submitted on or after the date that is 2 years after  
12 the date of enactment of this Act.

13 (d) REPORT TO CONGRESS.—Not later than 1 year  
14 after the date of enactment of this Act, the Secretary of  
15 Health and Human Services shall submit a report to Con-  
16 gress on the Secretary’s efforts, in coordination with in-  
17 dustry, to ensure implementation of the amendments  
18 made by subsections (a) and (b) by the date that is 2 years  
19 after the date of enactment of this Act.

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