

119TH CONGRESS
1ST SESSION

S. 3302

To amend the Federal Food, Drug, and Cosmetic Act with respect to molecularly targeted pediatric cancer investigations, and for other purposes.

IN THE SENATE OF THE UNITED STATES

DECEMBER 2, 2025

Mr. MULLIN (for himself, Mr. BENNET, Ms. KLOBUCHAR, Mrs. SHAHEEN, Mr. MARSHALL, Mr. SCOTT of Florida, Ms. COLLINS, Mr. COONS, Mrs. CAPITO, Mr. KELLY, Mr. HUSTED, Mr. KIM, Mr. SHEEHY, Mrs. MOODY, Mr. HICKENLOOPER, Ms. DUCKWORTH, Mr. BOOZMAN, Mr. SCHIFF, Mr. KENNEDY, Mr. REED, Mr. SCHMITT, Mr. MARKEY, Mr. BOOKER, and Mr. JUSTICE) 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 with respect to molecularly targeted pediatric cancer investigations, 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 “Mikaela Naylor Give
5 Kids a Chance Act of 2025”.

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 a molecularly targeted pediatric
 19 cancer investigation of—

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

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

25 “(I) an active ingredient of a
 26 drug or biological product—

1 “(aa) for which an approved
2 application under section 505(j)
3 under this Act or under section
4 351(k) of the Public Health
5 Service Act is in effect; and

6 “(bb) that is determined by
7 the Secretary, after consultation
8 with the applicant, to be part of
9 the standard of care for treating
10 a pediatric cancer; or

11 “(II) an active ingredient of a
12 drug or biological product—

13 “(aa) for which an approved
14 application under section 505(b)
15 of this Act or section 351(a) of
16 the Public Health Service Act to
17 treat an adult cancer is in effect
18 and is held by the same person
19 submitting the application under
20 paragraph (1)(B); and

21 “(bb) that is directed at a
22 molecular target that the Sec-
23 retary determines to be substan-
24 tially relevant to the growth or
25 progression of a pediatric cancer.

“(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 that is gathered using appropriate formulations for each age group for which the study is required, regarding dosing, safety, and preliminary efficacy to inform potential pediatric labeling.

“(ii) LIMITATION.—An investigation described in subparagraph (A)(ii) may be required only if the drug or biological product for which the application referred to in paragraph (1)(B) contains either—

“(I) a single new active ingredient; or

“(II) more than one active ingredient, if an application for the combination of active ingredients has not previously been approved but each active ingredient is in a drug product that has been previously approved to treat an adult cancer.

1 “(iii) RESULTS OF ALREADY-COM-
 2 PLETED PRECLINICAL STUDIES OF APPLI-
 3 CATION DRUG.—With respect to an inves-
 4 tigation required pursuant to paragraph
 5 (1)(B), the Secretary may require the re-
 6 sults of any completed preclinical studies
 7 relevant to the initial pediatric study plan
 8 be submitted to the Secretary at the same
 9 time that the initial pediatric study plan
 10 required under subsection (e)(1) is sub-
 11 mitted.

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

19 (2) DETERMINATION OF APPLICABLE REQUIRE-
 20 MENTS.—Section 505B(e)(1) of the Federal Food,
 21 Drug, and Cosmetic Act (21 U.S.C. 355c(e)(1)) is
 22 amended by adding at the end the following: “The
 23 Secretary shall determine whether subparagraph (A)
 24 or (B) of subsection (a)(1) applies with respect to an
 25 application before the date on which the applicant is

1 required to submit the initial pediatric study plan
2 under paragraph (2)(A).”.

3 (3) CLARIFYING APPLICABILITY.—Section
4 505B(a)(1) of the Federal Food, Drug, and Cos-
5 metic Act (21 U.S.C. 355c(a)(1)) is amended by
6 adding at the end the following:

7 “(C) RULE OF CONSTRUCTION.—No appli-
8 cation that is subject to the requirements of
9 subparagraph (B) shall be subject to the re-
10 quirements of subparagraph (A), and no appli-
11 cation (or supplement to an application) that is
12 subject to the requirements of subparagraph
13 (A) shall be subject to the requirements of sub-
14 paragraph (B).”.

15 (4) CONFORMING AMENDMENTS.—Section
16 505B(a) of the Federal Food, Drug, and Cosmetic
17 Act (21 U.S.C. 355c(a)) is amended—

18 (A) in paragraph (3)(C), as redesignated
19 by paragraph (1)(A) of this subsection, by
20 striking “investigations described in this para-
21 graph” and inserting “investigations referred to
22 in subparagraph (A)”; and

23 (B) in paragraph (3)(D), as redesignated
24 by paragraph (1)(A) of this subsection, by
25 striking “the assessments under paragraph

1 (2)(B)” and inserting “the assessments re-
2 quired under paragraph (1)(A)”.

3 (b) GUIDANCE.—The Secretary of Health and
4 Human Services, acting through the Commissioner of
5 Food and Drugs, shall—

6 (1) not later than 12 months after the date of
7 enactment of this Act, issue draft guidance on the
8 implementation of the amendments made by sub-
9 section (a); and

10 (2) not later than 12 months after closing the
11 comment period on such draft guidance, finalize
12 such guidance.

13 (c) APPLICABILITY.—The amendments made by this
14 section apply with respect to any application under section
15 505(b) of the Federal Food, Drug, and Cosmetic Act (21
16 U.S.C. 355(b)) and any application under section 351(a)
17 of the Public Health Service Act (42 U.S.C. 262(a)), that
18 is submitted on or after the date that is 3 years after the
19 date of enactment of this Act.

20 (d) REPORTS TO CONGRESS.—

21 (1) SECRETARY OF HEALTH AND HUMAN SERV-
22 ICES.—Not later than 6 years after the date of en-
23 actment of this Act, the Secretary of Health and
24 Human Services shall submit to the Committee on
25 Energy and Commerce of the House of Representa-

1 tives and the Committee on Health, Education,
2 Labor, and Pensions of the Senate a report on the
3 Secretary's efforts, in coordination with industry, to
4 ensure implementation of the amendments made by
5 subsection (a).

6 (2) GAO STUDY AND REPORT.—

7 (A) STUDY.—Not later than 8 years after
8 the date of enactment of this Act, the Comp-
9 troller General of the United States shall con-
10 duct a study of the effectiveness of requiring
11 assessments and investigations described in sec-
12 tion 505B of the Federal Food, Drug, and Cos-
13 metic Act (21 U.S.C. 355c), as amended by
14 subsection (a), in the development of drugs and
15 biological products for pediatric cancer indica-
16 tions, including consideration of any benefits to,
17 or burdens on, pediatric cancer drug develop-
18 ment.

19 (B) FINDINGS.—Not later than 10 years
20 after the date of enactment of this Act, the
21 Comptroller General shall submit to the Com-
22 mittee on Energy and Commerce of the House
23 of Representatives and the Committee on
24 Health, Education, Labor, and Pensions of the

1 Senate a report containing the findings of the
 2 study conducted under subparagraph (A).

3 **SEC. 3. EXTENSION OF AUTHORITY TO ISSUE PRIORITY RE-**
 4 **VIEW VOUCHERS TO ENCOURAGE TREAT-**
 5 **MENTS FOR RARE PEDIATRIC DISEASES.**

6 (a) EXTENSION.—Section 529(b)(5) of the Federal
 7 Food, Drug, and Cosmetic Act (21 U.S.C. 360ff(b)(5)) is
 8 amended by striking “December 20, 2024, unless” and all
 9 that follows through the period at the end and inserting
 10 “September 30, 2030.”.

11 (b) USER FEE PAYMENT.—Subsection 529(c)(4) of
 12 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
 13 360ff(c)(4)) is amended by striking subparagraph (A) and
 14 inserting the following:

15 “(A) IN GENERAL.—The priority review
 16 user fee required by this subsection shall be due
 17 upon the submission of a human drug applica-
 18 tion under section 505(b)(1) or section 351(a)
 19 of the Public Health Service Act for which the
 20 priority review voucher is used. All other user
 21 fees associated with the human drug application
 22 shall be due as required by the Secretary or
 23 under applicable law.”.

24 (c) GAO REPORT ON EFFECTIVENESS OF RARE PE-
 25 DIATRIC DISEASE PRIORITY VOUCHER AWARDS IN

1 INCENTIVIZING RARE PEDIATRIC DISEASE DRUG DEVEL-
2 OPMENT.—

3 (1) GAO STUDY.—

4 (A) STUDY.—The Comptroller General of
5 the United States shall conduct a study of the
6 effectiveness of awarding rare pediatric disease
7 priority vouchers under section 529 of the Fed-
8 eral Food, Drug, and Cosmetic Act (21 U.S.C.
9 360ff), as amended by subsection (a), in the de-
10 velopment of human drug products that treat or
11 prevent rare pediatric diseases (as defined in
12 such section 529).

13 (B) CONTENTS OF STUDY.—In conducting
14 the study under subparagraph (A), the Comp-
15 troller General shall examine the following:

16 (i) The indications for each drug or
17 biological product that—

18 (I) is the subject of a rare pedi-
19 atric disease product application (as
20 defined in section 529 of the Federal
21 Food, Drug, and Cosmetic Act (21
22 U.S.C. 360ff)) for which a priority re-
23 view voucher was awarded; and

24 (II) was approved under section
25 505 of the Federal Food, Drug, and

1 Cosmetic Act (42 U.S.C. 355) or li-
2 censed under section 351 of the Pub-
3 lic Health Service Act (42 U.S.C.
4 262).

5 (ii) Whether, and to what extent, an
6 unmet need related to the treatment or
7 prevention of a rare pediatric disease was
8 met through the approval or licensure of
9 such a drug or biological product.

10 (iii) The size of the company to which
11 a priority review voucher was awarded
12 under section 529 of the Federal Food,
13 Drug, and Cosmetic Act (21 U.S.C. 360ff)
14 for such a drug or biological product.

15 (iv) The value of such priority review
16 voucher if transferred.

17 (v) Identification of each drug for
18 which a priority review voucher awarded
19 under such section 529 was used.

20 (vi) The size of the company using
21 each priority review voucher awarded
22 under such section 529.

23 (vii) The length of the period of time
24 between the date on which a priority re-
25 view voucher was awarded under such sec-

tion 529 and the date on which it was used.

(viii) Whether, and to what extent, an unmet need related to the treatment or prevention of a rare pediatric disease was met through the approval under section 505 of the Federal Food, Drug, and Cosmetic Act (42 U.S.C. 355) or licensure under section 351 of the Public Health Service Act (42 U.S.C. 262) of a drug for which a priority review voucher was used.

(ix) Whether, and to what extent, companies were motivated by the availability of priority review vouchers under section 529 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff) to attempt to develop a drug for a rare pediatric disease.

(x) Whether, and to what extent, pediatric review vouchers awarded under such section were successful in stimulating development and expedited patient access to drug products for treatment or prevention of a rare pediatric disease that wouldn't

1 otherwise take place without the incentive
2 provided by such vouchers.

3 (xi) The impact of such priority re-
4 view vouchers on the workload, review
5 process, and public health prioritization ef-
6 forts of the Food and Drug Administra-
7 tion.

8 (xii) Any other incentives in Federal
9 law that exist for companies developing
10 drugs or biological products described in
11 clause (i).

12 (2) REPORT ON FINDINGS.—Not later than 5
13 years after the date of the enactment of this Act, the
14 Comptroller General of the United States shall sub-
15 mit to the Committee on Energy and Commerce of
16 the House of Representatives and the Committee on
17 Health, Education, Labor, and Pensions of the Sen-
18 ate a report containing the findings of the study
19 conducted under paragraph (1).

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