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

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A BILL

To amend the Federal Food, Drug, and Cosmetic Act with respect to molecularly targeted pediatric cancer investigations, 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 ''Mikaela Naylon Give Kids a Chance Act of 2025''.

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

(a) IN GENERAL.—

(1) ADDITIONAL ACTIVE INGREDIENT FOR APPLICATION DRUG; LIMITATION REGARDING NOVEL-COMBINATION APPLICATION DRUG.—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 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) such drug or biological product used in combination with—

''(I) an active ingredient of a drug or biological product—

"(aa) for which an approved application under section 505(j) under this Act or under section 351(k) of the Public Health Service Act is in effect; and

"(bb) that is determined by the Secretary, after consultation with the applicant, to be part of the standard of care for treating a pediatric cancer; or

"(II) an active ingredient of a drug or biological product—

"(aa) for which an approved application under section 505(b) of this Act or section 351(a) of the Public Health Service Act to treat an adult cancer is in effect and is held by the same person submitting the application under paragraph (1)(B); and

"(bb) that is directed at a molecular target that the Secretary determines to be substantially relevant to the growth or 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.

"(iii) RESULTS OF ALREADY-COMPLETED PRECLINICAL STUDIES OF APPLICATION DRUG.—With respect to an investigation required pursuant to paragraph (1)(B), the Secretary may require the results of any completed preclinical studies relevant to the initial pediatric study plan be submitted to the Secretary at the same time that the initial pediatric study plan required under subsection (e)(1) is submitted.

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

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

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

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

''(C) RULE OF CONSTRUCTION.—No application that is subject to the requirements of subparagraph (B) shall be subject to the requirements of subparagraph (A), and no application (or supplement to an application) that is subject to the requirements of subparagraph (A) shall be subject to the requirements of subparagraph (B).''.

(4) 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)''; and

(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)''.

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

(1) not later than 12 months after the date of enactment of this Act, issue draft guidance on the implementation of the amendments made by subsection (a); and

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

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

(d) REPORTS TO CONGRESS.—

(1) SECRETARY OF HEALTH AND HUMAN SERVICES.—Not later than 6 years after the date of enactment of this Act, the Secretary of Health and Human Services shall submit to the Committee on Energy and Commerce of the House of Representa-

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

(2) GAO STUDY AND REPORT.—

(A) STUDY.—Not later than 8 years after the date of enactment of this Act, the Comptroller General of the United States shall conduct a study of the effectiveness of requiring assessments and investigations described in section 505B of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c), as amended by subsection (a), in the development of drugs and biological products for pediatric cancer indications, including consideration of any benefits to, or burdens on, pediatric cancer drug development.

(B) FINDINGS.—Not later than 10 years after the date of enactment of this Act, the Comptroller General shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the

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

SEC. 3. EXTENSION OF AUTHORITY TO ISSUE PRIORITY REVIEW VOUCHERS TO ENCOURAGE TREATMENTS FOR RARE PEDIATRIC DISEASES.

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

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

''(A) IN GENERAL.—The priority review user fee required by this subsection shall be due upon the submission of a human drug application under section 505(b)(1) or section 351(a) of the Public Health Service Act for which the priority review voucher is used. All other user fees associated with the human drug application shall be due as required by the Secretary or under applicable law.''.

(c) GAO REPORT ON EFFECTIVENESS OF RARE PEDIATRIC DISEASE PRIORITY VOUCHER AWARDS IN

INCENTIVIZING RARE PEDIATRIC DISEASE DRUG DEVELOPMENT.—

(1) GAO STUDY.—

(A) STUDY.—The Comptroller General of the United States shall conduct a study of the effectiveness of awarding rare pediatric disease priority vouchers under section 529 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff), as amended by subsection (a), in the development of human drug products that treat or prevent rare pediatric diseases (as defined in such section 529).

(B) CONTENTS OF STUDY.—In conducting the study under subparagraph (A), the Comptroller General shall examine the following:

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

(I) is the subject of a rare pediatric disease product application (as defined in section 529 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff)) for which a priority review voucher was awarded; and

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

Cosmetic Act (42 U.S.C. 355) or licensed under section 351 of the Public Health Service Act (42 U.S.C. 262).

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

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

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

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

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

(vii) The length of the period of time between the date on which a priority review 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

otherwise take place without the incentive provided by such vouchers.

(xi) The impact of such priority review vouchers on the workload, review process, and public health prioritization efforts of the Food and Drug Administration.

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

(2) REPORT ON FINDINGS.—Not later than 5 years after the date of the enactment of this Act, the Comptroller General of the United States shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate a report containing the findings of the study conducted under paragraph (1).

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