AMENDMENT IN THE NATURE OF A SUBSTITUTE TO H.R. 3433 OFFERED BY MR. BILIRAKIS OF FLORIDA

Strike all after the enacting clause and insert the following:

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Give Kids a Chance Act of 2024''.

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 (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) such drug or biological product 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 to be 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 has been previously approved to treat an adult cancer.

''(iii) RESULTS OF ALREADY-COMPLETED PRECLINICAL STUDIES OF APPLICATION DRUG.—The Secretary may require that reports on an investigation required pursuant to paragraph (1)(B) 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 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) shall apply 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 2 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 Representatives 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 3 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.

(B) FINDINGS.—Not later than 7 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. ENSURING COMPLETION OF PEDIATRIC STUDY REQUIREMENTS.

(a) EQUAL ACCOUNTABILITY FOR PEDIATRIC STUDY REQUIREMENTS.—Section 505B(d) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(d)) is amended—

(1) in paragraph (1), by striking ''Beginning 270'' and inserting ''NONCOMPLIANCE LETTER.—Beginning 270'';

(2) in paragraph (2)—

(A) by striking ''The drug or'' and inserting ''EFFECT OF NONCOMPLIANCE.—The drug or''; and

(B) by striking ''(except that the drug or biological product shall not be subject to action under section 303)'' and inserting ''(except that the drug or biological product shall be subject

to action under section 303 only if such person demonstrated a lack of due diligence in satisfying the applicable requirement)''; and

(3) by adding at the end the following:

''(3) LIMITATION.—The Secretary shall not issue enforcement actions under section 303 for failures under this subsection in the case of a drug or biological product that is no longer marketed.''.

(b) DUE DILIGENCE.—Section 505B(d) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(d)), as amended by subsection (a), is further amended by adding at the end the following:

''(4) DUE DILIGENCE.—Before the Secretary may conclude that a person failed to submit or otherwise meet a requirement as described in the matter preceding paragraph (1), the Secretary shall—

''(A) issue a noncompliance letter pursuant to paragraph (1);

''(B) provide such person with a 45-day period beginning on the date of receipt of such noncompliance letter to respond in writing as set forth in such paragraph; and

''(C) after reviewing such written response, determine whether the person demonstrated a

lack of due diligence in satisfying such requirement.''.

(c) CONFORMING AMENDMENTS.—Section 303(f)(4)(A) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 333(f)(4)(A)) is amended by striking ''or 505–1'' and inserting ''505–1, or 505B''.

(d) TRANSITION RULE.—The Secretary of Health and Human Services may take enforcement action under section 303 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 333) only for failures described in section 505B(d) of such Act (21 U.S.C. 355c(d)) that occur on or after the date that is 180 days after the date of enactment of this Act.

SEC. 4. FDA REPORT ON PREA ENFORCEMENT.

Section 508(b) of the Food and Drug Administration Safety and Innovation Act (21 U.S.C. 355c–1(b)) is amended—

(1) in paragraph (11), by striking the semicolon at the end and inserting '', including an evaluation of compliance with deadlines provided for in deferrals and deferral extensions;'';

(2) in paragraph (15), by striking ''and'' at the end;

(3) in paragraph (16), by striking the period at the end and inserting ''; and''; and

(4) by adding at the end the following:

''(17) a listing of penalties, settlements, or payments under section 303 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 353) for failure to comply with requirements under such section 505B, including, for each penalty, settlement, or payment, the name of the drug, the sponsor thereof, and the amount of the penalty, settlement, or payment imposed; and''.

SEC. 5. EXTENSION OF AUTHORITY TO ISSUE PRIORITY REVIEW VOUCHERS TO ENCOURAGE TREATMENTS FOR RARE PEDIATRIC DISEASES.

Paragraph (5) of section 529(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff(b)) is amended by striking ''September 30, 2024, unless'' and all that follows through the period at the end and inserting ''September 30, 2029.''.

SEC. 6. LIMITATIONS ON EXCLUSIVE APPROVAL OR LICENSURE OF ORPHAN DRUGS.

(a) IN GENERAL.—Section 527 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360cc) is amended—

(1) in subsection (a), in the matter following paragraph (2), by striking ''same disease or condition'' and inserting ''same approved use or indication within such rare disease or condition'';

(2) in subsection (b)—

(A) in the matter preceding paragraph (1), by striking ''same rare disease or condition'' and inserting ''same approved use or indication for which such 7-year period applies to such already approved or licensed drug''; and

(B) in paragraph (1), by inserting '', relating to the approved use or indication,'' after ''the needs'';

(3) in subsection (c)(1), by striking ''same rare disease or condition as the already approved drug'' and inserting ''same use or indication for which the already approved or licensed drug was approved or licensed''; and

(4) by adding at the end the following:

''(f) APPROVED USE OR INDICATION DEFINED.—In this section, the term 'approved use or indication' means the use or indication approved under section 505 of this Act or licensed under section 351 of the Public Health Service Act for a drug designated under section 526 for a rare disease or condition.''.

(b) APPLICATION OF AMENDMENTS.—The amendments made by subsection (a) shall apply with respect to any drug designated under section 526 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb), regard-

less of the date on which the drug was so designated, and regardless of the date on which the drug was approved under section 505 of such Act (21 U.S.C. 355) or licensed under section 351 of the Public Health Service Act (42 U.S.C. 262).

SEC. 7. PROGRAM FOR PEDIATRIC STUDIES OF DRUGS.

Section 409I(d) of the Public Health Service Act (42 U.S.C. 284m(d)) is amended to read as follows:

''(d) FUNDING.—Of the amount made available for pediatric research to each national research institute and national center under this title for each of fiscal years 2025, 2026, and 2027, the Director of NIH is authorized to make available up to one percent of such amount for pediatric research under this section.''.

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