AMENDMENT TO THE AMENDMENT IN THE NATURE OF A SUBSTITUTE TO H.R. 3433

OFFERED BY M__. _____

Page 8, line 15, strike ''2030'' and insert ''2027''.

At the end of the amendment, add the following:

SEC. 5. PEDIATRIC STUDIES OF ORPHAN DRUGS.

(a) APPLICATION OF PEDIATRIC RESEARCH REQUIREMENTS TO ORPHAN DRUGS.—

(1) IN GENERAL.—Section 505B(k) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(k)) is amended to read as follows:

''(k) RELATION TO ORPHAN DRUGS.—

''(1) IN GENERAL.—This section does not apply to a drug or biological product for an indication for which orphan designation has been granted under section 526 unless the Secretary determines that pediatric assessments of such drug or biological product required under this section could represent a meaningful therapeutic benefit as described in subsection (c).

''(2) DEFERRALS AND WAIVERS.—Deferrals and waivers under subsections (a)(4) and (a)(5)

shall apply to assessments described in this subsection to the same extent and in the same manner as such deferrals and waivers apply with respect to the assessments under subsection (a)(1), and waivers under subsection (b)(2) shall apply to assessments described in this subsection to the same extent and in the same manner as such waivers apply with respect to the assessments required pursuant to subsection (b)(1).''.

(2) APPLICABILITY.—The amendment made by paragraph (1) applies only to applications described in subparagraph (A) or (B) of section 505B(a)(1) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)(1)) that are submitted on or after the later of—

(A) the date that is 18 months after the date of issuance of the final guidance under subsection (b); and

(B) such later date as may be specified by the Secretary of Health and Human Services (referred to in this Act as the ''Secretary'') by regulation.

(b) GUIDANCE.—

(1) ISSUANCE.—The Secretary shall—

(A) not later than 12 months after the date of enactment of this Act, issue draft guidance describing how, upon the applicability of the amendment made by subsection (a)(1), the requirements of section 505B of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c) will apply with respect to any drug or biological product for an indication within a disease or condition for which orphan designation has been granted under section 526 of such Act (21 U.S.C. 360bb); and

(B) not later than 18 months after the date of the public meeting required by subsection (c)(1), finalize such draft guidance.

(2) CONTENTS.—The guidance under subsection (b) shall address the following:

(A) Information regarding how full and partial waivers under subsections (a)(5)(A), (a)(5)(B), and (b)(2) of section 505B of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c) for any drug or biological product for an indication within a disease or condition for which orphan designation has been granted under section 526 of such Act (21 U.S.C. 360bb) will be granted.

(B) Application of the requirements of section 505B(e) of such Act (21 U.S.C. 355c(e)) to drugs or biological products for an indication within a disease or condition for which orphan designation has been granted under section 526 of such Act (21 U.S.C. 360bb), including submission and timing of planned requests for full or partial waivers and responses by the Food and Drug Administration to those requests.

(C) Rare diseases and conditions (as defined in section 526(a)(2) of such Act (21 U.S.C. 360bb(a)(2)) that should be added to the lists under section 505B(a)(5)(E) and 505B(b)(2)(E) of such Act, as added by this Act, and a process for regularly updating such lists.

(D) Situations where the initial pediatric study plan under section 505B(e) of such Act (21 U.S.C. 355c(e)) includes a plan to fulfill the requirements of section 505B(a) of such Act (21 U.S.C. 355c(a)) without requesting waivers in any age group.

(E) Consideration of how the Secretary will balance the unique scientific challenges of rare disease drug development with the need for

improved pediatric labeling of drugs and biological products for indications within diseases or conditions for which orphan designation has been granted under section 526 of such Act (21 U.S.C. 360bb).

(F) Considerations of the strengths, weaknesses, appropriateness, and limitations of different types of real-world evidence specific to the fulfillment of requirements under section 505B of such Act (21 U.S.C. 355c).

(G) Consideration of input received from the public meeting set forth in subsection (c).

(c) PUBLIC MEETING.—The Secretary shall—

(1) not later than 6 months after the date of issuance of the draft guidance under subsection (b)(1)(A), hold a public meeting to inform the final guidance to be issued under subsection (b)(1)(B); and

(2) publish prior notice of such meeting in the Federal Register.

(d) GAO STUDY.—Not later than 4 years after the applicability date described in subsection (a)(2), the Comptroller General of the United States shall submit to the Committee on Energy and Commerce and the Committee on Ways and Means of the House of Representa-

tives and the Committee on Health, Education, Labor, and Pensions of the Senate a report that—

(1) addresses the impacts of this Act on—

(A) rare disease drug development in the United States; and

(B) the availability of pediatric information on drugs and biological products within diseases or conditions for indications for which orphan designation has been granted under section 526 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb); and

(2) includes—

(A) the findings of a survey of companies of varying sizes engaged in the development of orphan drugs, which shall include questions regarding the feasibility and other challenges of conducting pediatric studies for such indications;

(B) input from patient groups and medical provider associations; and

(C) an assessment of the impact changes to required pediatric studies had on drug development for rare diseases.

(e) RULE OF CONSTRUCTION.—Nothing in this section shall be construed to limit requirements for investiga-

tions, as described in section 505B(a)(3) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)(3)), of molecularly targeted pediatric cancer drugs for which orphan designation has been granted under section 526 of such Act (21 U.S.C. 360bb).

(f) CERTAINTY REGARDING WAIVERS.—Section 505B of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c) is amended—

(1) in subsection (a)(5), by adding at the end the following:

"(E) AUTOMATIC FULL WAIVER LIST.—The Secretary shall maintain a list, posted on the website of the Food and Drug Administration, of adult-related diseases and conditions—

"(i) with respect to which the necessary studies are impossible or highly impracticable, as described in subparagraph (A)(i); or

"(ii) for which a drug or biological product for such disease or condition otherwise meets the criteria described in subparagraph (A).";

(2) in subsection (b)(2), by adding at the end the following:

''(E) AUTOMATIC FULL WAIVER LIST.—The Secretary shall maintain a list, posted on the website of the Food and Drug Administration, of adult-related diseases and conditions with respect to which the necessary studies would meet the criteria for a full waiver under subparagraph (A) .''; and

(3) in subsection (e)(4), by adding at the end the following: ''If, at the time of an applicant's submission of the initial pediatric study plan, the disease or condition for which the drug is intended to treat appears on the list under subsection (a)(5)(E), then the assessments for such disease or condition shall be waived under subsection (a)(5).''.

SEC. 6. REAUTHORIZING THE PROGRAM FOR PEDIATRIC STUDIES OF DRUGS.

Section 409I(d)(1) of the Public Health Service Act (42 U.S.C. 284m(d)(1)) is amended to read as follows:

''(1) IN GENERAL.—There is authorized to be appropriated to carry out this section $50,000,000 for each of fiscal years 2023 through 2027.''.

SEC. 7. 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 (2), by striking ''(except that the drug or biological product shall not be subject to action under section 303)''; and

(2) 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—

''(A) is no longer marketed;

''(B) is no longer protected by any patent or exclusivity period;

''(C) is subject to an unexpired deferral or deferral extension under this section; or

''(D) has been issued a noncompliance letter under paragraph (1) but with respect to which the relevant person has not been given a reasonable period to make a response in writing in accordance with paragraph (4)(B).''.

(b) DUE DILIGENCE.—Section 505B(d) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355c(d)) 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 oth-

erwise meet a requirement as described in the matter preceding paragraph (1), the Secretary shall—

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

''(B) give the person a reasonable period to respond in writing as set forth in such paragraph; and

''(C) determine that the response indicates 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 shall take no enforcement actions under section 303 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 333) for failures described in section 505B(d) of such Act (21 U.S.C. 355c(d)) before the date this is 12 months after the date of finalization of the guidance required by section 2(b).

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