II

117TH CONGRESS
2D SESSION

S. 4446

To modernize the process of accelerated approval of a drug for a serious or life-threatening disease or condition.

IN THE SENATE OF THE UNITED STATES

JUNE 22, 2022

Ms. COLLINS (for herself and Mr. KAINE) 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 modernize the process of accelerated approval of a drug for a serious or life-threatening disease or condition.

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 ''Modernizing the Accelerated Approval Pathway Act of 2022''.

SEC. 2. MODERNIZING ACCELERATED APPROVAL.

(a) IN GENERAL.—Section 506(c) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356(c)) is amended—

(1) in paragraph (2)—

(A) by redesignating subparagraphs (A) and (B) as clauses (i) and (ii), respectively, and adjusting the margins accordingly;

(B) by striking ''Approval of a product'' and inserting the following:

''(A) IN GENERAL.—Approval of a product'';

(C) in clause (i) of such subparagraph (A), as so redesignated, by striking ''appropriate postapproval studies'' and inserting ''an appropriate postapproval study or studies (which may be augmented or supported by real world evidence)''; and

(D) by adding at the end the following:

''(B) STUDIES NOT REQUIRED.—If the Secretary does not require that the sponsor of a product approved under accelerated approval conduct a postapproval study under this paragraph, the Secretary shall publish on the website of the Food and Drug Administration the rationale for why such study is not appropriate or necessary.

''(C) POSTAPPROVAL STUDY CONDITIONS.—Not later than the time of approval of a product under accelerated approval, the Sec-

retary shall specify the conditions for a post-approval study or studies required to be conducted under this paragraph with respect to such product, which may include enrollment targets, the study protocol, and milestones, including the target date of study completion.

''(D) STUDIES BEGUN BEFORE APPROVAL.—The Secretary may require such study or studies to be underway prior to approval.''; and

(2) in paragraph (3)—

(A) by redesignating subparagraphs (A) through (D) as clauses (i) through (iv), respectively, and adjusting the margins accordingly;

(B) by striking ''The Secretary may'' and inserting the following:

''(A) IN GENERAL.—The Secretary may'';

(C) in clause (i) of such subparagraph (A), as so redesignated, by striking ''drug with due diligence'' and inserting ''product with due diligence, including with respect to conditions specified by the Secretary under paragraph (2)(C)'';

(D) in clause (iii) of such subparagraph (A), as so redesignated, by inserting ''shown to be'' after ''product is not''; and

(E) by adding at the end the following:

''(B) EXPEDITED PROCEDURES DESCRIBED.—Expedited procedures described in this subparagraph shall consist of, prior to the withdrawal of accelerated approval—

''(i) providing the sponsor with—

''(I) due notice;

''(II) an explanation for the proposed withdrawal;

''(III) an opportunity for a meeting with the Commissioner or the Commissioner's designee; and

''(IV) an opportunity for written appeal to—

''(aa) the Commissioner; or

''(bb) a designee of the Commissioner who has not participated in the proposal withdrawal of approval (other than a meeting pursuant to subclause (III)) and is not subordinate of an individual (other than the Commissioner) who participated in such proposed withdrawal;

"(ii) providing an opportunity for public comment on the proposing to withdrawal approval;

"(iii) the publication of a summary of the public comments received, and the Secretary's response to such comments, on the website of the Food and Drug Administration; and

"(iv) convening and consulting an advisory committee on issues related to the proposed withdrawal, if requested by the sponsor and if no such advisory committee has previously advised the Secretary on such issues with respect to the withdrawal of the product prior to the sponsor's request.".

(b) REPORTS OF POSTMARKETING STUDIES.—Section 506B(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356b(a)) is amended—

(1) by redesignating paragraph (2) as paragraph (3); and

(2) by inserting after paragraph (1) the following:

"(2) ACCELERATED APPROVAL.—Notwithstanding paragraph (1), a sponsor of a drug ap-

proved under accelerated approval shall submit to the Secretary a report of the progress of any study required under section 506(c), including progress toward enrollment targets, milestones, and other information as required by the Secretary, not later than 180 days after the approval of such drug and not less frequently than every 180 days thereafter, until the study is completed or terminated. The Secretary shall promptly publish on the website of the Food and Drug Administration the information so reported, in an easily searchable format.''.

(c) ENFORCEMENT.—Section 301 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 331), is amended by adding at the end the following:

''(fff) The failure of a sponsor of a product approved under accelerated approval pursuant to section 506(c)—

''(1) to conduct with due diligence any post-approval study required under section 506(c) with respect to such product; or

''(2) to submit timely reports with respect to such product in accordance with section 506B(a)(2).''.

(d) GUIDANCE.—

(1) IN GENERAL.—The Secretary of Health and Human Services shall issue guidance describing—

(A) how sponsor questions related to the identification of novel surrogate or intermediate clinical endpoints may be addressed in early-stage development meetings with the Food and Drug Administration;

(B) the use of novel clinical trial designs that may be used to conduct appropriate post-approval studies as may be required under section 506(c)(2)(A) of the Federal Food, Drug, and Cosmetic Act, as amended by subsection (a);

(C) the expedited procedures described in section 506(c)(3)(B) of the Federal Food, Drug, and Cosmetic Act; and

(D) considerations related to the use of surrogate or intermediate clinical endpoints that may support the accelerated approval of an application under 506(c)(1)(A), including considerations in evaluating the evidence related to any such endpoints.

(2) FINAL GUIDANCE.—The Secretary shall issue—

(A) draft guidance under paragraph (1) not later than 18 months after the date of enactment of this Act; and

(B) final guidance not later than 1 year after the close of the public comment period on such draft guidance.

(e) RARE DISEASE ENDPOINT ADVANCEMENT PILOT.—

(1) IN GENERAL.—The Secretary of Health and Human Services shall establish a pilot program under which the Secretary will establish procedures to provide increased interaction with sponsors of rare disease drug development programs for purposes of advancing the development of efficacy endpoints, including surrogate and intermediate endpoints, for drugs intended to treat rare diseases, including through—

(A) determining eligibility of participants for such program; and

(B) developing and implementing a process for applying to, and participating in, such a program.

(2) PUBLIC WORKSHOPS.—The Secretary shall conduct up to 3 public workshops, which shall be completed not later than September 30, 2026, to discuss topics relevant to the development of endpoints for rare diseases, which may include discussions about—

(A) novel endpoints developed through the pilot program established under this subsection; and

(B) as appropriate, the use of real world evidence and real work data to support the validation of efficacy endpoints, including surrogate and intermediate endpoints, for rare diseases.

(3) REPORT.—Not later than September 30, 2027, the Secretary 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 describing the outcomes of the pilot program established under this subsection.

(4) GUIDANCE.—Not later than September 30, 2027, the Secretary shall issue guidance describing best practices and strategies for development of efficacy endpoints, including surrogate and intermediate endpoints, for rare diseases.

(5) SUNSET.—The Secretary may not accept any new application or request to participate in the program established by this subsection on or after October 1, 2027.

(f) ACCELERATED APPROVAL COUNCIL.—

(1) GENERAL.—Not later than 180 days after the date of enactment of this Act, the Secretary of Health and Human Services shall establish an intra-agency coordinating council within the Food and Drug Administration to ensure the consistent and appropriate use of accelerated approval across the Food and Drug Administration, pursuant to section 506(c) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356(c)).

(2) MEMBERSHIP.—The members of the Council shall consist of the following senior officials, or a designee of such official, from the Food and Drug Administration and relevant Centers:

(A) The Director of the Center for Drug Evaluation and Research.

(B) The Director of the Center for Biologics Evaluation and Research.

(C) The Director of the Oncology Center of Excellence.

(D) The Director of the Office of New Drugs.

(E) The Director of the Office of Orphan Products Development.

(F) The Director of the Office of Tissues and Advanced Therapies.

(G) The Director of the Office of Medical Policy.

(H) At least 3 directors of review division overseeing products approved under accelerated approval, including at least one director of a review division within the Office of Neuroscience.

(3) DUTIES OF THE COUNCIL.—

(A) MEETINGS.—The Council shall convene not fewer than 3 times per calendar year to discuss issues related to accelerated approval, including any relevant cross-disciplinary approaches related to product review with respect to accelerated approval.

(B) POLICY DEVELOPMENT.—The Council shall directly engage with product review teams to support the consistent and appropriate use of accelerated approval across the Food and Drug Administration. Such activities may include—

(i) developing guidance for Food and Drug Administration staff and best practices for, and across, product review teams, including with respect to communication between sponsors and the Food and Drug Administration and the review of products under accelerated approval;

(ii) providing training for product review teams; and

(iii) advising review divisions on product-specific development, review, and withdrawal of products under accelerated approval.

(4) PUBLICATION OF A REPORT.—Not later than 1 year after the date of enactment of this Act, and annually thereafter, the council shall publish on the public website of the Food and Drug Administration a report on the activities of the council.

(g) RULE OF CONSTRUCTION.—Nothing in this section (including the amendments made by this section) shall be construed to affect products approved pursuant to 506(c) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356(c)) prior to the date of enactment of this Act.

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