

117TH CONGRESS  
2D SESSION

**S.** \_\_\_\_\_

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

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IN THE SENATE OF THE UNITED STATES

Ms. COLLINS (for herself and Mr. KAINE) introduced the following bill; which was read twice and referred to the Committee on \_\_\_\_\_

\_\_\_\_\_  
**A BILL**

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

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 “Modernizing the Accel-  
5 erated Approval Pathway Act of 2022”.

6 **SEC. 2. MODERNIZING ACCELERATED APPROVAL.**

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

10               (1) in paragraph (2)—

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

4 (B) by striking “Approval of a product”  
5 and inserting the following:

6 “(A) IN GENERAL.—Approval of a prod-  
7 uct”;

8 (C) in clause (i) of such subparagraph (A),  
9 as so redesignated, by striking “appropriate  
10 postapproval studies” and inserting “an appro-  
11 priate postapproval study or studies (which may  
12 be augmented or supported by real world evi-  
13 dence)”; and

14 (D) by adding at the end the following:

15 “(B) STUDIES NOT REQUIRED.—If the  
16 Secretary does not require that the sponsor of  
17 a product approved under accelerated approval  
18 conduct a postapproval study under this para-  
19 graph, the Secretary shall publish on the  
20 website of the Food and Drug Administration  
21 the rationale for why such study is not appro-  
22 priate or necessary.

23 “(C) POSTAPPROVAL STUDY CONDI-  
24 TIONS.—Not later than the time of approval of  
25 a product under accelerated approval, the Sec-

1           retary shall specify the conditions for a post-  
2           approval study or studies required to be con-  
3           ducted under this paragraph with respect to  
4           such product, which may include enrollment  
5           targets, the study protocol, and milestones, in-  
6           cluding the target date of study completion.

7           “(D) STUDIES BEGUN BEFORE AP-  
8           PROVAL.—The Secretary may require such  
9           study or studies to be underway prior to ap-  
10          proval.”; and

11          (2) in paragraph (3)—

12           (A) by redesignating subparagraphs (A)  
13           through (D) as clauses (i) through (iv), respec-  
14           tively, and adjusting the margins accordingly;

15           (B) by striking “The Secretary may” and  
16           inserting the following:

17           “(A) IN GENERAL.—The Secretary may”;

18           (C) in clause (i) of such subparagraph (A),  
19           as so redesignated, by striking “drug with due  
20           diligence” and inserting “product with due dili-  
21           gence, including with respect to conditions spec-  
22           ified by the Secretary under paragraph (2)(C)”;

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

1 (E) by adding at the end the following:

2 “(B) EXPEDITED PROCEDURES DE-  
3 SCRIBED.—Expedited procedures described in  
4 this subparagraph shall consist of, prior to the  
5 withdrawal of accelerated approval—

6 “(i) providing the sponsor with—

7 “(I) due notice;

8 “(II) an explanation for the pro-  
9 posed withdrawal;

10 “(III) an opportunity for a meet-  
11 ing with the Commissioner or the  
12 Commissioner’s designee; and

13 “(IV) an opportunity for written  
14 appeal to—

15 “(aa) the Commissioner; or

16 “(bb) a designee of the  
17 Commissioner who has not par-  
18 ticipated in the proposal with-  
19 drawal of approval (other than a  
20 meeting pursuant to subclause  
21 (III)) and is not subordinate of  
22 an individual (other than the  
23 Commissioner) who participated  
24 in such proposed withdrawal;

1                   “(ii) providing an opportunity for  
2                   public comment on the proposing to with-  
3                   drawal approval;

4                   “(iii) the publication of a summary of  
5                   the public comments received, and the Sec-  
6                   retary’s response to such comments, on the  
7                   website of the Food and Drug Administra-  
8                   tion; and

9                   “(iv) convening and consulting an ad-  
10                  visory committee on issues related to the  
11                  proposed withdrawal, if requested by the  
12                  sponsor and if no such advisory committee  
13                  has previously advised the Secretary on  
14                  such issues with respect to the withdrawal  
15                  of the product prior to the sponsor’s re-  
16                  quest.”.

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

20                         (1) by redesignating paragraph (2) as para-  
21                         graph (3); and

22                         (2) by inserting after paragraph (1) the fol-  
23                         lowing:

24                                 “(2) ACCELERATED APPROVAL.—Notwith-  
25                                 standing paragraph (1), a sponsor of a drug ap-

1       proved under accelerated approval shall submit to  
2       the Secretary a report of the progress of any study  
3       required under section 506(c), including progress to-  
4       ward enrollment targets, milestones, and other infor-  
5       mation as required by the Secretary, not later than  
6       180 days after the approval of such drug and not  
7       less frequently than every 180 days thereafter, until  
8       the study is completed or terminated. The Secretary  
9       shall promptly publish on the website of the Food  
10      and Drug Administration the information so re-  
11      ported, in an easily searchable format.”.

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

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

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

20          “(2) to submit timely reports with respect to  
21          such product in accordance with section  
22          506B(a)(2).”.

23      (d) GUIDANCE.—

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

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

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

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

15 (D) considerations related to the use of  
16 surrogate or intermediate clinical endpoints  
17 that may support the accelerated approval of an  
18 application under 506(c)(1)(A), including con-  
19 siderations in evaluating the evidence related to  
20 any such endpoints.

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

23 (A) draft guidance under paragraph (1)  
24 not later than 18 months after the date of en-  
25 actment of this Act; and

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

4 (e) RARE DISEASE ENDPOINT ADVANCEMENT  
5 PILOT.—

6 (1) IN GENERAL.—The Secretary of Health and  
7 Human Services shall establish a pilot program  
8 under which the Secretary will establish procedures  
9 to provide increased interaction with sponsors of  
10 rare disease drug development programs for pur-  
11 poses of advancing the development of efficacy  
12 endpoints, including surrogate and intermediate  
13 endpoints, for drugs intended to treat rare diseases,  
14 including through—

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

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

20 (2) PUBLIC WORKSHOPS.—The Secretary shall  
21 conduct up to 3 public workshops, which shall be  
22 completed not later than September 30, 2026, to  
23 discuss topics relevant to the development of  
24 endpoints for rare diseases, which may include dis-  
25 cussions about—



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

4 (B) as appropriate, the use of real world  
5 evidence and real work data to support the vali-  
6 dation of efficacy endpoints, including surrogate  
7 and intermediate endpoints, for rare diseases.

8 (3) REPORT.—Not later than September 30,  
9 2027, the Secretary shall submit to the Committee  
10 on Energy and Commerce of the House of Rep-  
11 resentatives and the Committee on Health, Edu-  
12 cation, Labor, and Pensions of the Senate a report  
13 describing the outcomes of the pilot program estab-  
14 lished under this subsection.

15 (4) GUIDANCE.—Not later than September 30,  
16 2027, the Secretary shall issue guidance describing  
17 best practices and strategies for development of effi-  
18 cacy endpoints, including surrogate and intermediate  
19 endpoints, for rare diseases.

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

24 (f) ACCELERATED APPROVAL COUNCIL.—

1           (1) GENERAL.—Not later than 180 days after  
2           the date of enactment of this Act, the Secretary of  
3           Health and Human Services shall establish an intra-  
4           agency coordinating council within the Food and  
5           Drug Administration to ensure the consistent and  
6           appropriate use of accelerated approval across the  
7           Food and Drug Administration, pursuant to section  
8           506(c) of the Federal Food, Drug, and Cosmetic Act  
9           (21 U.S.C. 356(c)).

10           (2) MEMBERSHIP.—The members of the Coun-  
11           cil shall consist of the following senior officials, or  
12           a designee of such official, from the Food and Drug  
13           Administration and relevant Centers:

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

16                   (B) The Director of the Center for Bio-  
17                   logics Evaluation and Research.

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

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

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

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

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

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

7 (3) DUTIES OF THE COUNCIL.—

8 (A) MEETINGS.—The Council shall con-  
9 vene not fewer than 3 times per calendar year  
10 to discuss issues related to accelerated approval,  
11 including any relevant cross-disciplinary ap-  
12 proaches related to product review with respect  
13 to accelerated approval.

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

19 (i) developing guidance for Food and  
20 Drug Administration staff and best prac-  
21 tices for, and across, product review teams,  
22 including with respect to communication  
23 between sponsors and the Food and Drug  
24 Administration and the review of products  
25 under accelerated approval;

1 (ii) providing training for product re-  
2 view teams; and

3 (iii) advising review divisions on prod-  
4 uct-specific development, review, and with-  
5 drawal of products under accelerated ap-  
6 proval.

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

12 (g) RULE OF CONSTRUCTION.—Nothing in this sec-  
13 tion (including the amendments made by this section)  
14 shall be construed to affect products approved pursuant  
15 to 506(c) of the Federal Food, Drug, and Cosmetic Act  
16 (21 U.S.C. 356(c)) prior to the date of enactment of this  
17 Act.