

119TH CONGRESS
1ST SESSION

S. 705

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

FEBRUARY 25, 2025

Mr. REED (for himself and Mrs. CAPITO) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To amend the Federal Food, Drug, and Cosmetic Act with respect to molecularly targeted pediatric cancer investigations, and for other purposes.

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 “Innovation in Pediatric
5 Drugs Act of 2025”.

6 **SEC. 2. ENSURING COMPLETION OF PEDIATRIC STUDY RE-**
7 **QUIREMENTS.**

8 (a) **EQUAL ACCOUNTABILITY FOR PEDIATRIC STUDY**
9 **REQUIREMENTS.**—Section 505B(d) of the Federal Food,

1 Drug, and Cosmetic Act (21 U.S.C. 355c(d)) is amend-
2 ed—

3 (1) in paragraph (1), by striking “Beginning
4 270” and inserting “NONCOMPLIANCE LETTER.—
5 Beginning 270”;

6 (2) in paragraph (2)—

7 (A) by striking “The drug or” and insert-
8 ing “EFFECT OF NONCOMPLIANCE.—The drug
9 or”; and

10 (B) by striking “(except that the drug or
11 biological product shall not be subject to action
12 under section 303)” and inserting “(except that
13 the drug or biological product shall be subject
14 to action under section 303 only if such person
15 demonstrated a lack of due diligence in satis-
16 fying the applicable requirement)”; and

17 (3) by adding at the end the following:

18 “(3) LIMITATION.—The Secretary shall not
19 issue enforcement actions under section 303 for fail-
20 ures under this subsection in the case of a drug or
21 biological product that is no longer marketed.”.

22 (b) DUE DILIGENCE.—Section 505B(d) of the Fed-
23 eral Food, Drug, and Cosmetic Act (21 U.S.C. 355c(d)),
24 as amended by subsection (a), is further amended by add-
25 ing at the end the following:

1 “(4) DUE DILIGENCE.—Before the Secretary
2 may conclude that a person failed to submit or oth-
3 erwise meet a requirement as described in the mat-
4 ter preceding paragraph (1), the Secretary shall—

5 “(A) issue a noncompliance letter pursuant
6 to paragraph (1);

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

11 “(C) after reviewing such written response,
12 determine whether the person demonstrated a
13 lack of due diligence in satisfying such require-
14 ment.”.

15 (c) CONFORMING AMENDMENTS.—Section
16 303(f)(4)(A) of the Federal Food, Drug, and Cosmetic Act
17 (21 U.S.C. 333(f)(4)(A)) is amended by striking “or 505–
18 1” and inserting “505–1, or 505B”.

19 (d) TRANSITION RULE.—The Secretary of Health
20 and Human Services may take enforcement action under
21 section 303 of the Federal Food, Drug, and Cosmetic Act
22 (21 U.S.C. 333) for failures described in section 505B(d)
23 of such Act (21 U.S.C. 355c(d)) only if such failures occur
24 on or after the date that is 180 days after the date of
25 enactment of this Act.

1 **SEC. 3. FDA REPORT ON PREA ENFORCEMENT.**

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

5 (1) in paragraph (11), by striking the semicolon
6 at the end and inserting “, including an evaluation
7 of compliance with deadlines provided for in defer-
8 rals and deferral extensions;”;

9 (2) in paragraph (15), by striking “and” at the
10 end;

11 (3) in paragraph (16), by striking the period at
12 the end and inserting “; and”; and

13 (4) by adding at the end the following:

14 “(17) a listing of penalties, settlements, or pay-
15 ments under section 303 of the Federal Food, Drug,
16 and Cosmetic Act (21 U.S.C. 353) for failure to
17 comply with requirements under such section 505B,
18 including, for each penalty, settlement, or payment,
19 the name of the drug, the sponsor thereof, and the
20 amount of the penalty, settlement, or payment im-
21 posed.”.

22 **SEC. 4. PROGRAM FOR PEDIATRIC STUDIES OF DRUGS.**

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

25 “(d) FUNDING.—Of the amount made available for
26 pediatric research to each national research institute and

1 national center under this title for each of fiscal years
2 2026 through 2030, the Director of NIH is authorized to
3 make available up to one percent of such amount for pedi-
4 atric research under this section.”.

5 **SEC. 5. PEDIATRIC STUDIES OF ORPHAN DRUGS.**

6 (a) APPLICATION OF PEDIATRIC RESEARCH RE-
7 QUIREMENTS TO ORPHAN DRUGS.—

8 (1) IN GENERAL.—Section 505B(k) of the Fed-
9 eral Food, Drug, and Cosmetic Act (21 U.S.C.
10 355c(k)) is amended to read as follows:

11 “(k) RELATION TO ORPHAN DRUGS.—

12 “(1) IN GENERAL.—This section does not apply
13 to a drug or biological product for an indication for
14 which orphan designation has been granted under
15 section 526 unless the Secretary determines that pe-
16 diatric assessments of such drug or biological prod-
17 uct required under this section could represent a
18 meaningful therapeutic benefit as described in sub-
19 section (c).

20 “(2) DEFERRALS AND WAIVERS.—Deferrals
21 and waivers under subsections (a)(4) and (a)(5)
22 shall apply to assessments described in this sub-
23 section to the same extent and in the same manner
24 as such deferrals and waivers apply with respect to
25 the assessments under subsection (a)(1), and waiv-

ers 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 section as the “Secretary”) by regulation.

(b) GUIDANCE.—

(1) ISSUANCE.—The Secretary shall—

(A) not later than 1 year 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

1 Food, Drug, and Cosmetic Act (21 U.S.C.
2 355c) will apply with respect to any drug or bi-
3 ological product for an indication within a dis-
4 ease or condition for which orphan designation
5 has been granted under section 526 of such Act
6 (21 U.S.C. 360bb); and

7 (B) not later than 18 months after the
8 date of the public meeting required by sub-
9 section (c)(1), finalize such draft guidance.

10 (2) CONTENTS.—The guidance under sub-
11 section (b) shall address the following:

12 (A) Information regarding how full and
13 partial waivers under subsections (a)(5)(A),
14 (a)(5)(B), and (b)(2) of section 505B of the
15 Federal Food, Drug, and Cosmetic Act (21
16 U.S.C. 355c) for any drug or biological product
17 for an indication within a disease or condition
18 for which orphan designation has been granted
19 under section 526 of such Act (21 U.S.C.
20 360bb) will be granted.

21 (B) Application of the requirements of sec-
22 tion 505B(e) of such Act (21 U.S.C. 355c(e))
23 to drugs or biological products for an indication
24 within a disease or condition for which orphan
25 designation has been granted under section 526

1 of such Act (21 U.S.C. 360bb), including sub-
2 mission and timing of planned requests for full
3 or partial waivers and responses by the Food
4 and Drug Administration to those requests.

5 (C) Rare diseases and conditions (as de-
6 fined in section 526(a)(2) of such Act (21
7 U.S.C. 360bb(a)(2)) that should be added to
8 the lists under section 505B(a)(5)(E) and
9 505B(b)(2)(E) of such Act, as added by sub-
10 section (f), and a process for regularly updating
11 such lists.

12 (D) Situations where the initial pediatric
13 study plan under section 505B(e) of such Act
14 (21 U.S.C. 355c(e)) includes a plan to fulfill
15 the requirements of section 505B(a) of such
16 Act (21 U.S.C. 355c(a)) without requesting
17 waivers in any age group.

18 (E) Consideration of how the Secretary
19 will balance the unique scientific challenges of
20 rare disease drug development with the need for
21 improved pediatric labeling of drugs and bio-
22 logical products for indications within diseases
23 or conditions for which orphan designation has
24 been granted under section 526 of such Act (21
25 U.S.C. 360bb).

1 (F) Considerations of the strengths, weak-
2 nesses, appropriateness, and limitations of dif-
3 ferent types of real-world evidence specific to
4 the fulfillment of requirements under section
5 505B of such Act (21 U.S.C. 355c).

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

8 (c) PUBLIC MEETING.—The Secretary shall—

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

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

16 (d) GAO STUDY.—Not later than 4 years after the
17 applicability date described in subsection (a)(2), the
18 Comptroller General of the United States shall submit to
19 the Committee on Energy and Commerce and the Com-
20 mittee on Ways and Means of the House of Representa-
21 tives and the Committee on Health, Education, Labor,
22 and Pensions of the Senate a report that—

23 (1) addresses the impacts of this Act on—

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

1 (B) the availability of pediatric information
2 on drugs and biological products within diseases
3 or conditions for indications for which orphan
4 designation has been granted under section 526
5 of the Federal Food, Drug, and Cosmetic Act
6 (21 U.S.C. 360bb); and

7 (2) includes—

8 (A) the findings of a survey of companies
9 of varying sizes engaged in the development of
10 orphan drugs, which shall include questions re-
11 garding the feasibility and other challenges of
12 conducting pediatric studies for such indica-
13 tions;

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

16 (C) an assessment of the impact changes
17 to required pediatric studies had on drug devel-
18 opment for rare diseases.

19 (e) RULE OF CONSTRUCTION.—Nothing in this sec-
20 tion shall be construed to limit requirements for investiga-
21 tions, as described in section 505B(a)(3) of the Federal
22 Food, Drug, and Cosmetic Act (21 U.S.C. 355c(a)(3)), of
23 molecularly targeted pediatric cancer drugs for which or-
24 phan designation has been granted under section 526 of
25 such Act (21 U.S.C. 360bb).

1 (f) CERTAINTY REGARDING WAIVERS.—Section
 2 505B of the Federal Food, Drug, and Cosmetic Act (21
 3 U.S.C. 355c) is amended—

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

6 “(E) AUTOMATIC FULL WAIVER LIST.—

7 The Secretary shall maintain a list, posted on
 8 the website of the Food and Drug Administra-
 9 tion, of adult-related diseases and conditions—

10 “(i) with respect to which the nec-
 11 essary studies are impossible or highly im-
 12 practicable, as described in subparagraph
 13 (A)(i); or

14 “(ii) for which a drug or biological
 15 product for such disease or condition oth-
 16 erwise meets the criteria described in sub-
 17 paragraph (A).”;

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

20 “(E) AUTOMATIC FULL WAIVER LIST.—

21 The Secretary shall maintain a list, posted on
 22 the website of the Food and Drug Administra-
 23 tion, of adult-related diseases and conditions
 24 with respect to which the necessary studies

1 would meet the criteria for a full waiver under
2 subparagraph (A).”; and
3 (3) in subsection (e)(4), by adding at the end
4 the following: “If, at the time of an applicant’s sub-
5 mission of the initial pediatric study plan, the dis-
6 ease or condition for which the drug is intended to
7 treat appears on the list under subsection (a)(5)(E),
8 then the assessments for such disease or condition
9 shall be waived under subsection (a)(5).”.

○