

119TH CONGRESS
1ST SESSION

S. 3551

To amend titles XVIII and XIX of the Social Security Act and title XXVII of the Public Health Service Act to provide for coverage of certain drugs used in the treatment or management of a rare disease or condition, and for other purposes.

IN THE SENATE OF THE UNITED STATES

DECEMBER 17, 2025

Mr. TILLIS (for himself and Mr. HEINRICH) introduced the following bill;
which was read twice and referred to the Committee on Finance

A BILL

To amend titles XVIII and XIX of the Social Security Act and title XXVII of the Public Health Service Act to provide for coverage of certain drugs used in the treatment or management of a rare disease or condition, 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 “Providing Realistic Op-
5 portunity To Equal and Comparable Treatment for Rare
6 Act” or the “PROTECT for Rare Act”.

1 **SEC. 2. COVERAGE OF CERTAIN DRUGS USED IN TREAT-**
2 **MENT OR MANAGEMENT OF A RARE DISEASE**
3 **OR CONDITION.**

4 (a) MEDICARE.—

5 (1) DEFINITION.—

6 (A) IN GENERAL.—Section 1861(t)(2) of
7 the Social Security Act (42 U.S.C. 1395x(t)(2))
8 is amended—

9 (i) in subparagraph (A), by inserting
10 after “regimen” the following: “, or used
11 in the treatment or management of a dis-
12 ease or condition affecting 200,000 or
13 fewer individuals in the United States,”;
14 and

15 (ii) in subparagraph (B)(ii)—

16 (I) in subclause (I), by striking
17 “, or” at the end and inserting a
18 semicolon;

19 (II) in subclause (II), by striking
20 the period at the end and inserting “;
21 or”; and

22 (III) by adding at the end the
23 following new subclause:

24 “(III) in the case of a drug that
25 is used in the treatment or manage-
26 ment of a disease or condition affect-

1 ing 200,000 or fewer individuals in
 2 the United States, such use is sup-
 3 ported by peer-reviewed medical lit-
 4 erature, including clinical guidelines,
 5 and is not specifically listed as not in-
 6 dicated in one or more of the com-
 7 pendia described in section
 8 1927(g)(1)(B)(i), or listed as contra-
 9 indicated in the labeling approved by
 10 the Food and Drug Administration.”.

11 (B) EFFECTIVE DATE.—The amendments
 12 made by subparagraph (A) shall apply to items
 13 and services furnished on or after January 1,
 14 2027..

15 (2) MEDICALLY ACCEPTED USES OF COVERED
 16 PART D DRUGS IN TREATING RARE CONDITIONS.—

17 (A) IN GENERAL.—Section 1860D–
 18 2(e)(4)(A)(i) of the Social Security Act (42
 19 U.S.C. 1395w–104(e)(4)(A)(i)) is amended, in
 20 the matter preceding subclause (I), by inserting
 21 “or in the case of a covered part D drug used
 22 in the treatment or management of a disease or
 23 condition affecting 200,000 or fewer individuals
 24 in the United States,” after “regimen,”

1 (B) EFFECTIVE DATE.—The amendment
 2 made by subparagraph (A) shall apply to plan
 3 years beginning on or after January 1, 2027.

4 (b) MEDICAID.—

5 (1) IN GENERAL.—Section 1927(k)(6) of the
 6 Social Security Act (42 U.S.C. 1396r–8(k)(6)) is
 7 amended to read as follows:

8 “(6) MEDICALLY ACCEPTED INDICATION.—The
 9 term ‘medically accepted indication’ means any use
 10 for a covered outpatient drug—

11 “(A) which is approved under the Federal
 12 Food, Drug, and Cosmetic Act;

13 “(B) which is supported by one or more ci-
 14 tations included or approved for inclusion in
 15 any of the compendia described in subsection
 16 (g)(1)(B)(i); or

17 “(C) which, in the case of a drug used to
 18 treat or manage a disease or condition affecting
 19 200,000 or fewer individuals in the United
 20 States—

21 “(i) is a use of such drug that is sup-
 22 ported by peer-reviewed medical literature,
 23 clinical guidelines, or an expert in such dis-
 24 ease or condition, as identified by a med-
 25 ical society involved in the treatment or

1 management of such disease or condition;
2 and

3 “(ii) is a use of such drug that is not
4 listed as not indicated in the compendia
5 described in subsection (g)(1)(B)(i), or list-
6 ed as contraindicated in the labeling ap-
7 proved by the Food and Drug Administra-
8 tion.”.

9 (2) CONFORMING AMENDMENT.—Section
10 1927(d)(4)(C) of the Social Security Act (42 U.S.C.
11 1396r–8(d)(4)(C)) is amended by striking “com-
12 pendia” and inserting “sources”.

13 (3) EFFECTIVE DATE.—The amendments made
14 by this subsection shall apply with respect to covered
15 outpatient drugs furnished on or after January 1,
16 2027.

17 (c) PRIVATE HEALTH INSURANCE.—

18 (1) IN GENERAL.—

19 (A) PHSA.—Part D of title XXVII of the
20 Public Health Service Act (42 U.S.C. 300gg–
21 111 et seq.) is amended by adding at the end
22 the following new section:

1 **“SEC. 2799A-11. EXPEDITED PROCESS FOR REVIEW ASSOCI-**
 2 **ATED WITH CERTAIN DRUGS USED IN TREAT-**
 3 **MENT OR MANAGEMENT OF A RARE DISEASE**
 4 **OR CONDITION.**

5 “A group health plan or a health insurance issuer of-
 6 fering group or individual health insurance coverage shall
 7 provide an expedited process pursuant to section 2719 by
 8 which a participant, beneficiary, or enrollee, or a designee
 9 or prescribing physician (or other prescriber, as appro-
 10 priate) of the participant, beneficiary, or enrollee, may ap-
 11 peal any denial of coverage for a drug or biological prod-
 12 uct—

13 “(1) approved under section 505 of the Federal
 14 Food, Drug, and Cosmetic Act or licensed under sec-
 15 tion 351;

16 “(2) for which the use is related to treatment
 17 or management of a disease or condition affecting
 18 200,000 or fewer individuals in the United States;
 19 and

20 “(3) the use of which is supported by—

21 “(A) the labeling for the drug or biological
 22 product approved pursuant to such section 505
 23 or 351; or

24 “(B) peer-reviewed literature, including
 25 clinical guidelines, and that is not reviewed un-
 26 favorably in the compendia described in section

1 1927(g)(1)(B)(i) of the Social Security Act and
 2 is not listed as a contraindication in such ap-
 3 proved labeling.”.

4 (B) ERISA.—

5 (i) IN GENERAL.—Subpart B of part
 6 7 of subtitle B of title I of the Employee
 7 Retirement Income Security Act of 1974
 8 (29 U.S.C. 1185 et seq.) is amended by
 9 adding at the end the following new sec-
 10 tion:

11 **“SEC. 736. EXPEDITED PROCESS FOR REVIEW ASSOCIATED**
 12 **WITH CERTAIN DRUGS USED IN TREATMENT**
 13 **OR MANAGEMENT OF A RARE DISEASE OR**
 14 **CONDITION.**

15 “A group health plan or a health insurance issuer of-
 16 fering group health insurance coverage shall provide an
 17 expedited process pursuant to section 2719 of the Public
 18 Health Service Act (42 U.S.C. 300gg–19) by which a par-
 19 ticipant or beneficiary, or a designee or prescribing physi-
 20 cian (or other prescriber, as appropriate) of the partici-
 21 pant or beneficiary, may appeal any denial of coverage for
 22 a drug or biological product—

23 “(1) approved under section 505 of the Federal
 24 Food, Drug, and Cosmetic Act (21 U.S.C. 355) or

1 licensed under section 351 of the Public Health
 2 Service Act (42 U.S.C. 262);

3 “(2) for which the use is related to treatment
 4 or management of a disease or condition affecting
 5 200,000 or fewer individuals in the United States;
 6 and

7 “(3) the use of which is supported by—

8 “(A) the labeling for the drug or biological
 9 product approved pursuant to such section 505
 10 or 351; or

11 “(B) peer-reviewed literature, including
 12 clinical guidelines, and that is not reviewed un-
 13 favorably in the compendia described in section
 14 1927(g)(1)(B)(i) of the Social Security Act (42
 15 U.S.C. 1396r–8(g)(1)(B)(i)) and is not listed as
 16 a contraindication in such approved labeling.”.

17 (ii) CLERICAL AMENDMENT.—The
 18 table of contents in section 1 of the Em-
 19 ployee Retirement Income Security Act of
 20 1974 (29 U.S.C. 1001 et seq.) is amended
 21 by inserting after the item relating to sec-
 22 tion 725 the following new item:

“Sec. 726. Expedited process for review associated with certain drugs used in
 treatment or management of a rare disease or condition.”.

23 (C) IRC.—

1 (i) IN GENERAL.—Subchapter B of
 2 chapter 100 of the Internal Revenue Code
 3 of 1986 is amended by adding at the end
 4 the following new section:

5 **“SEC. 9826. EXPEDITED PROCESS FOR REVIEW ASSOCIATED**
 6 **WITH CERTAIN DRUGS USED IN TREATMENT**
 7 **OR MANAGEMENT OF A RARE DISEASE OR**
 8 **CONDITION.**

9 “A group health plan shall provide an expedited proc-
 10 ess pursuant to section 2719 of the Public Health Service
 11 Act (42 U.S.C. 300gg–19) by which a participant or bene-
 12 ficiary, or a designee or prescribing physician (or other
 13 prescriber, as appropriate) of the participant or bene-
 14 ficiary, may appeal any denial of coverage for a drug or
 15 biological product—

16 “(1) approved under section 505 of the Federal
 17 Food, Drug, and Cosmetic Act (21 U.S.C. 355) or
 18 licensed under section 351 of the Public Health
 19 Service Act (42 U.S.C. 262);

20 “(2) for which the use is related to treatment
 21 or management of a disease or condition affecting
 22 200,000 or fewer individuals in the United States;
 23 and

24 “(3) the use of which is supported by—

1 “(A) the labeling for the drug or biological
2 product approved pursuant to such section 505
3 or 351; or

4 “(B) peer-reviewed literature, including
5 clinical guidelines, and that is not reviewed un-
6 favorably in the compendia described in section
7 1927(g)(1)(B)(i) of the Social Security Act (42
8 U.S.C. 1396r-8(g)(1)(B)(i)) and is not listed as
9 a contraindication in such approved labeling.”.

10 (ii) CLERICAL AMENDMENT.—The
11 table of sections for subchapter B of chap-
12 ter 100 of the Internal Revenue Code of
13 1986 is amended by adding at the end the
14 following new item:

“Sec. 9826. Expedited process for review associated with certain drugs used in
treatment or management of a rare disease or condition.”.

15 (2) EFFECTIVE DATE.—The amendments made
16 by this subsection shall apply with respect to plan
17 years beginning on or after January 1, 2027.

○