

117TH CONGRESS  
1ST SESSION

# S. 670

To amend the Federal Food, Drug, and Cosmetic Act to accelerate development of therapies across the spectrum of rare diseases and conditions and facilitate patient access to such therapies, and for other purposes.

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## IN THE SENATE OF THE UNITED STATES

MARCH 10, 2021

Ms. KLOBUCHAR (for herself and Mr. WICKER) 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 amend the Federal Food, Drug, and Cosmetic Act to accelerate development of therapies across the spectrum of rare diseases and conditions and facilitate patient access to such therapies, 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 “Speeding Therapy Ac-  
5 cess Today Act of 2021”.

6 **SEC. 2. TABLE OF CONTENTS.**

7 The table of contents of this Act is as follows:

Sec. 1. Short title.

Sec. 2. Table of contents.

Sec. 3. Intercenter Institute on Rare Diseases and Conditions.

Sec. 4. Rare Disease and Condition Drug Advisory Committee.

Sec. 5. Grants and contracts for development of drugs for rare diseases and conditions.

1 **SEC. 3. INTERCENTER INSTITUTE ON RARE DISEASES AND**  
 2 **CONDITIONS.**

3 (a) ESTABLISHMENT REQUIRED.—The first sentence  
 4 of section 1014(a) of the Federal Food, Drug, and Cos-  
 5 metic Act (21 U.S.C. 399g(a)) is amended by inserting  
 6 “, at least one of which shall be focused on rare diseases  
 7 and conditions” before the period at the end of the sen-  
 8 tence.

9 (b) TIMING OF ESTABLISHMENT.—Subsection (c) of  
 10 section 1014 of the Federal Food, Drug, and Cosmetic  
 11 Act (21 U.S.C. 399g) is amended to read as follows:

12 “(c) TIMING.—Not later than the date that is 1 year  
 13 after the date of enactment of the Speeding Therapy Ac-  
 14 cess Today Act of 2021, the Secretary shall establish, in  
 15 accordance with this section and section 529B, an Insti-  
 16 tute under subsection (a) focused on rare diseases and  
 17 conditions, to be known as the Intercenter Institute on  
 18 Rare Diseases and Conditions.”.

19 (c) RESPONSIBILITIES.—Subchapter B of chapter V  
 20 of the Federal Food, Drug, and Cosmetic Act (relating  
 21 to drugs for rare diseases or conditions) is amended by  
 22 inserting after section 529A of such Act (21 U.S.C. 360ff–  
 23 1) the following new section:

1 **“SEC. 529B. INTERCENTER INSTITUTE ON RARE DISEASES**  
2 **AND CONDITIONS.**

3 “(a) RESPONSIBILITIES.—In addition to carrying out  
4 activities listed in section 1014(a), the Intercenter Insti-  
5 tute on Rare Diseases and Conditions shall—

6 “(1) serve as the Food and Drug Administra-  
7 tion’s coordinating office for engagement with rare  
8 disease and condition stakeholders, complementing  
9 but not supplanting engagement activities between  
10 stakeholders and the review divisions;

11 “(2) build, within the Food and Drug Adminis-  
12 tration, knowledge and understanding associated  
13 with the review of medical products to treat rare dis-  
14 eases and conditions, including advancements in trial  
15 design, statistical analysis, regulatory science, prod-  
16 uct manufacturing, and other topics as determined  
17 by the Secretary;

18 “(3) implement cross-center rare disease and  
19 condition-focused meetings and policy development;

20 “(4) coordinate rare disease and condition-spe-  
21 cific regulatory science initiatives;

22 “(5) facilitate stakeholder engagement to the  
23 external community and international regulatory  
24 agencies on rare disease and condition product devel-  
25 opment;

1 “(6) establish and implement the Accelerating  
2 Lifesavings Therapies in Treating Ultra-rare Dis-  
3 ease Entities Program under subsection (b); and

4 “(7) establish and carry out the rare disease  
5 and condition third-party payor program under sub-  
6 section (d).

7 “(b) ALTITUDE PROGRAM.—

8 “(1) IN GENERAL.—The Intercenter Institute  
9 shall establish and implement a program, to be  
10 known as the Accelerating Lifesavings Therapies in  
11 Treating Ultra-rare Disease Entities Program, to  
12 identify and make recommendations to address cur-  
13 rent and emerging regulatory science and public pol-  
14 icy challenges associated with developing medical  
15 products to treat rare diseases or conditions in an  
16 individual or very small populations.

17 “(2) ISSUES.—The program under paragraph  
18 (1) shall focus on issues including—

19 “(A) manufacturing standards for thera-  
20 pies described in such paragraph, including in  
21 non-industry settings;

22 “(B) trial designs and metrics;

23 “(C) regulatory flexibilities for abbreviated  
24 toxicology studies, overlapping animal studies,  
25 and patient dosing;

1           “(D) regulatory science, chemistry, manu-  
2           facturing, and other needs associated with de-  
3           veloping such therapies; and

4           “(E) other issues as determined by the  
5           Secretary.

6           “(c) PROPOSALS FOR AMENDING LABELS.—

7           “(1) STAKEHOLDER GROUP.—Not later than  
8           180 days after the date of enactment of this section,  
9           the Intercenter Institute shall convene a meeting of  
10          stakeholders from the rare disease community, in-  
11          cluding patients, caregivers, product manufacturers,  
12          third-party payors, and others, to consider potential  
13          amendments to labels for medical products to treat  
14          rare diseases or conditions approved pursuant to a  
15          pathway under section 506.

16          “(2) GUIDANCE.—Not later than 90 days after  
17          the date of the meeting under paragraph (1), the  
18          Secretary shall issue guidance to propose changes to  
19          how the labels of medical products to treat rare dis-  
20          eases or conditions demonstrate clinical benefits and  
21          reflect relevant scientific data including surrogate  
22          endpoints.

23          “(d) RARE DISEASE AND CONDITION THIRD-PARTY  
24          PAYOR PROGRAM.—

1           “(1) IN GENERAL.—The Intercenter Institute  
2           shall establish and carry out a voluntary rare disease  
3           and condition early third-party payor feedback pro-  
4           gram—

5                   “(A) to inform coverage policies for rare  
6           disease therapies; and

7                   “(B) to inform clinical trial design, patient  
8           engagement, and other data collections.

9           “(2) PROGRAM REQUIREMENTS.—The program  
10          under paragraph (1) shall—

11                   “(A) facilitate voluntary communication  
12          between sponsors of medical products to treat  
13          rare diseases and conditions and third-party  
14          payors; and

15                   “(B) require participation of the Centers  
16          for Medicare & Medicaid Services with rep-  
17          resentation from—

18                           “(i) the Center for Medicare; and

19                           “(ii) the Center for Medicaid and  
20          CHIP Services.

21          “(3) ANNUAL REPORT.—The Intercenter Insti-  
22          tute shall—

23                   “(A) on an annual basis, submit a report  
24          to that Congress on—

1 “(i) the participation within the pro-  
 2 gram under paragraph (1); and

3 “(ii) the impacts of the program  
 4 under paragraph (1); and

5 “(B) post each such report on the public  
 6 website of the Intercenter Institute.

7 “(4) BULLETIN TO MEDICAID DIRECTORS.—  
 8 Following the approval, clearance, or authorization  
 9 by the Food and Drug Administration of a medical  
 10 product to treat a rare disease or condition, the Sec-  
 11 retary shall issue a bulletin to State Medicaid direc-  
 12 tors containing information to help inform coverage  
 13 decisions on the product by State Medicaid and Chil-  
 14 dren’s Health Insurance programs.

15 “(e) DEFINITION.—In this section, the terms ‘Inter-  
 16 center Institute on Rare Diseases and Conditions’ and  
 17 ‘Intercenter Institute’ refer to the Intercenter Institute on  
 18 Rare Diseases and Conditions established pursuant to sec-  
 19 tion 1014.”.

20 **SEC. 4. RARE DISEASE AND CONDITION DRUG ADVISORY**  
 21 **COMMITTEE.**

22 Subchapter B of chapter V of the Federal Food,  
 23 Drug, and Cosmetic Act is further amended by inserting  
 24 after section 529B of such Act, as inserted by section 3,  
 25 the following new section:

1 **“SEC. 529C. RARE DISEASE AND CONDITION DRUG ADVI-**  
 2 **SORY COMMITTEE.**

3 “(a) IN GENERAL.—The Secretary shall establish  
 4 and maintain a committee, to be known as the Rare Dis-  
 5 ease and Condition Drug Advisory Committee (in this sec-  
 6 tion referred to as the ‘Advisory Committee’).

7 “(b) DUTY OF COMMITTEE.—The Advisory Com-  
 8 mittee shall advise the Secretary on issues associated with  
 9 development of therapies to treat rare diseases or condi-  
 10 tions.

11 “(c) SPECIFIC ISSUES.—In advising the Secretary,  
 12 the Advisory Committee may address issues including—

13 “(1) modified or new regulatory pathways to  
 14 support review of therapies;

15 “(2) clinical trial design needs, including devel-  
 16 opment of innovative approaches to clinical trials;

17 “(3) qualifications of biomarkers or other drug  
 18 development tools for use in reviews;

19 “(4) modified or new standards to support the  
 20 review of already marketed drugs being evaluated  
 21 for repurposing to treat a rare disease or condition;  
 22 and

23 “(5) issues—

24 “(A) that pertain to an application for ap-  
 25 proval of a therapy to treat a rare disease or  
 26 condition; and

1           “(B) with respect to which a review divi-  
 2           sion has requested that the Advisory Committee  
 3           provide advice.

4           “(d) MEMBERSHIP.—

5           “(1) IN GENERAL.—The Advisory Committee  
 6           shall consist of—

7           “(A) not more than 15 members appointed  
 8           by the Secretary in accordance with paragraph  
 9           (2); and

10          “(B) the nonvoting ex officio members  
 11          under paragraph (3).

12          “(2) APPOINTED MEMBERS.—

13          “(A) SPECIAL GOVERNMENT EMPLOY-  
 14          EES.—Members of the Advisory Committee ap-  
 15          pointed pursuant to paragraph (1)(A) shall  
 16          serve as special Government employees (as de-  
 17          fined in section 202(a) of title 18, United  
 18          States Code).

19          “(B) ELIGIBILITY.—To be eligible for ap-  
 20          pointment pursuant to paragraph (1)(A), an in-  
 21          dividual shall—

22                 “(i) be eligible to serve as special Gov-  
 23                 ernment employee (as defined in section  
 24                 202(a) of title 18, United States Code);  
 25                 and

1 “(ii) have expertise in the fields of  
2 public policy, law, regulatory policy, eco-  
3 nomics, patient-focused product develop-  
4 ment, or patient advocacy.

5 “(C) COMPOSITION.—Of the members of  
6 the Advisory Committee appointed pursuant to  
7 paragraph (1)(A)—

8 “(i) up to 10 shall be selected from  
9 among experts in the disciplines relevant to  
10 the activities of the Intercenter Institute  
11 on Rare Diseases and Conditions, to in-  
12 clude at least one expert in each of—

13 “(I) rare disease product develop-  
14 ment;

15 “(II) conducting clinical trials  
16 with respect to rare diseases and con-  
17 ditions, including with respect to very  
18 small patient populations;

19 “(III) rare disease and condition  
20 natural history and related studies;

21 “(IV) health economics per-  
22 taining to the development of medical  
23 products for rare diseases or condi-  
24 tions;

1 “(V) manufacturing and related  
2 needs associated with medical prod-  
3 ucts for rare diseases or conditions;  
4 and

5 “(VI) patient experience data col-  
6 lection; and

7 “(ii) up to 5 shall be selected from the  
8 public, to include—

9 “(I) at least 4 individuals who  
10 are representatives of the rare disease  
11 patient community;

12 “(II) at least one individual who  
13 is directly impacted by a rare disease  
14 or condition; and

15 “(III) at least one person who  
16 serves as a family caregiver to a per-  
17 son diagnosed with a rare disease or  
18 condition.

19 “(3) NONVOTING EX OFFICIO MEMBERS.—The  
20 nonvoting ex officio members of the Advisory Com-  
21 mittee under paragraph (1)(B) shall consist of the  
22 following:

23 “(A) The Secretary (or the Secretary’s  
24 designee).

1           “(B) The Director of the Intercenter Insti-  
2           tute on Rare Diseases and Conditions.

3           “(C) The Director of the Center for Bio-  
4           logics Evaluation and Research (or the Direc-  
5           tor’s designee).

6           “(D) The Director of the Center for Drug  
7           Evaluation and Research (or the Director’s des-  
8           ignee).

9           “(E) The Director of the Center for De-  
10          vices and Radiological Health (or the Director’s  
11          designee).

12          “(F) The Director of the National Center  
13          for the Advancing Translational Sciences of the  
14          National Institutes of Health (or the Director’s  
15          designee).

16          “(G) The Administrator of the Centers for  
17          Medicare & Medicaid Services (or the Adminis-  
18          trator’s designee).

19          “(H) Any additional officers or employees  
20          of the Department of Health and Human Serv-  
21          ices as the Secretary determines necessary for  
22          the Advisory Committee to effectively carry out  
23          its functions.

1           “(4) CHAIR.—The Chair of the Advisory Com-  
2       mittee shall be the Director of the Intercenter Insti-  
3       tute for Rare Diseases and Conditions.

4           “(5) TERMS.—

5               “(A) MEMBERS.—

6                   “(i) IN GENERAL.—The term of a  
7       member of the Advisory Committee ap-  
8       pointed pursuant to paragraph (1)(A) shall  
9       be 4 years, except that any member ap-  
10      pointed to fill a vacancy in an unexpired  
11      term shall be appointed for the remainder  
12      of that term.

13                  “(ii) CONTINUED SERVICE.—A mem-  
14      ber appointed pursuant to paragraph  
15      (1)(A) may continue serving as a member  
16      of the Advisory Committee for up to 180  
17      days after the expiration of that member’s  
18      term if a successor has not been appointed.

19                  “(B) REAPPOINTMENT.—A member of the  
20      Advisory Committee who has been appointed  
21      pursuant to paragraph (1)(A) for a term of 4  
22      years may not be reappointed to serve as a  
23      member of the Advisory Committee before the  
24      date that is 2 years after the date of expiration  
25      of that member’s term.

1       “(e) QUORUM.—A majority of the appointed mem-  
2   bers of the Advisory Committee shall constitute a quorum  
3   for the conduct of business.”.

4   **SEC. 5. GRANTS AND CONTRACTS FOR DEVELOPMENT OF**  
5                   **DRUGS FOR RARE DISEASES AND CONDI-**  
6                   **TIONS.**

7       (a) AUTHORITY OF SECRETARY.—Section 5(a) of the  
8   Orphan Drug Act (21 U.S.C. 360ee(a)) is amended—

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

11              (2) by inserting before the period at the end “,  
12   and (4) developing practices pertaining to the chem-  
13   istry, manufacturing, regulatory approval of, and  
14   controls of individualized therapies or therapies to  
15   treat very small populations”.

16       (b) ALTITUDE PROGRAM.—In supporting grants  
17   and contracts under section 5(a)(4) of the Orphan Drug  
18   Act, as added by subsection (a), the Secretary of Health  
19   and Human Services shall consult with the Director of the  
20   Intercenter Institute on Rare Diseases and Conditions re-  
21   garding the Accelerating Lifesavings Therapies in Treat-  
22   ing Ultra-rare Disease Entities Program established under  
23   section 529B(b) of the Federal Food, Drug, and Cosmetic  
24   Act, as added by section 3(c) of this Act, to—

- 1           (1) identify the regulatory science and related
- 2           challenges and needs associated with developing indi-
- 3           vidualized therapies or therapies to treat very small
- 4           patient populations; and
- 5           (2) support research to address such challenges.

○