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.

*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 ''Speeding Therapy Access Today Act of 2021''.

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

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.

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

(a) ESTABLISHMENT REQUIRED.—The first sentence of section 1014(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 399g(a)) is amended by inserting ", at least one of which shall be focused on rare diseases and conditions" before the period at the end of the sentence.

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

"(c) TIMING.—Not later than the date that is 1 year after the date of enactment of the Speeding Therapy Access Today Act of 2021, the Secretary shall establish, in accordance with this section and section 529B, an Institute under subsection (a) focused on rare diseases and conditions, to be known as the Intercenter Institute on Rare Diseases and Conditions.".

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

**"SEC. 529B. INTERCENTER INSTITUTE ON RARE DISEASES AND CONDITIONS.**

''(a) RESPONSIBILITIES.—In addition to carrying out activities listed in section 1014(a), the Intercenter Institute on Rare Diseases and Conditions shall—

''(1) serve as the Food and Drug Administration's coordinating office for engagement with rare disease and condition stakeholders, complementing but not supplanting engagement activities between stakeholders and the review divisions;

''(2) build, within the Food and Drug Administration, knowledge and understanding associated with the review of medical products to treat rare diseases and conditions, including advancements in trial design, statistical analysis, regulatory science, product manufacturing, and other topics as determined by the Secretary;

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

''(4) coordinate rare disease and condition-specific regulatory science initiatives;

''(5) facilitate stakeholder engagement to the external community and international regulatory agencies on rare disease and condition product development;

''(6) establish and implement the Accelerating Lifesavings Therapies in Treating Ultra-rare Disease Entities Program under subsection (b); and

''(7) establish and carry out the rare disease and condition third-party payor program under subsection (d).

''(b) ALTITUDE PROGRAM.—

''(1) IN GENERAL.—The Intercenter Institute shall establish and implement a program, to be known as the Accelerating Lifesavings Therapies in Treating Ultra-rare Disease Entities Program, to identify and make recommendations to address current and emerging regulatory science and public policy challenges associated with developing medical products to treat rare diseases or conditions in an individual or very small populations.

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

''(A) manufacturing standards for therapies described in such paragraph, including in non-industry settings;

''(B) trial designs and metrics;

''(C) regulatory flexibilities for abbreviated toxicology studies, overlapping animal studies, and patient dosing;

''(D) regulatory science, chemistry, manufacturing, and other needs associated with developing such therapies; and

''(E) other issues as determined by the Secretary.

''(c) PROPOSALS FOR AMENDING LABELS.—

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

''(2) GUIDANCE.—Not later than 90 days after the date of the meeting under paragraph (1), the Secretary shall issue guidance to propose changes to how the labels of medical products to treat rare diseases or conditions demonstrate clinical benefits and reflect relevant scientific data including surrogate endpoints.

''(d) RARE DISEASE AND CONDITION THIRD-PARTY PAYOR PROGRAM.—

''(1) IN GENERAL.—The Intercenter Institute shall establish and carry out a voluntary rare disease and condition early third-party payor feedback program—

''(A) to inform coverage policies for rare disease therapies; and

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

''(2) PROGRAM REQUIREMENTS.—The program under paragraph (1) shall—

''(A) facilitate voluntary communication between sponsors of medical products to treat rare diseases and conditions and third-party payors; and

''(B) require participation of the Centers for Medicare & Medicaid Services with representation from—

''(i) the Center for Medicare; and

''(ii) the Center for Medicaid and CHIP Services.

''(3) ANNUAL REPORT.—The Intercenter Institute shall—

''(A) on an annual basis, submit a report to that Congress on—

''(i) the participation within the program under paragraph (1); and

''(ii) the impacts of the program under paragraph (1); and

''(B) post each such report on the public website of the Intercenter Institute.

''(4) BULLETIN TO MEDICAID DIRECTORS.—Following the approval, clearance, or authorization by the Food and Drug Administration of a medical product to treat a rare disease or condition, the Secretary shall issue a bulletin to State Medicaid directors containing information to help inform coverage decisions on the product by State Medicaid and Children's Health Insurance programs.

''(e) DEFINITION.—In this section, the terms 'Intercenter Institute on Rare Diseases and Conditions' and 'Intercenter Institute' refer to the Intercenter Institute on Rare Diseases and Conditions established pursuant to section 1014.''.

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

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

**"SEC. 529C. RARE DISEASE AND CONDITION DRUG ADVISORY COMMITTEE.**

''(a) IN GENERAL.—The Secretary shall establish and maintain a committee, to be known as the Rare Disease and Condition Drug Advisory Committee (in this section referred to as the 'Advisory Committee').

''(b) DUTY OF COMMITTEE.—The Advisory Committee shall advise the Secretary on issues associated with development of therapies to treat rare diseases or conditions.

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

''(1) modified or new regulatory pathways to support review of therapies;

''(2) clinical trial design needs, including development of innovative approaches to clinical trials;

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

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

''(5) issues—

''(A) that pertain to an application for approval of a therapy to treat a rare disease or condition; and

"(B) with respect to which a review division has requested that the Advisory Committee provide advice.

"(d) MEMBERSHIP.—

"(1) IN GENERAL.—The Advisory Committee shall consist of—

"(A) not more than 15 members appointed by the Secretary in accordance with paragraph (2); and

"(B) the nonvoting ex officio members under paragraph (3).

"(2) APPOINTED MEMBERS.—

"(A) SPECIAL GOVERNMENT EMPLOYEES.—Members of the Advisory Committee appointed pursuant to paragraph (1)(A) shall serve as special Government employees (as defined in section 202(a) of title 18, United States Code).

"(B) ELIGIBILITY.—To be eligible for appointment pursuant to paragraph (1)(A), an individual shall—

"(i) be eligible to serve as special Government employee (as defined in section 202(a) of title 18, United States Code); and

''(ii) have expertise in the fields of public policy, law, regulatory policy, economics, patient-focused product development, or patient advocacy.

''(C) COMPOSITION.—Of the members of the Advisory Committee appointed pursuant to paragraph (1)(A)—

''(i) up to 10 shall be selected from among experts in the disciplines relevant to the activities of the Intercenter Institute on Rare Diseases and Conditions, to include at least one expert in each of—

''(I) rare disease product development;

''(II) conducting clinical trials with respect to rare diseases and conditions, including with respect to very small patient populations;

''(III) rare disease and condition natural history and related studies;

''(IV) health economics pertaining to the development of medical products for rare diseases or conditions;

"(V) manufacturing and related needs associated with medical products for rare diseases or conditions; and

"(VI) patient experience data collection; and

"(ii) up to 5 shall be selected from the public, to include—

"(I) at least 4 individuals who are representatives of the rare disease patient community;

"(II) at least one individual who is directly impacted by a rare disease or condition; and

"(III) at least one person who serves as a family caregiver to a person diagnosed with a rare disease or condition.

"(3) NONVOTING EX OFFICIO MEMBERS.—The nonvoting ex officio members of the Advisory Committee under paragraph (1)(B) shall consist of the following:

"(A) The Secretary (or the Secretary's designee).

"(B) The Director of the Intercenter Institute on Rare Diseases and Conditions.

"(C) The Director of the Center for Biologics Evaluation and Research (or the Director's designee).

"(D) The Director of the Center for Drug Evaluation and Research (or the Director's designee).

"(E) The Director of the Center for Devices and Radiological Health (or the Director's designee).

"(F) The Director of the National Center for the Advancing Translational Sciences of the National Institutes of Health (or the Director's designee).

"(G) The Administrator of the Centers for Medicare & Medicaid Services (or the Administrator's designee).

"(H) Any additional officers or employees of the Department of Health and Human Services as the Secretary determines necessary for the Advisory Committee to effectively carry out its functions.

''(4) CHAIR.—The Chair of the Advisory Committee shall be the Director of the Intercenter Institute for Rare Diseases and Conditions.

''(5) TERMS.—

''(A) MEMBERS.—

''(i) IN GENERAL.—The term of a member of the Advisory Committee appointed pursuant to paragraph (1)(A) shall be 4 years, except that any member appointed to fill a vacancy in an unexpired term shall be appointed for the remainder of that term.

''(ii) CONTINUED SERVICE.—A member appointed pursuant to paragraph (1)(A) may continue serving as a member of the Advisory Committee for up to 180 days after the expiration of that member's term if a successor has not been appointed.

''(B) REAPPOINTMENT.—A member of the Advisory Committee who has been appointed pursuant to paragraph (1)(A) for a term of 4 years may not be reappointed to serve as a member of the Advisory Committee before the date that is 2 years after the date of expiration of that member's term.

''(e) QUORUM.—A majority of the appointed members of the Advisory Committee shall constitute a quorum for the conduct of business.''.

**SEC. 5. GRANTS AND CONTRACTS FOR DEVELOPMENT OF DRUGS FOR RARE DISEASES AND CONDITIONS.**

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

(1) in paragraph (2), by striking ''and'' at the end; and

(2) by inserting before the period at the end '', and (4) developing practices pertaining to the chemistry, manufacturing, regulatory approval of, and controls of individualized therapies or therapies to treat very small populations''.

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

(1) identify the regulatory science and related challenges and needs associated with developing individualized therapies or therapies to treat very small patient populations; and

(2) support research to address such challenges.

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