

117TH CONGRESS
1ST SESSION

H. R. 1730

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.

IN THE HOUSE OF REPRESENTATIVES

MARCH 10, 2021

Mr. BILIRAKIS (for himself and Mr. BUTTERFIELD) introduced the following bill; which was referred to the Committee on Energy and Commerce

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.

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