

117TH CONGRESS
2D SESSION

S. 4071

To amend the Federal Food, Drug, and Cosmetic Act to improve the treatment of rare diseases and conditions, and for other purposes.

IN THE SENATE OF THE UNITED STATES

APRIL 7, 2022

Mr. CASEY (for himself and Mr. SCOTT of South Carolina) 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 to improve the treatment of rare diseases and conditions, 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 “Helping Experts Accel-
5 erate Rare Treatments Act of 2022”.

6 **SEC. 2. IMPROVING THE TREATMENT OF RARE DISEASES**
7 **AND CONDITIONS.**

8 (a) ANNUAL REPORT ON ORPHAN DRUG PRO-
9 GRAM.—Subchapter B of chapter V of the Federal Food,

1 Drug, and Cosmetic Act (21 U.S.C. 360aa et seq.) is
 2 amended by adding at the end the following new section:

3 **“SEC. 529B. ANNUAL REPORT ON ORPHAN DRUG PROGRAM.**

4 “(a) IN GENERAL.—Not later than 1 year after the
 5 date of enactment of the Helping Experts Accelerate Rare
 6 Treatments Act of 2022, and not less frequently than an-
 7 nually thereafter, the Secretary shall submit to Congress
 8 a report summarizing the activities of the Food and Drug
 9 Administration related to designating drugs under section
 10 526 for a rare disease or condition and approving such
 11 drugs under section 505 of this Act or licensing such
 12 drugs under section 351 of the Public Health Service Act,
 13 including—

14 “(1) the number of applications for such drugs
 15 under section 505 of this Act and section 351 of the
 16 Public Health Service Act received by the Food and
 17 Drug Administration, the number of such applica-
 18 tions accepted for filing, the number of such applica-
 19 tions rejected for filing, and the numbers of such ap-
 20 plications pending, approved, and disapproved by the
 21 Food and Drug Administration, arrayed by the re-
 22 view division assigned to the application;

23 “(2) the size of the affected population in the
 24 United States of each disease or condition addressed
 25 by an application described in paragraph (1), as-

1 sessed taking into consideration the documentation
2 required by section 316.20 of title 21, Code of Fed-
3 eral Regulations (or any successor regulation) and
4 other relevant information available to the Secretary;
5 and

6 “(3) the extent of consultation with stake-
7 holders and external experts pursuant to section
8 569.

9 “(b) MANNER OF SUBMISSION.—The information re-
10 quired to be reported under this section may be submitted
11 as part of another report to Congress related to the regu-
12 lation of drugs.

13 “(c) PUBLIC AVAILABILITY.—The Secretary shall
14 make each report under subsection (a) available to the
15 public, including by posting the report on the website of
16 the Food and Drug Administration.”.

17 (b) STUDY ON EUROPEAN UNION SAFETY AND EFFI-
18 CACY REVIEWS OF DRUGS FOR RARE DISEASES AND CON-
19 DITIONS.—

20 (1) IN GENERAL.—Not later than 1 year after
21 the date of enactment of this Act, the Secretary of
22 Health and Human Services shall seek to enter into
23 an agreement with the National Academies of
24 Sciences, Engineering, and Medicine (referred to in
25 this section as the “National Academies”) to exam-

1 ine and report on European Union safety and effi-
2 cacy reviews of drugs for rare diseases and condi-
3 tions, the use and sufficiency of existing mechanisms
4 and tools of the Food and Drug Administration in
5 ensuring that patient and physician perspectives are
6 considered throughout such reviews, and opportuni-
7 ties to improve such reviews in the United States.

8 (2) CONTENT.—The report developed under
9 paragraph (1) shall—

10 (A) assess and evaluate, with respect to
11 drugs for rare diseases and conditions—

12 (i) any flexibilities, authorities, or
13 mechanisms available in the European
14 Union;

15 (ii) consideration and use by the Eu-
16 ropean Medicines Agency of supplemental
17 data submitted during the orphan drug ap-
18 plication review process, including data as-
19 sociated with open label extension studies
20 and expanded access programs; and

21 (iii) each formal or informal process
22 that the Food and Drug Administration
23 has utilized to gather external expertise on
24 orphan drug applications, separately con-
25 sidering orphan drugs for diseases or con-

1 ditions that affect fewer than 20,000 indi-
2 viduals in the United States, compared to
3 orphan drugs for other rare diseases or
4 conditions; and

5 (B) provide recommendations for changes
6 to the processes and authorities of the Food
7 and Drug Administration to facilitate develop-
8 ment of, and access to, orphan drugs, which
9 may include—

10 (i) new tools or mechanisms to im-
11 prove efforts and initiatives of the Food
12 and Drug Administration to collect and
13 consider external expertise on orphan drug
14 applications, separately considering treat-
15 ments for diseases or conditions that affect
16 fewer than 20,000 individuals in the
17 United States, including with respect to
18 processes related to application review, in-
19 cluding structured benefit-risk assess-
20 ments, advisory committee deliberations,
21 and postapproval safety monitoring; and

22 (ii) in the case of a need for input
23 from external experts where there are lim-
24 ited clinical and research experts available
25 with respect to a rare disease or condition,

1 setting forth alternative processes to ad-
2 dress or resolve any conflicts of interest
3 that would otherwise impede inclusion of
4 input from such external experts.

5 (3) INPUT.—In conducting the study and devel-
6 oping the report under paragraph (2), the National
7 Academies shall—

8 (A) consider input from the Department of
9 Health and Human Services, and any other
10 Federal agencies, as appropriate; and

11 (B) consult with relevant stakeholders,
12 which may include conducting public meetings
13 and other forms of engagement, as appropriate,
14 with patient groups, health care providers, med-
15 ical professional societies, public health experts,
16 medical product manufacturers, and other enti-
17 ties with relevant experience.

18 (4) DEFINITIONS.—In this subsection—

19 (A) the term “orphan drug” means a drug
20 designated as a drug for a rare disease or con-
21 dition under section 526 of the Federal Food,
22 Drug, and Cosmetic Act (21 U.S.C. 360bb);
23 and

24 (B) the term “rare disease or condition”
25 has the meaning given such term in section 526

1 of the Federal Food, Drug, and Cosmetic Act
2 (21 U.S.C. 360bb).

3 (c) REVIEW PROCESS.—

4 (1) CONSULTATION WITH STAKEHOLDERS.—

5 Section 569(a)(1) of the Federal Food, Drug, and
6 Cosmetic Act (21 U.S.C. 360bbb–8(a)(1)) is amend-
7 ed—

8 (A) by striking “at a time” and inserting
9 “at any time”;

10 (B) by striking “Consistent with sections”
11 and inserting the following:

12 “(A) IN GENERAL.—Consistent with sec-
13 tions”; and

14 (C) by adding at the end the following:

15 “(B) CONSULTATION WITH PATIENTS AND
16 PATIENT GROUPS.—

17 “(i) IN GENERAL.—The Secretary
18 may, as appropriate, consult with patients
19 and relevant patient groups impacted by
20 the rare disease or condition, together with
21 at least one expert included on the list
22 under paragraph (2)(A) and selected by
23 such groups—

24 “(I) during the review process of
25 an application for a new drug or bio-

1 logical product for a rare disease or
2 condition or a drug or biological prod-
3 uct that is genetically targeted; and

4 “(II) as applicable, during meet-
5 ings between the Food and Drug Ad-
6 ministration and sponsors prior to the
7 submission of an application for a
8 drug described in subclause (I).

9 “(ii) CONFLICTS OF INTEREST.—For
10 purposes of clause (i), to be eligible for
11 consultation pursuant to clause (i), pa-
12 tients and relevant patient groups may not
13 have any financial interest in the applica-
14 ble drug or biological product, and external
15 experts shall be in compliance with applica-
16 ble law, including section 208 of title 18,
17 United States Code.

18 “(C) CONSULTATION WITH DISPROPOR-
19 TIONATELY AFFECTED COMMUNITIES.—To the
20 extent an application for a new drug or biologi-
21 cal product relates to a rare disease or condi-
22 tion that disproportionately affects communities
23 of color or other historically underrepresented
24 and vulnerable populations, the Secretary is en-
25 couraged to consult with patients of that sub-

1 population, or one or more patient groups that
2 represent that subpopulation.”.

3 (2) REQUIRING APPROPRIATE EXPERT CON-
4 SULTATION.—Section 569(a)(2) of the Federal
5 Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb–
6 8(a)(2)) is amended—

7 (A) in subparagraph (A), by striking the
8 second sentence; and

9 (B) by striking subparagraph (B) and in-
10 serting the following:

11 “(B) CONSULTATION.—With respect to
12 any application under section 505 of this Act or
13 section 351 of the Public Health Service Act for
14 a drug designated under section 526 for a rare
15 disease or condition or a drug or biological
16 product that is genetically targeted, the Sec-
17 retary may, as appropriate, consult—

18 “(i) with an expert with respect to the
19 disease or condition referenced in the ap-
20 plication who appears on the list described
21 in subparagraph (A); or

22 “(ii) if no such expert is available, in-
23 cluding because of conflicts of interest,
24 with an expert on the list described in sub-

paragraph (A) in the science of small population studies.

“(C) AVAILABILITY AT MEETINGS.—In connection with each drug product advisory committee meeting concerning a drug or biological product for a rare disease or condition, the Secretary may, as appropriate—

“(i) include—

“(I) an expert in the rare disease or condition; or

“(II) if no such expert is available, including because of conflicts of interest, an expert in the science of small population studies; and

“(ii) invite at least one disease or condition expert identified by the relevant patient groups to participate as a nonvoting member of the advisory committee.”.

(3) ADDITIONAL TOPIC FOR CONSULTATION.—

Section 569(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb–8(b)) is amended—

(A) in paragraph (6), by striking “; and” and inserting “;”;

(B) in paragraph (7), by striking the period and inserting “; and”; and

- 1 (C) by adding at the end the following:
- 2 “(8) the science of small population studies.”.

