

117TH CONGRESS
2D SESSION

H. R. 6888

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

IN THE HOUSE OF REPRESENTATIVES

MARCH 1, 2022

Mr. TONKO (for himself and Mr. MCKINLEY) 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 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 the end of each
5 of the 4 years following the date of enactment of the Help-
6 ing Experts Accelerate Rare Treatments Act of 2022, the
7 Secretary shall submit to the Congress a report summa-
8 rizing the activities of the Food and Drug Administration
9 related to designating drugs under section 526 for a rare
10 disease or condition and approving such drugs under sec-
11 tion 505 of this Act or licensing such drugs under section
12 351 of the Public Health Service Act, including—

13 “(1) the number of applications for such drugs
14 under section 505 of this Act and section 351 of the
15 Public Health Service Act received by the Food and
16 Drug Administration, the number of such applica-
17 tions accepted and rejected for filing, and the num-
18 ber of such applications pending, approved, and dis-
19 approved by the Food and Drug Administration,
20 arrayed by the review division assigned to the appli-
21 cation; and

22 “(2) assess the extent to which the Food and
23 Drug Administration is consulting with external ex-
24 perts pursuant to section 569(a)(2) on topics per-
25 taining to drugs for a rare disease or condition, in-

1 including how and when any such consultation is oc-
2 ccurring.

3 “(b) PUBLIC AVAILABILITY.—The Secretary shall
4 make each report under subsection (a) available to the
5 public, including by posting the report on the website of
6 the Food and Drug Administration.

7 “(c) DEFINITION.—In this section, the term ‘rare
8 disease or condition’ means a disease or condition affect-
9 ing fewer than 200,000 persons in the United States.”.

10 (b) STUDY ON EUROPEAN UNION SAFETY AND EFFI-
11 CACY REVIEWS OF DRUGS FOR RARE DISEASES AND CON-
12 DITIONS.—

13 (1) IN GENERAL.—The Comptroller General of
14 the United States shall enter into a contract with an
15 appropriate entity to conduct a study on the Euro-
16 pean Union process for evaluating the safety and ef-
17 ficacy of drugs for rare diseases or conditions, in-
18 cluding—

19 (A) any flexibilities, authorities, or mecha-
20 nisms available in the European Union specific
21 to rare diseases or conditions; and

22 (B) consideration and use of supplemental
23 data submitted during the review process, in-
24 cluding data associated with open label exten-

1 sion studies and expanded access programs spe-
2 cific to rare diseases or conditions.

3 (2) CONSULTATION.—The contract under para-
4 graph (1) shall provide for consultation with relevant
5 stakeholders, including—

6 (A) rare disease or condition patients; and

7 (B) patient groups that—

8 (i) represent rare disease or condition
9 patients; and

10 (ii) have international patient out-
11 reach.

12 (3) REPORT.—The contract under paragraph
13 (1) shall provide for—

14 (A) not later than 2 years after the date
15 of enactment of this Act—

16 (i) the completion of the study under
17 paragraph (1); and

18 (ii) the submission of a report on the
19 results of such study to the Congress; and

20 (B) the inclusion in the report under sub-
21 paragraph (A)(ii) of recommendations for
22 changes to the processes and authorities of the
23 Food and Drug Administration to facilitate de-
24 velopment of, and access to, treatments for rare
25 diseases or conditions.

1 (4) PUBLIC AVAILABILITY.—The contract under
2 paragraph (1) shall provide for the appropriate enti-
3 ty referred to in paragraph (1) to make the report
4 under paragraph (3) available to the public, includ-
5 ing by posting the report on the website of the ap-
6 propriate entity.

7 (c) PUBLIC MEETING.—

8 (1) IN GENERAL.—Not later than December 31,
9 2023, the Secretary of Health and Human Services,
10 acting through the Commissioner of Food and
11 Drugs, shall convene one or more public meetings to
12 solicit input from stakeholders regarding the ap-
13 proaches described in paragraph (2).

14 (2) APPROACHES.—The public meeting or
15 meetings under paragraph (1) shall address ap-
16 proaches to increasing and improving engagement
17 with rare disease or condition patients, groups rep-
18 resenting such patients, rare disease or condition ex-
19 perts, and experts on small population studies, in
20 order to improve the understanding with respect to
21 rare diseases or conditions of—

22 (A) patient burden;

23 (B) treatment options; and

24 (C) side effects of treatments, including—

1 (i) comparing the side effects of treat-
2 ments; and

3 (ii) understanding the risks of side ef-
4 fects relative to the health status of the pa-
5 tient and the progression of the disease or
6 condition.

7 (3) PUBLIC DOCKET.—The Secretary of Health
8 and Human Services shall establish a public docket
9 to receive written comments related to the ap-
10 proaches addressed during each public meeting
11 under paragraph (1). Such public docket shall re-
12 main open for 60 days following the date of each
13 such public meeting.

14 (4) REPORTS.—Not later than 180 days after
15 each public meeting under paragraph (1), the Com-
16 missioner of Food and Drugs shall develop and pub-
17 lish on the website of the Food and Drug Adminis-
18 tration a report on—

19 (A) the approaches discussed at the public
20 meeting; and

21 (B) any related recommendations.

22 (d) CONSULTATION ON THE SCIENCE OF SMALL
23 POPULATION STUDIES.—Section 569(a)(2) of the Federal
24 Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb–8(b))
25 is amended by adding at the end the following:

1 “(C) SMALL POPULATION STUDIES.—The
2 external experts on the list maintained pursuant
3 to subparagraph (A) may include experts on the
4 science of small population studies.”.

5 (e) STUDY ON SUFFICIENCY AND USE OF FDA
6 MECHANISMS FOR INCORPORATING THE PATIENT AND
7 CLINICIAN PERSPECTIVE IN FDA PROCESSES RELATED
8 TO APPLICATIONS CONCERNING DRUGS FOR RARE DIS-
9 EASES OR CONDITIONS.—

10 (1) IN GENERAL.—The Comptroller General of
11 the United States shall conduct a study on the use
12 of Food and Drug Administration mechanisms and
13 tools to ensure that patient and physician perspec-
14 tives are considered and incorporated throughout the
15 processes of the Food and Drug Administration—

16 (A) for approving or licensing under sec-
17 tion 505 of the Federal Food, Drug, or Cos-
18 metic Act (21 U.S.C. 355) or section 351 of the
19 Public Health Service Act (42 U.S.C. 262) a
20 drug designated as a drug for a rare disease or
21 condition under section 526 of the Federal
22 Food, Drug, and Cosmetic Act (21 U.S.C.
23 360bb); and

1 (B) in making any determination related
2 to such a drug's approval, including assessment
3 of the drug's—

- 4 (i) safety or effectiveness; or
5 (ii) postapproval safety monitoring.

6 (2) TOPICS.—The study under paragraph (1)
7 shall—

8 (A) identify and compare the processes
9 that the Food and Drug Administration has
10 formally put in place and utilized to gather ex-
11 ternal expertise (including patients, patient
12 groups, and physicians) on specific applications
13 for diseases or conditions affecting 20,000 or
14 fewer patients in the United States and specific
15 applications for diseases or conditions affecting
16 200,000 or fewer patients in the United States;

17 (B) examine tools or mechanisms to im-
18 prove efforts and initiatives of the Food and
19 Drug Administration to collect and consider
20 such external expertise with respect to applica-
21 tions for diseases or conditions affecting 20,000
22 or fewer patients in the United States com-
23 pared to applications for diseases or conditions
24 affecting 200,000 or fewer patients in the
25 United States throughout the application review

1 and approval or licensure processes, including
2 within internal benefit-risk assessments, advi-
3 sory committee processes, and postapproval
4 safety monitoring; and

5 (C) examine processes or alternatives to
6 address or resolve conflicts of interest that im-
7 pede the Food and Drug Administration in
8 gaining external expert input on rare diseases
9 or conditions with a limited set of clinical and
10 research experts.

11 (3) REPORT.—Not later than 2 years after the
12 date of enactment of this Act, the Comptroller Gen-
13 eral of the United States shall—

14 (A) complete the study under paragraph
15 (1);

16 (B) submit a report on the results of such
17 study to the Congress; and

18 (C) include in such report recommenda-
19 tions, if appropriate, for changes to the proc-
20 esses and authorities of the Food and Drug Ad-
21 ministration to improve the collection and con-
22 sideration of external expert opinions of pa-
23 tients, patient groups, and physicians with ex-
24 pertise in rare diseases or conditions, including
25 any specific recommendations for diseases or

1 conditions affecting 20,000 or fewer patients in
2 the United States.

3 (f) DEFINITION.—In this section, the term “rare dis-
4 ease or condition” means a disease or condition affecting
5 fewer than 200,000 persons in the United States.

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