

117TH CONGRESS  
1ST SESSION

# H. R. 1184

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

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## IN THE HOUSE OF REPRESENTATIVES

FEBRUARY 18, 2021

Mr. TONKO (for himself, Mr. MCKINLEY, Mr. FITZPATRICK, and Mr. SAN NICOLAS) introduced the following bill; which was referred to the Committee on Energy and Commerce

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## 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 2021”.

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 2021, and not less than annually there-  
7 after, the Secretary shall submit to the Congress a report  
8 summarizing the activities of the Food and Drug Adminis-  
9 tration for 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 sections 505 of this Act and section 351 of  
15 the Public Health Service Act received by the Food  
16 and Drug Administration, the number of such appli-  
17 cations considered by each Food and Drug Adminis-  
18 tration division, and the number of such applications  
19 pending and approved;

20               “(2) the number of applications described in  
21 paragraph (1) disaggregated by—

22                       “(A) the disease or condition to be ad-  
23 dressed; and

24                       “(B) the target subpopulations identified  
25 in such applications;

1           “(3) the prevalence in the United States of each  
2 disease or condition addressed by an application de-  
3 scribed in paragraph (1);

4           “(4) integration of Food and Drug Administra-  
5 tion staff with expertise in rare diseases and condi-  
6 tions in each review of an application described in  
7 paragraph (1);

8           “(5) identification of all external experts with  
9 expertise in rare diseases and conditions who are  
10 consulted in connection with, or who otherwise par-  
11 ticipate in, each review of an application described in  
12 paragraph (1); and

13           “(6) the status of each application described in  
14 paragraph (1).

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

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

22           (1) IN GENERAL.—The Comptroller General of  
23 the United States shall conduct a study on Euro-  
24 pean Union safety and efficacy reviews of drugs for  
25 rare diseases and conditions, including—

1 (A) any differential prevalence-based mech-  
2 anisms; and

3 (B) consideration and use of supplemental  
4 data submitted during the review process, in-  
5 cluding data associated with open label exten-  
6 sion studies and expanded access programs.

7 (2) REPORT.—Not later than 1 year after the  
8 date of enactment of this Act, the Comptroller Gen-  
9 eral shall—

10 (A) complete the study under paragraph  
11 (1);

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

14 (C) include in such report recommenda-  
15 tions for changes to the processes and authori-  
16 ties of the Food and Drug Administration to fa-  
17 cilitate development of, and access to, treat-  
18 ments for rare diseases and conditions.

19 (3) PUBLIC AVAILABILITY.—The Comptroller  
20 General of the United States shall make each report  
21 under paragraph (2) available to the public, includ-  
22 ing by posting the report on the website of the Gov-  
23 ernment Accountability Office.

1 (c) REVIEW PROCESS.—Subparagraph (A) of section  
2 569(a)(2) of the Federal Food, Drug, and Cosmetic Act  
3 (21 U.S.C. 360bbb–8(a)(2)) is amended to read as follows:

4 “(A) IN GENERAL.—

5 “(i) LIST.—The Secretary shall de-  
6 velop and maintain a list of external ex-  
7 perts who, because of their special exper-  
8 tise, are qualified to provide advice on rare  
9 disease and condition issues, including top-  
10 ics described in subsection (b).

11 “(ii) AVAILABILITY AT MEETINGS.—  
12 The Secretary shall ensure availability, for  
13 consultation purposes, of an external ex-  
14 pert on rare diseases, as described in sub-  
15 paragraph (B), in connection with each  
16 drug product advisory committee meeting  
17 concerning a drug or biological product for  
18 a rare disease or condition.

19 “(iii) REVIEW OF APPLICATIONS.—  
20 The Secretary shall ensure that each re-  
21 view of an application submitted under sec-  
22 tion 505 of this Act or section 351 of the  
23 Public Health Service Act for an indication  
24 associated with a rare disease or condition  
25 includes Food and Drug Administration

1 staff with expertise on rare diseases and  
2 conditions as an integral part of the review  
3 team.”.

4 (d) RISK EVALUATION AND MITIGATION STRATEGY  
5 ELEMENTS.—Subsection (a) of section 505–1 of the Fed-  
6 eral Food, Drug, and Cosmetic Act (21 U.S.C. 355–1) is  
7 amended by adding at the end the following new para-  
8 graph:

9 “(5) ORPHAN DRUGS.—In making any deter-  
10 mination regarding the inclusion, in the risk evalua-  
11 tion and mitigation strategy for a drug that has  
12 been designated as a drug for a rare disease or con-  
13 dition pursuant to section 526, of an element that  
14 requires patient action, restrictions on use of the  
15 drug, or patient participation, the Secretary shall  
16 consult with patients impacted by the rare disease or  
17 condition in considering the burden of participation  
18 and likely patient compliance.”.

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