

116TH CONGRESS 2D SESSION

H. R. 7567

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

July 9, 2020

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 2020".
- 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 2020, 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

rare diseases and conditions, including—

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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 (21 U.S.C. 360bbb–8(a)(2)) is amended to read as follows: 3 "(A) IN GENERAL.— 4 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

staff with expertise on rare diseases and conditions as an integral part of the review team.".

4 (d) RISK EVALUATION AND MITIGATION STRATEGY
5 ELEMENTS.—Subsection (a) of section 505–1 of the Fed6 eral Food, Drug, and Cosmetic Act (21 U.S.C. 355–1) is
7 amended by adding at the end the following new para8 graph:

"(5) ORPHAN DRUGS.—In making any determination regarding the inclusion, in the risk evaluation and mitigation strategy for a drug that has been designated as a drug for a rare disease or condition pursuant to section 526, of an element that requires patient action, restrictions on use of the drug, or patient participation, the Secretary shall consult with patients impacted by the rare disease or condition in considering the burden of participation and likely patient compliance.".

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