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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.

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.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Helping Experts Accelerate Rare Treatments Act of 2021''.

SEC. 2. IMPROVING THE TREATMENT OF RARE DISEASES AND CONDITIONS.

(a) ANNUAL REPORT ON ORPHAN DRUG PROGRAM.—Subchapter B of chapter V of the Federal Food,

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

"SEC. 529B. ANNUAL REPORT ON ORPHAN DRUG PROGRAM.

"(a) IN GENERAL.—Not later than 1 year after the date of enactment of the Helping Experts Accelerate Rare Treatments Act of 2021, and not less than annually thereafter, the Secretary shall submit to the Congress a report summarizing the activities of the Food and Drug Administration for designating drugs under section 526 for a rare disease or condition and approving such drugs under section 505 of this Act or licensing such drugs under section 351 of the Public Health Service Act, including—

"(1) the number of applications for such drugs under sections 505 of this Act and section 351 of the Public Health Service Act received by the Food and Drug Administration, the number of such applications considered by each Food and Drug Administration division, and the number of such applications pending and approved;

"(2) the number of applications described in paragraph (1) disaggregated by—

"(A) the disease or condition to be addressed; and

"(B) the target subpopulations identified in such applications;

''(3) the prevalence in the United States of each disease or condition addressed by an application described in paragraph (1);

''(4) integration of Food and Drug Administration staff with expertise in rare diseases and conditions in each review of an application described in paragraph (1);

''(5) identification of all external experts with expertise in rare diseases and conditions who are consulted in connection with, or who otherwise participate in, each review of an application described in paragraph (1); and

''(6) the status of each application described in paragraph (1).

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

(b) STUDY ON EUROPEAN UNION SAFETY AND EFFICACY REVIEWS OF DRUGS FOR RARE DISEASES AND CONDITIONS.—

(1) IN GENERAL.—The Comptroller General of the United States shall conduct a study on European Union safety and efficacy reviews of drugs for rare diseases and conditions, including—

(A) any differential prevalence-based mechanisms; and

(B) consideration and use of supplemental data submitted during the review process, including data associated with open label extension studies and expanded access programs.

(2) REPORT.—Not later than 1 year after the date of enactment of this Act, the Comptroller General shall—

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

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

(C) include in such report recommendations for changes to the processes and authorities of the Food and Drug Administration to facilitate development of, and access to, treatments for rare diseases and conditions.

(3) PUBLIC AVAILABILITY.—The Comptroller General of the United States shall make each report under paragraph (2) available to the public, including by posting the report on the website of the Government Accountability Office.

(c) REVIEW PROCESS.—Subparagraph (A) of section 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:

"(A) IN GENERAL.—

"(i) LIST.—The Secretary shall develop and maintain a list of external experts who, because of their special expertise, are qualified to provide advice on rare disease and condition issues, including topics described in subsection (b).

"(ii) AVAILABILITY AT MEETINGS.—The Secretary shall ensure availability, for consultation purposes, of an external expert on rare diseases, as described in subparagraph (B), in connection with each drug product advisory committee meeting concerning a drug or biological product for a rare disease or condition.

"(iii) REVIEW OF APPLICATIONS.—The Secretary shall ensure that each review of an application submitted under section 505 of this Act or section 351 of the Public Health Service Act for an indication associated with a rare disease or condition includes Food and Drug Administration

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

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

''(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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