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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

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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 2022''.

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 the end of each of the 4 years following the date of enactment of the Helping Experts Accelerate Rare Treatments Act of 2022, the Secretary shall submit to the Congress a report summarizing the activities of the Food and Drug Administration related to 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 section 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 accepted and rejected for filing, and the number of such applications pending, approved, and disapproved by the Food and Drug Administration, arrayed by the review division assigned to the application; and

"(2) assess the extent to which the Food and Drug Administration is consulting with external experts pursuant to section 569(a)(2) on topics pertaining to drugs for a rare disease or condition, in-

cluding how and when any such consultation is occurring.

''(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.

''(c) DEFINITION.—In this section, the term 'rare disease or condition' means a disease or condition affecting fewer than 200,000 persons in the United States.''.

(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 enter into a contract with an appropriate entity to conduct a study on the European Union process for evaluating the safety and efficacy of drugs for rare diseases or conditions, including—

(A) any flexibilities, authorities, or mechanisms available in the European Union specific to rare diseases or conditions; and

(B) consideration and use of supplemental data submitted during the review process, including data associated with open label exten-

sion studies and expanded access programs specific to rare diseases or conditions.

(2) CONSULTATION.—The contract under paragraph (1) shall provide for consultation with relevant stakeholders, including—

(A) rare disease or condition patients; and

(B) patient groups that—

(i) represent rare disease or condition patients; and

(ii) have international patient outreach.

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

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

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

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

(B) the inclusion in the report under subparagraph (A)(ii) of 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 or conditions.

(4) PUBLIC AVAILABILITY.—The contract under paragraph (1) shall provide for the appropriate entity referred to in paragraph (1) to make the report under paragraph (3) available to the public, including by posting the report on the website of the appropriate entity.

(c) PUBLIC MEETING.—

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

(2) APPROACHES.—The public meeting or meetings under paragraph (1) shall address approaches to increasing and improving engagement with rare disease or condition patients, groups representing such patients, rare disease or condition experts, and experts on small population studies, in order to improve the understanding with respect to rare diseases or conditions of—

(A) patient burden;

(B) treatment options; and

(C) side effects of treatments, including—

(i) comparing the side effects of treatments; and

(ii) understanding the risks of side effects relative to the health status of the patient and the progression of the disease or condition.

(3) PUBLIC DOCKET.—The Secretary of Health and Human Services shall establish a public docket to receive written comments related to the approaches addressed during each public meeting under paragraph (1). Such public docket shall remain open for 60 days following the date of each such public meeting.

(4) REPORTS.—Not later than 180 days after each public meeting under paragraph (1), the Commissioner of Food and Drugs shall develop and publish on the website of the Food and Drug Administration a report on—

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

(B) any related recommendations.

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

"(C) SMALL POPULATION STUDIES.—The external experts on the list maintained pursuant to subparagraph (A) may include experts on the science of small population studies.".

(e) STUDY ON SUFFICIENCY AND USE OF FDA MECHANISMS FOR INCORPORATING THE PATIENT AND CLINICIAN PERSPECTIVE IN FDA PROCESSES RELATED TO APPLICATIONS CONCERNING DRUGS FOR RARE DISEASES OR CONDITIONS.—

(1) IN GENERAL.—The Comptroller General of the United States shall conduct a study on the use of Food and Drug Administration mechanisms and tools to ensure that patient and physician perspectives are considered and incorporated throughout the processes of the Food and Drug Administration—

(A) for approving or licensing under section 505 of the Federal Food, Drug, or Cosmetic Act (21 U.S.C. 355) or section 351 of the Public Health Service Act (42 U.S.C. 262) a drug designated as a drug for a rare disease or condition under section 526 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb); and

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

(i) safety or effectiveness; or

(ii) postapproval safety monitoring.

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

(A) identify and compare the processes that the Food and Drug Administration has formally put in place and utilized to gather external expertise (including patients, patient groups, and physicians) on specific applications for diseases or conditions affecting 20,000 or fewer patients in the United States and specific applications for diseases or conditions affecting 200,000 or fewer patients in the United States;

(B) examine tools or mechanisms to improve efforts and initiatives of the Food and Drug Administration to collect and consider such external expertise with respect to applications for diseases or conditions affecting 20,000 or fewer patients in the United States compared to applications for diseases or conditions affecting 200,000 or fewer patients in the United States throughout the application review

and approval or licensure processes, including within internal benefit-risk assessments, advisory committee processes, and postapproval safety monitoring; and

(C) examine processes or alternatives to address or resolve conflicts of interest that impede the Food and Drug Administration in gaining external expert input on rare diseases or conditions with a limited set of clinical and research experts.

(3) REPORT.—Not later than 2 years after the date of enactment of this Act, the Comptroller General of the United States 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, if appropriate, for changes to the processes and authorities of the Food and Drug Administration to improve the collection and consideration of external expert opinions of patients, patient groups, and physicians with expertise in rare diseases or conditions, including any specific recommendations for diseases or

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

(f) DEFINITION.—In this section, the term ''rare disease or condition'' means a disease or condition affecting fewer than 200,000 persons in the United States.

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