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119TH CONGRESS
1ST SESSION

S. 822

To amend the Federal Food, Drug, and Cosmetic Act to establish a process for science-focused drug development meetings led by the Reagan-Udall Foundation for the Food and Drug Administration with respect to drugs for rare diseases and conditions, and for other purposes.

IN THE SENATE OF THE UNITED STATES

MARCH 3, 2025

Ms. KLOBUCHAR (for herself and Mr. WICKER) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

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A BILL

To amend the Federal Food, Drug, and Cosmetic Act to establish a process for science-focused drug development meetings led by the Reagan-Udall Foundation for the Food and Drug Administration with respect to drugs for 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 ''Scientific External Process for Educated Review of Therapeutics Act of 2025'' or the ''Scientific EXPERT Act of 2025''.

SEC. 2. SCIENCE-FOCUSED DRUG DEVELOPMENT MEETINGS.

The Federal Food, Drug, and Cosmetic Act (21 U.S.C. 301 et seq.) is amended by inserting after section 770 (21 U.S.C. 379dd) the following:

"SEC. 770A. SCIENCE-FOCUSED DRUG DEVELOPMENT MEETINGS.

''(a) IN GENERAL.—The Secretary shall develop and implement a process for Foundation led, science-focused drug development meetings to provide an opportunity for academic researchers and medical experts, drug sponsors, scientific organizations, and patient organizations to—

''(1) discuss science-related challenges impacting the development of drugs for rare diseases and conditions;

''(2) identify scientific approaches and opportunities to facilitate the development, review, and approval of such drugs; and

''(3) align on novel approaches for the development of drugs for particular diseases, including appropriate clinical trial designs and metrics, manufacturing standards, patient populations, clinical endpoints, the use of biomarkers as surrogate endpoints, and natural history as a control, to advance treatment options to address unmet medical needs.

''(b) ARRANGEMENT.—

''(1) QUALIFIED THIRD PARTY CONVENOR.—The Secretary shall enter into an arrangement with the Foundation under which the Foundation agrees to convene EL–SFDD meetings regarding drugs for rare diseases or conditions in accordance with this section.

''(2) MINIMUM NUMBER OF MEETINGS.—The Foundation shall convene no fewer than 4 EL–SFDD meetings each year, with each such meeting focused on addressing a different rare disease or condition or a different group of rare diseases and conditions.

''(3) STEERING COMMITTEE.—

''(A) IN GENERAL.—The Foundation shall establish and maintain a permanent steering committee, to be known as the Science-Focused Drug Development Multistakeholder Steering Committee, to advise the Foundation on implementation of this section, including by—

''(i) establishing a process by which licensed and board-certified medical clinicians and academics with expertise in a rare disease or condition, sponsors of drugs for a rare disease or condition, sci-

entific organizations, rare disease or condition patient organizations, and other entities can provide suggested meeting topics to the Foundation;

''(ii) reviewing such suggested meeting topics for EL–SFDD meetings; and

''(iii) based on the criteria under subparagraph (B), recommending to the Foundation topics for EL–SFDD meetings.

''(B) CRITERIA FOR MEETINGS.—In formulating recommendations under subparagraph (A), the Foundation shall consider—

''(i) unmet therapeutic needs;

''(ii) the size of the patient population of the rare disease or condition;

''(iii) whether there were or are multiple products in development to prevent or treat the rare disease or condition involved;

''(iv) whether there is a need for increased regulatory flexibility to facilitate the development of products;

''(v) whether the rare disease or condition involved would benefit from clarity and alignment on drug development ques-

tions (such as clinical trial design, natural history as a control, appropriate clinical endpoints, biomarkers that may serve as surrogate endpoints, and other approaches) to expedite drug development for such disease or condition; and

''(vi) whether the discussions about such rare disease or condition may have broader impact on other rare diseases and conditions.

''(C) MEMBERSHIP.—The members of the Steering Committee shall be subject to all relevant conflict of interest policies of the Foundation, and shall include—

''(i) representatives of the Center for Drug Evaluation and Research, the Center for Biologics Evaluation and Research, and the Center for Devices and Radiological Health;

''(ii) academic and licensed and board-certified medical clinicians;

''(iii) patient representatives; and

''(iv) industry representatives engaged in the development of drugs for rare diseases and conditions.

''(4) PLANNING PROCESS.—In planning an EL–SFDD meeting under this section, the Foundation, in consultation with the stakeholders listed in paragraph (5), shall develop—

''(A) a list of the specific objectives of the meeting related to key drug development issues for the rare disease or condition, or group of rare diseases and conditions, with a goal of expediting drug development;

''(B) a proposed agenda for the meeting; and

''(C) a list of medical experts, drug sponsors, scientific organizations, patient organizations, and other entities to be invited to participate in the meeting.

''(5) AGENCY AND STAKEHOLDER ENGAGEMENT.—Throughout the process of planning an EL–SFDD meeting, the Foundation shall consult with—

''(A) appropriate staff of the Food and Drug Administration;

''(B) the Steering Committee;

''(C) industry representatives engaged in the development of products for rare diseases and conditions to be discussed at such EL–SFDD meeting;

"(D) patient representatives of rare diseases and conditions under discussion in such EL–SFDD meeting;

"(E) academics or board-certified and licensed medical clinicians with expertise in treating a rare disease or condition; and

"(F) other appropriate stakeholders.

"(6) POST-MEETING REPORTS.—

"(A) IN GENERAL.—Not later than 180 days after an EL–SFDD meeting, the Foundation shall make publicly available on the website of the Food and Drug Administration—

"(i) a transcript and recording of the meeting; and

"(ii) in consultation with the stakeholders listed in paragraph (5), a summary analysis of the input received during the meeting that is relevant to approval or licensing of drugs for the rare disease or condition involved.

"(B) CONTENTS.—Each publication under subparagraph (A) shall include a clear identification of—

"(i) areas of consensus;

''(ii) areas where additional clarification or information is needed to reach consensus; and

''(iii) next steps agreed upon with the Food and Drug Administration.

''(c) REPRESENTATIVES OF FDA REVIEW DIVISIONS.—The Secretary shall require appropriate representatives of the review divisions of the Food and Drug Administration to participate in each EL–SFDD meeting under this section.

''(d) RULES OF CONSTRUCTION.—Nothing in this section shall be construed—

''(1) to prevent other third-party organizations from organizing similarly structured EL–SFDD-like meetings to discuss challenges in rare disease drug development;

''(2) to require the Food and Drug Administration to participate in additional meetings described in paragraph (1);

''(3) to alter the protections offered by laws, regulations, or policies governing disclosure of confidential commercial or trade secret information and any other information exempt from disclosure pursuant to section 552(b) of title 5, United States Code;

''(4) to limit the ability of the Secretary to consult with individuals and organizations;

''(5) to create a legal right for consultation on any matter or require the Secretary to meet with any particular expert or stakeholder;

''(6) to alter agreed-upon goals and procedures identified in the letters described in section 1001(b) of the FDA User Fee Reauthorization Act of 2022; or

''(7) to increase the number of review cycles for drugs.

''(e) DEFINITIONS.—In this section:

''(1) The term 'EL–SFDD meeting' means an externally led, science-focused drug development meeting.

''(2) The term 'rare disease or condition' has the meaning given such term in section 526.

''(3) The term 'Steering Committee' means the Science-Focused Drug Development Multistakeholder Steering Committee established under subsection (b)(3).

''(f) AUTHORIZATION OF APPROPRIATIONS.—

''(1) IN GENERAL.—To carry out this section, there is authorized to be appropriated $1,000,000 for each of fiscal years 2025 through 2029.

''(2) RULE OF CONSTRUCTION.—Nothing in this section shall be construed to prohibit the Foundation from soliciting or accepting funds pursuant to section 770(i) for the purposes of planning or operating an EL–SFDD meeting authorized by this section.

"SEC. 770B. REQUIRED ACTIONS FOLLOWING EL–SFDD MEETINGS.

''(a) INCORPORATION OF INPUT INTO RISK-BENEFIT ASSESSMENTS.—In approving or licensing a drug under subsection (c) or (j) of section 505 of this Act or subsection (a) or (k) of section 351 of the Public Health Service Act, the Secretary shall make public—

''(1) a statement of whether any EL–SFDD meeting under section 770A was held that was relevant to such approval or licensure; and

''(2) if a meeting described in paragraph (1) was held, a description of how the Secretary incorporated input from such meeting in the risk-benefit assessment described in section 505(d).

''(b) ANNUAL REPORT.—On an annual basis, the Secretary shall submit a report to Congress summarizing—

''(1) the number and topics of EL–SFDD meetings held during the reporting period;

''(2) the extent of participation in such meetings from the review divisions of the Food and Drug Administration;

''(3) the impact of EL–SFDD meetings on the workload and resources of the Food and Drug Administration; and

''(4) an assessment of how the input received during such meetings was used in—

''(A) deliberations throughout the drug development lifecycle;

''(B) regulatory decision-making; and

''(C) formulating recommendations for future meetings.

''(c) DEFINITION.—In this section, the term 'EL–SFDD meeting' has the meaning given to that term in section 770A.

''(d) AUTHORIZATION OF APPROPRIATIONS.—To carry out this section, there is authorized to be appropriated $1,000,000 for each of fiscal years 2025 through 2029.''.

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