Hatch

AMENDMENT NO. 1 Calendar No._____

Purpose: To expand patient access to experimental treatments in clinical trials, and for other purposes.

IN THE SENATE OF THE UNITED STATES—115th Cong., 1st Sess.

S. 934

To amend the Federal Food, Drug, and Cosmetic Act to revise and extend the user-fee programs for prescription drugs, medical devices, generic drugs, and biosimilar biological products, and for other purposes.

Referred to the Committee on _____ and ordered to be printed

Ordered to lie on the table and to be printed

AMENDMENT intended to be proposed by Mr. HATCH (for himself, Mr. BENNET, Mr. BURR, and Mr. CASEY)

Viz:

At the appropriate place, insert the following:

SEC. ___. EXPANDED ACCESS.

(a) PATIENT ACCESS TO EXPERIMENTAL TREATMENTS.—

(1) PUBLIC MEETING.—

(A) IN GENERAL.—The Secretary of Health and Human Services (referred to in this section as the "Secretary"), acting through the Commissioner of Food and Drugs, in coordination with the Director of the National Institutes

of Health, and in consultation with patients, health care providers, drug sponsors, bioethicists, and other stakeholders, shall, not later than 180 days after the date of enactment of this Act, convene a public meeting to discuss clinical trial inclusion and exclusion criteria to inform the guidance under paragraph (3). The Secretary shall inform the Comptroller General of the United States of the date when the public meeting will take place.

(B) TOPICS.—The Secretary shall provide a publicly available report on the topics discussed at the meeting described in subparagraph (A) within 30 days of such meeting. Such topics shall include discussion of—

(i) the rationale for, and potential barriers for patients created by, clinical trial inclusion and exclusion criteria;

(ii) how patient populations most likely to be affected by a drug can benefit from the results of trials that employ alternative designs, as well as potential risks associated with alternative clinical trial designs;

(iii) barriers to participation in clinical trials, including—

(I) information regarding any potential risks and benefits of participation;

(II) regulatory, geographical, and socioeconomic barriers; and

(III) the impact of exclusion criteria on the enrollment in clinical trials of infants and children, pregnant and lactating women, seniors, individuals with advanced disease, and individuals with co-morbid conditions;

(iv) clinical trial designs and methods that increase enrollment of more diverse patient populations while facilitating the collection of data to support substantial evidence of safety and effectiveness; and

(v) how changes to clinical trial inclusion and exclusion criteria may impact the complexity of the clinical trial design and length of clinical trials, and potential approaches to mitigating those impacts to ensure that the ability to demonstrate safety

and effectiveness is not hindered through potential changes in eligibility criteria.

(2) REPORT.—Not later than 1 year after the Secretary issues a report on the topics discussed at the public meeting under paragraph (1)(B), the Comptroller General of the United States shall report to the Committee on Health, Education, Labor, and Pensions of the Senate and the Committee on Energy and Commerce of the House of Representatives on individual access to investigational drugs through the expanded access program under section 561(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb(b)). The report shall include—

(A) a description of actions taken by manufacturers under section 561A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb–0);

(B) consideration of whether Form FDA 3926 and the guidance document entitled "Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers", issued by the Food and Drug Administration in June 2016, has reduced application burden with respect to individuals and physicians seeking access to investigational new drugs pursu-

ant to section 561(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) and improved clarity for patients, physicians, and drug manufacturers about such process;

(C) consideration of whether the guidance or regulations released or updated under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) have improved access for individual patients who do not qualify for clinical trials of such investigational drugs, and what barriers to such access remain;

(D) an assessment of how patients and health care providers navigate different avenues to engage with the Food and Drug Administration or drug sponsors on expanded access; and

(E) an analysis of the Secretary's report under paragraph (1)(B).

(3) GUIDANCE.—

(A) IN GENERAL.—Not later than 180 days after the publication of the report under paragraph (1), the Secretary, acting through the Commissioner of Food and Drugs, shall issue one or more draft guidances regarding eligibility criteria for clinical trials. Not later than 18 months after the public comment period on

each such draft guidance ends, the Secretary shall issue a revised draft guidance or final guidance.

(B) CONTENTS.—The guidance documents described in subparagraph (A) shall address methodological approaches that a manufacturer or sponsor of an investigation of a new drug may take to—

(i) broaden eligibility criteria for clinical trials, especially with respect to drugs for the treatment of serious and life-threatening conditions or diseases for which there is an unmet medical need; and

(ii) develop eligibility criteria for, and increase trial recruitment to, clinical trials so that enrollment in such trials more accurately reflects the patients most likely to receive the drug, as applicable and as appropriate, while supporting findings of substantial evidence of safety and effectiveness.

(b) IMPROVING INSTITUTIONAL REVIEW BOARD REVIEW OF SINGLE PATIENT EXPANDED ACCESS PROTOCOL.—Not later than 1 year after the date of enactment of this Act, the Secretary, acting through the Commis-

sioner of Food and Drugs, shall issue guidance or regulations, or revise existing guidance or regulations, to streamline the institutional review board review for individual pediatric and adult patient expanded access protocol under 561(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb(b)). Such guidance or regulation may include a description of the conditions under which an institutional review board chair (or designee) may review individual patient expanded access protocol submitted under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) for a drug and how centralized institutional review boards may facilitate the use of expanded access protocols. The Secretary shall update any relevant forms associated with individual patient expanded access protocol as necessary.

(c) EXPANDED ACCESS POLICY TRANSPARENCY.—Section 561A(f) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb–0(f)) is amended—

(1) in the matter preceding paragraph (1), by striking "later" and inserting "earlier";

(2) by striking paragraph (1);

(3) by redesignating paragraph (2) as paragraph (1);

(4) in paragraph (1) as so redesignated, by striking the period at the end and inserting "; or"; and

(5) by adding at the end the following:

"(2) as applicable, 15 days after the drug receives a designation as a breakthrough therapy, fast track product, or regenerative advanced therapy under subsection (a), (b), or (g), respectively, of section 506.".