

115TH CONGRESS 1ST SESSION

S. 1048

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

IN THE SENATE OF THE UNITED STATES

May 4, 2017

Mr. Hatch (for himself, Mr. Bennet, Mr. Burr, and Mr. Casey) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To expand patient access to experimental treatments in clinical trials, 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 "Enhanced Clinical
- 5 Trial Design Act of 2017".
- 6 SEC. 2. PATIENT ACCESS TO EXPERIMENTAL TREATMENTS.
- 7 (a) Public Meeting.—
- 8 (1) IN GENERAL.—The Secretary of Health and
- 9 Human Services (referred to in this Act as the "Sec-
- 10 retary"), acting through the Commissioner of Food

- 1 and Drugs, in coordination with the Director of the 2 National Institutes of Health, and in consultation 3 with patients, health care providers, drug sponsors, 4 bioethicists, and other stakeholders, shall, not later 5 than 180 days after the date of enactment of this 6 Act, convene a public meeting to discuss clinical trial 7 inclusion and exclusion criteria to inform the guid-8 ance under subsection (c). The Secretary shall in-9 form the Comptroller General of the United States 10 of the date when the public meeting will take place.
 - (2) Topics.—The Secretary shall provide a publicly available report on the topics discussed at the meeting described in paragraph (1) within 30 days of such meeting. Such topics shall include discussion of—
 - (A) the rationale for, and potential barriers for patients created by, clinical trial inclusion and exclusion criteria;
 - (B) 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;
 - (C) barriers to participation in clinical trials, including—

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1	(i) information regarding any poten-
2	tial risks and benefits of participation;
3	(ii) regulatory, geographical, and so-
4	cioeconomic barriers; and
5	(iii) the impact of exclusion criteria on
6	the enrollment in clinical trials of infants
7	and children, pregnant and lactating
8	women, seniors, individuals with advanced
9	disease, and individuals with co-morbid
10	conditions;
11	(D) clinical trial designs and methods that
12	increase enrollment of more diverse patient pop-
13	ulations while facilitating the collection of data
14	to support substantial evidence of safety and ef-
15	fectiveness; and
16	(E) how changes to clinical trial inclusion
17	and exclusion criteria may impact the com-
18	plexity of the clinical trial design and length of
19	clinical trials, and potential approaches to miti-
20	gating those impacts to ensure that the ability
21	to demonstrate safety and effectiveness is not
22	hindered through potential changes in eligibility
23	criteria.
24	(b) Report.—Not later than 1 year after the Sec-
25	retary issues a report on the topics discussed at the public

- 1 meeting under subsection (a)(2), the Comptroller General
- 2 of the United States shall report to the Committee on
- 3 Health, Education, Labor, and Pensions of the Senate and
- 4 the Committee on Energy and Commerce of the House
- 5 of Representatives on individual access to investigational
- 6 drugs through the expanded access program under section
- 7 561(b) of the Federal Food, Drug, and Cosmetic Act (21
- 8 U.S.C. 360bbb(b)). The report shall include—
- 9 (1) a description of actions taken by manufac-
- turers under section 561A of the Federal Food,
- 11 Drug, and Cosmetic Act (21 U.S.C. 360bbb-0);
- 12 (2) consideration of whether Form FDA 3926
- and the guidance document entitled "Expanded Ac-
- cess to Investigational Drugs for Treatment Use—
- 15 Questions and Answers", issued by the Food and
- Drug Administration in June 2016, has reduced ap-
- plication burden with respect to individuals and phy-
- sicians seeking access to investigational new drugs
- pursuant to section 561(b) of the Federal Food,
- 20 Drug, and Cosmetic Act (21 U.S.C. 360bbb) and
- 21 improved clarity for patients, physicians, and drug
- 22 manufacturers about such process;
- 23 (3) consideration of whether the guidance or
- regulations released or updated under section 561 of
- 25 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;
 - (4) 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
 - (5) an analysis of the Secretary's report under subsection (a)(2).

(c) Guidance.—

- (1) In General.—Not later than 180 days after the publication of the report under subsection (a) 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.
- (2) Contents.—The guidance documents described in paragraph (1) shall address methodological approaches that a manufacturer or sponsor of an investigation of a new drug may take to—

1 (A) broaden eligibility criteria for clinical
2 trials, especially with respect to drugs for the
3 treatment of serious and life-threatening condi4 tions or diseases for which there is an unmet
5 medical need; and

(B) 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.

13 SEC. 3. IMPROVING INSTITUTIONAL REVIEW BOARD RE-

14 VIEW OF SINGLE PATIENT EXPANDED AC-

CESS PROTOCOL.

16 Not later than 1 year after the date of enactment of this Act, the Secretary of Health and Human Services (referred to in this section as the "Secretary"), acting 18 through the Commissioner of Food and Drugs, shall issue 19 guidance or regulations, or revise existing guidance or reg-21 ulations, to streamline the institutional review board re-22 view for individual pediatric and adult patient expanded 23 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

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- 1 under which an institutional review board chair (or des-
- 2 ignee) may review individual patient expanded access pro-
- 3 tocol submitted under section 505(i) of the Federal Food,
- 4 Drug, and Cosmetic Act (21 U.S.C. 355(i)) for a drug
- 5 and how centralized institutional review boards may facili-
- 6 tate the use of expanded access protocols. The Secretary
- 7 shall update any relevant forms associated with individual
- 8 patient expanded access protocol as necessary.

9 SEC. 4. EXPANDED ACCESS POLICY TRANSPARENCY.

- 10 Section 561A(f) of the Federal Food, Drug, and Cos-
- 11 metic Act (21 U.S.C. 360bbb-0(f)) is amended—
- 12 (1) in the matter preceding paragraph (1), by
- striking "later" and inserting "earlier";
- 14 (2) by striking paragraph (1);
- 15 (3) by redesignating paragraph (2) as para-
- 16 graph (1);
- 17 (4) in paragraph (1) as so redesignated, by
- striking the period at the end and inserting "; or";
- 19 and
- 20 (5) by adding at the end the following:
- 21 "(2) as applicable, 15 days after the drug re-
- ceives a designation as a breakthrough therapy, fast
- track product, or regenerative advanced therapy

- 1 under subsection (a), (b), or (g), respectively, of sec-
- 2 tion 506.".

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