

115TH CONGRESS  
1ST SESSION

# S. 1048

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

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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

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## 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.

11 (2) TOPICS.—The Secretary shall provide a  
12 publicly available report on the topics discussed at  
13 the meeting described in paragraph (1) within 30  
14 days of such meeting. Such topics shall include dis-  
15 cussion of—

16 (A) the rationale for, and potential barriers  
17 for patients created by, clinical trial inclusion  
18 and exclusion criteria;

19 (B) how patient populations most likely to  
20 be affected by a drug can benefit from the re-  
21 sults of trials that employ alternative designs,  
22 as well as potential risks associated with alter-  
23 native clinical trial designs;

24 (C) barriers to participation in clinical  
25 trials, including—

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-  
10 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  
13 and the guidance document entitled “Expanded Ac-  
14 cess to Investigational Drugs for Treatment Use—  
15 Questions and Answers”, issued by the Food and  
16 Drug Administration in June 2016, has reduced ap-  
17 plication burden with respect to individuals and phy-  
18 sicians seeking access to investigational new drugs  
19 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  
24 regulations released or updated under section 561 of  
25 the Federal Food, Drug, and Cosmetic Act (21

1 U.S.C. 360bbb) have improved access for individual  
2 patients who do not qualify for clinical trials of such  
3 investigational drugs, and what barriers to such ac-  
4 cess remain;

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

9 (5) an analysis of the Secretary's report under  
10 subsection (a)(2).

11 (c) GUIDANCE.—

12 (1) IN GENERAL.—Not later than 180 days  
13 after the publication of the report under subsection  
14 (a) the Secretary, acting through the Commissioner  
15 of Food and Drugs, shall issue one or more draft  
16 guidances regarding eligibility criteria for clinical  
17 trials. Not later than 18 months after the public  
18 comment period on each such draft guidance ends,  
19 the Secretary shall issue a revised draft guidance or  
20 final guidance.

21 (2) CONTENTS.—The guidance documents de-  
22 scribed in paragraph (1) shall address methodo-  
23 logical approaches that a manufacturer or sponsor of  
24 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 condi-  
4 tions or diseases for which there is an unmet  
5 medical need; and

6 (B) develop eligibility criteria for, and in-  
7 crease trial recruitment to, clinical trials so that  
8 enrollment in such trials more accurately re-  
9 flects the patients most likely to receive the  
10 drug, as applicable and as appropriate, while  
11 supporting findings of substantial evidence of  
12 safety and effectiveness.

13 **SEC. 3. IMPROVING INSTITUTIONAL REVIEW BOARD RE-**  
14 **VIEW OF SINGLE PATIENT EXPANDED AC-**  
15 **CESS PROTOCOL.**

16 Not later than 1 year after the date of enactment  
17 of this Act, the Secretary of Health and Human Services  
18 (referred to in this section as the “Secretary”), acting  
19 through the Commissioner of Food and Drugs, shall issue  
20 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,  
24 and Cosmetic Act (21 U.S.C. 360bbb(b)). Such guidance  
25 or regulation may include a description of the conditions

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  
13 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  
18 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-  
22 ceives a designation as a breakthrough therapy, fast  
23 track product, or regenerative advanced therapy

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

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