

112TH CONGRESS
2D SESSION

S. 2236

To provide for the expedited development and evaluation of drugs designated as breakthrough drugs.

IN THE SENATE OF THE UNITED STATES

MARCH 26, 2012

Mr. BENNET (for himself, Mr. HATCH, and Mr. BURR) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To provide for the expedited development and evaluation of drugs designated as breakthrough drugs.

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 “Advancing Break-
5 through Therapies for Patients Act of 2012”.

6 **SEC. 2. BREAKTHROUGH THERAPIES AND FAST TRACK**
7 **PRODUCTS.**

8 (a) IN GENERAL.—Section 506 of the Federal Food,
9 Drug, and Cosmetic Act (21 U.S.C. 356) is amended—

1 (1) in the heading, by inserting “**BREAK-**
2 **THROUGH THERAPIES AND**” before “**FAST**”;

3 (2) by redesignating subsections (a) through (d)
4 as subsections (b) through (e), respectively;

5 (3) by inserting before subsection (b), as so re-
6 designated, the following:

7 “(a) DESIGNATION OF A DRUG AS A BREAKTHROUGH
8 THERAPY.—

9 “(1) IN GENERAL.—The Secretary shall, at the
10 request of the sponsor of a drug, expedite the devel-
11 opment and review of such drug if the drug is in-
12 tended, alone or in combination with 1 or more other
13 drugs, to treat a serious or life-threatening disease
14 or condition and preliminary clinical evidence indi-
15 cates that the drug may demonstrate substantial im-
16 provement over existing therapies on 1 or more clini-
17 cally significant endpoints (such as substantial treat-
18 ment effects observed early in clinical development).
19 (In this section, such a drug is referred to as a
20 ‘breakthrough therapy’.)

21 “(2) REQUEST FOR DESIGNATION.—The spon-
22 sor of a drug may request the Secretary to designate
23 the drug as a breakthrough therapy. A request for
24 the designation may be made concurrently with, or
25 at any time after, the submission of an application

1 for the investigation of the drug under section 505(i)
2 or section 351(a)(3) of the Public Health Service
3 Act.

4 “(3) DESIGNATION.—

5 “(A) IN GENERAL.—Not later than 60 cal-
6 endar days after the receipt of a request under
7 paragraph (2), the Secretary shall determine
8 whether the drug that is the subject of the re-
9 quest meets the criteria described in paragraph
10 (1). If the Secretary finds that the drug meets
11 the criteria, the Secretary shall designate the
12 drug as a breakthrough therapy and shall take
13 such actions as are appropriate to expedite the
14 development and review of the application for
15 approval of such drug.

16 “(B) ACTIONS.—The actions to expedite
17 the development and review of an application
18 under subparagraph (A) shall include—

19 “(i) holding meetings with the sponsor
20 and the review team throughout the devel-
21 opment of the drug;

22 “(ii) providing timely advice to the
23 sponsor regarding the development of the
24 drug to ensure that the development pro-
25 gram to gather the non-clinical and clinical

1 data necessary for approval is as efficient
2 as practicable;

3 “(iii) involving senior managers and
4 experienced review staff, as appropriate, in
5 a collaborative, cross-disciplinary review;

6 “(iv) providing timely interactive com-
7 munication with sponsors;

8 “(v) assigning a cross-disciplinary
9 project lead for the Food and Drug Ad-
10 ministration review team to facilitate an
11 efficient review of the development pro-
12 gram and to serve as a scientific liaison be-
13 tween the review team and the sponsor;
14 and

15 “(vi) taking steps to ensure that the
16 design of the clinical trials is as efficient as
17 practicable, when scientifically appropriate,
18 such as by minimizing the number of pa-
19 tients enrolled in the trial and the duration
20 of the trial and considering alternatives to
21 the traditional multi-phase, sequential de-
22 velopment approach, designed to abbrevi-
23 ate, consolidate, and condense clinical
24 trials and studies.”;

1 (4) in subsection (e)(1), as so redesignated, by
2 inserting “breakthrough therapies and” after “appli-
3 cable to”; and

4 (5) by adding at the end the following:

5 “(f) GUIDANCE; AMENDED REGULATIONS.—

6 “(1) IN GENERAL.—

7 “(A) GUIDANCE.—Not later than 18
8 months after the date of enactment of the Ad-
9 vancing Breakthrough Therapies for Patients
10 Act of 2012, the Secretary shall issue draft
11 guidance on implementing the requirements
12 with respect to breakthrough therapies, acceler-
13 ated approval, and fast track products as set
14 forth in subsections (a) through (c), as amend-
15 ed by the Advancing Breakthrough Therapies
16 for Patients Act of 2012. After an opportunity
17 for public comment and not later than 2 years
18 after the date of enactment of the Advancing
19 Breakthrough Therapies for Patients Act of
20 2012, the Secretary shall issue final guidance.

21 “(B) AMENDED REGULATIONS.—Not later
22 than 2 years after the date of enactment of the
23 Advancing Breakthrough Therapies for Patients
24 Act of 2012, the Secretary shall amend the ap-
25 plicable regulations under title 21, Code of Fed-

1 eral Regulations, as may be necessary to imple-
2 ment the requirements under subsections (a)
3 through (c), as amended by the Advancing
4 Breakthrough Therapies for Patients Act of
5 2012.

6 “(2) REQUIREMENTS.—Guidance and regula-
7 tions promulgated under this section shall—

8 “(A) distinguish between products that
9 may qualify for—

10 “(i) treatment as a breakthrough
11 therapy;

12 “(ii) treatment as a fast track prod-
13 uct;

14 “(iii) accelerated approval; and

15 “(iv) a combination of all of the des-
16 ignations described in clauses (i) through
17 (iii); and

18 “(B) specify the actions the Secretary shall
19 take to expedite the development and review of
20 a breakthrough therapy pursuant to such des-
21 ignation under 506(a)(3), including updating
22 good review management practices to reflect
23 breakthrough therapies.

24 “(g) INDEPENDENT REVIEW.—Not later than 3
25 years after the date of enactment of this Act, the Sec-

1 retary shall, in conjunction with other planned reviews,
2 contract with an independent entity with expertise in as-
3 sessing the quality, efficiency, and predictability of bio-
4 pharmaceutical development and regulatory review pro-
5 grams to evaluate the manner by which the Food and
6 Drug Administration has applied the processes described
7 in the section, as amended by the Advancing Break-
8 through Therapies for Patients Act of 2012, and the im-
9 pact of such processes on the development and timely
10 availability of innovative treatments for patients affected
11 by serious or life-threatening conditions. Such evaluation
12 shall be completed not later than 4 years after the date
13 of enactment of the Advancing Breakthrough Therapies
14 for Patients Act of 2012 and shall be made publicly avail-
15 able upon completion.

16 “(h) REPORT.—Beginning in fiscal year 2013, the
17 Secretary shall annually prepare and submit to the Com-
18 mittee on Health, Education, Labor, and Pensions of the
19 Senate and the Committee on Energy and Commerce of
20 the House of Representatives, and make publicly available,
21 with respect to this section for the previous fiscal year—

22 “(1) the number of drugs for which a sponsor
23 requested designation as a breakthrough therapy;

24 “(2) the number of products designated as a
25 breakthrough therapy; and

1 “(3) for each breakthrough therapy approved in
2 the fiscal year—

3 “(A) the point in the drug development
4 and review process at which such breakthrough
5 designation occurred;

6 “(B) the total time from designation as a
7 breakthrough therapy, including the total time
8 to review and act on an application designated
9 as a breakthrough therapy, to approval of the
10 drug; and

11 “(C) the number of breakthrough therapies
12 approved on the first review out of the total
13 number of such therapies so approved.”.

14 (b) CONFORMING AMENDMENTS.—Section 506B(e)
15 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
16 356b) is amended by striking “section 506(b)(2)(A)” each
17 place such term appears and inserting “section
18 506(c)(2)(A)”.

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