

112TH CONGRESS  
2D SESSION

# H. R. 5334

To amend chapter V of the Federal Food, Drug, and Cosmetic Act to expedite the development and review of breakthrough therapies.

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## IN THE HOUSE OF REPRESENTATIVES

MAY 7, 2012

Mr. BILBRAY (for himself and Ms. DEGETTE) introduced the following bill;  
which was referred to the Committee on Energy and Commerce

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## A BILL

To amend chapter V of the Federal Food, Drug, and Cosmetic Act to expedite the development and review of breakthrough therapies.

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. BREAKTHROUGH THERAPIES.**

4       (a) IN GENERAL.—Section 506 (21 U.S.C. 356) is  
5       amended—

6               (1) by redesignating subsection (d) as sub-  
7       section (f);

8               (2) by redesignating subsections (a) through (c)  
9       as subsections (b) through (d), respectively;

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

3           “(a) DESIGNATION OF A DRUG AS A BREAKTHROUGH  
4           THERAPY.—

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

17          “(2) REQUEST FOR DESIGNATION.—The spon-  
18          sor of a drug may request the Secretary to designate  
19          the drug as a breakthrough therapy. A request for  
20          the designation may be made concurrently with, or  
21          at any time after, the submission of an application  
22          for the investigation of the drug under section 505(i)  
23          or section 351(a)(3) of the Public Health Service  
24          Act.

25          “(3) DESIGNATION.—

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

12          “(B) ACTIONS.—The actions to expedite  
13          the development and review of an application  
14          under subparagraph (A) may include, as appro-  
15          priate—

16               “(i) holding meetings with the sponsor  
17               and the review team throughout the devel-  
18               opment of the drug;

19               “(ii) providing timely advice to, and  
20               interactive communication with, the spon-  
21               sor regarding the development of the drug  
22               to ensure that the development program to  
23               gather the non-clinical and clinical data  
24               necessary for approval is as efficient as  
25               practicable;

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

4 “(iv) assigning a cross-disciplinary  
5 project lead for the Food and Drug Ad-  
6 ministration review team to facilitate an  
7 efficient review of the development pro-  
8 gram and to serve as a scientific liaison be-  
9 tween the review team and the sponsor;  
10 and

11 “(v) taking steps to ensure that the  
12 design of the clinical trials is as efficient as  
13 practicable, when scientifically appropriate,  
14 such as by minimizing the number of pa-  
15 tients exposed to a potentially less effica-  
16 cious treatment.”;

17 (4) in subsection (f)(1), as so redesignated, by  
18 striking “applicable to accelerated approval” and in-  
19 serting “applicable to breakthrough therapies, accel-  
20 erated approval, and”; and

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

22 “(g) REPORT.—Beginning in fiscal year 2013, the  
23 Secretary shall annually prepare and submit to the Com-  
24 mittee on Health, Education, Labor, and Pensions of the  
25 Senate and the Committee on Energy and Commerce of

1 the House of Representatives, and make publicly available,  
2 with respect to this section for the previous fiscal year—

3 “(1) the number of drugs for which a sponsor  
4 requested designation as a breakthrough therapy;  
5 and

6 “(2) the number of products designated as a  
7 breakthrough therapy.”.

8 (b) GUIDANCE; AMENDED REGULATIONS.—

9 (1) IN GENERAL.—

10 (A) GUIDANCE.—Not later than 18  
11 months after the date of enactment of this Act,  
12 the Secretary of Health and Human Services  
13 (referred to in this section as the “Secretary”)  
14 shall issue draft guidance on implementing the  
15 requirements with respect to breakthrough  
16 therapies, as set forth in section 506(a) of the  
17 Federal Food, Drug, and Cosmetic Act (21  
18 U.S.C. 356(a)), as amended by this section.  
19 The Secretary shall issue final guidance not  
20 later than 1 year after the close of the comment  
21 period for the draft guidance.

22 (B) AMENDED REGULATIONS.—

23 (i) IN GENERAL.—If the Secretary de-  
24 termines that it is necessary to amend the  
25 regulations under title 21, Code of Federal

1 Regulations in order to implement the  
2 amendments made by this section to sec-  
3 tion 506(a) of the Federal Food, Drug,  
4 and Cosmetic Act, the Secretary shall  
5 amend such regulations not later than 2  
6 years after the date of enactment of this  
7 Act.

8 (ii) PROCEDURE.—In amending regu-  
9 lations under clause (i), the Secretary  
10 shall—

11 (I) issue a notice of proposed  
12 rulemaking that includes the proposed  
13 regulation;

14 (II) provide a period of not less  
15 than 60 days for comments on the  
16 proposed regulation; and

17 (III) publish the final regulation  
18 not less than 30 days before the effec-  
19 tive date of the regulation.

20 (iii) RESTRICTIONS.—Notwithstanding  
21 any other provision of law, the Secretary  
22 shall promulgate regulations implementing  
23 the amendments made by this section only  
24 as described in clause (ii).

1           (2) REQUIREMENTS.—Guidance issued under  
2       this section shall—

3           (A) specify the process and criteria by  
4       which the Secretary makes a designation under  
5       section 506(a)(3) of the Federal Food, Drug,  
6       and Cosmetic Act; and

7           (B) specify the actions the Secretary shall  
8       take to expedite the development and review of  
9       a breakthrough therapy pursuant to such des-  
10      ignation under such section 506(a)(3), includ-  
11      ing updating good review management practices  
12      to reflect breakthrough therapies.

13       (c) INDEPENDENT REVIEW.—Not later than 3 years  
14   after the date of enactment of this Act, the Comptroller  
15   General of the United States, in consultation with appro-  
16   priate experts, shall assess the manner by which the Food  
17   and Drug Administration has applied the processes de-  
18   scribed in section 506(a) of the Federal Food, Drug, and  
19   Cosmetic Act, as amended by this section, and the impact  
20   of such processes on the development and timely avail-  
21   ability of innovative treatments for patients affected by se-  
22   rious or life-threatening conditions. Such assessment shall  
23   be made publicly available upon completion.

24       (d) CONFORMING AMENDMENTS.—Section 506B(e)  
25   (21 U.S.C. 356b) is amended by striking “section

- 1 506(b)(2)(A)” each place such term appears and inserting
- 2 “section 506(c)(2)(A)”.

