

112TH CONGRESS  
2D SESSION

# H. R. 4132

To amend section 506 of the Federal Food, Drug, and Cosmetic Act to expedited approval of drugs for serious or life-threatening diseases or conditions.

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## IN THE HOUSE OF REPRESENTATIVES

MARCH 5, 2012

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

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## A BILL

To amend section 506 of the Federal Food, Drug, and Cosmetic Act to expedited approval of drugs for serious or life-threatening diseases or conditions.

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 “Faster Access to Spe-  
5 cialized Treatments Act” or “FAST Act”.

6 **SEC. 2. FINDINGS; SENSE OF CONGRESS.**

7 (a) FINDINGS.—Congress finds the following:

8 (1) The Food and Drug Administration (FDA)  
9 serves a critical role in helping to assure that new

1 medicines are safe and effective. Regulatory innova-  
2 tion is one element of the Nation's strategy to ad-  
3 dress serious and life-threatening diseases or condi-  
4 tions by promoting investment in and development  
5 of innovative treatments for unmet medical needs.

6 (2) Over the previous two decades, since the ac-  
7 celerated approval mechanism was established, ad-  
8 vances in medical sciences, including genomics, mo-  
9 lecular biology, and bioinformatics, have provided an  
10 unprecedented understanding of the underlying bio-  
11 logical mechanism and pathogenesis of disease. A  
12 new generation of modern, targeted medicines is cur-  
13 rently under development to treat serious and life-  
14 threatening diseases, some applying drug develop-  
15 ment strategies based on biomarkers or  
16 pharmacogenomics, predictive toxicology, clinical  
17 trial enrichment techniques, and novel clinical trial  
18 designs, such as adaptive clinical trials.

19 (3) As a result of these remarkable scientific  
20 and medical advances, FDA should be encouraged to  
21 implement more broadly effective processes for the  
22 expedited development and review of innovative new  
23 medicines intended to address unmet medical needs  
24 for serious or life-threatening diseases or conditions,  
25 including those for rare diseases or conditions, using

1 a broad range of surrogate or clinical endpoints and  
2 modern scientific tools earlier in the drug develop-  
3 ment cycle when appropriate. This may result in  
4 fewer, smaller, or shorter clinical trials for the in-  
5 tended patient population or targeted subpopulation  
6 without compromising or altering FDA's existing  
7 high standards for the approval of drugs.

8 (4) Patients benefit from expedited access to  
9 safe and effective innovative therapies to treat  
10 unmet medical needs for serious or life-threatening  
11 diseases or conditions.

12 (5) For these reasons, the existing statutory au-  
13 thority governing expedited approval of drugs or se-  
14 rious or life-threatening conditions should be amend-  
15 ed in order to enhance FDA's authority to consider  
16 appropriate scientific data, methods, and tools, and  
17 to expedite development and access to novel treat-  
18 ments for patients with a broad range of serious or  
19 life-threatening diseases or conditions.

20 (b) SENSE OF CONGRESS.—It is the sense of Con-  
21 gress that the Food and Drug Administration should  
22 apply the accelerated approval and the fast track provi-  
23 sions set forth in section 506 of the Federal Food, Drug,  
24 and Cosmetic Act (21 U.S.C. 356), as amended by section  
25 3, to the greatest extent possible to help expedite the de-

1 velopment and availability to patients of treatments for  
2 serious or life-threatening diseases or conditions while  
3 maintaining appropriate safety and effectiveness stand-  
4 ards for such treatments.

5 **SEC. 3. EXPEDITED APPROVAL OF DRUGS FOR SERIOUS OR**  
6 **LIFE-THREATENING DISEASES OR CONDI-**  
7 **TIONS.**

8 Section 506 of the Federal Food, Drug, and Cosmetic  
9 Act (21 U.S.C. 356) is amended to read as follows:

10 **“SEC. 506. EXPEDITED APPROVAL OF DRUGS FOR SERIOUS**  
11 **OR LIFE-THREATENING DISEASES OR CONDI-**  
12 **TIONS.**

13 “(a) DESIGNATION OF DRUG AS A FAST TRACK  
14 PRODUCT.—

15 “(1) IN GENERAL.—The Secretary shall, at the  
16 request of the sponsor of a new drug, facilitate the  
17 development and expedite the review of such drug if  
18 it is intended, whether alone or in combination with  
19 one or more other drugs, for the treatment of a seri-  
20 ous or life-threatening disease or condition, and it  
21 demonstrates the potential to address unmet medical  
22 needs for such a disease or condition. (In this sec-  
23 tion, such a drug is referred to as a ‘fast track prod-  
24 uct’.)

1           “(2) REQUEST FOR DESIGNATION.—The spon-  
2           sor of a new drug may request the Secretary to des-  
3           ignate the drug as a fast track product. A request  
4           for the designation may be made concurrently with,  
5           or at any time after, submission of an application  
6           for the investigation of the drug under section 505(i)  
7           or section 351(a)(3) of the Public Health Service  
8           Act.

9           “(3) DESIGNATION.—Within 60 calendar days  
10          after the receipt of a request under paragraph (2),  
11          the Secretary shall determine whether the drug that  
12          is the subject of the request meets the criteria de-  
13          scribed in paragraph (1). If the Secretary finds that  
14          the drug meets the criteria, the Secretary shall des-  
15          ignate the drug as a fast track product and shall  
16          take such actions as are appropriate to expedite the  
17          development and review of the application for ap-  
18          proval of such product.

19          “(b) ACCELERATED APPROVAL OF A DRUG FOR A  
20          SERIOUS OR LIFE-THREATENING DISEASE OR CONDI-  
21          TION, INCLUDING A FAST TRACK PRODUCT.—

22                 “(1) IN GENERAL.—The Secretary may approve  
23                 an application for approval of a product for a seri-  
24                 ous or life-threatening disease or condition, including  
25                 a fast track product, under section 505(c) or section

1 351(a) of the Public Health Service Act upon mak-  
2 ing a determination (taking into account the severity  
3 or rarity of the disease or condition and the avail-  
4 ability of alternative treatments) that the product  
5 has an effect on—

6 “(A) a surrogate endpoint that is reason-  
7 ably likely to predict clinical benefit; or

8 “(B) a clinical endpoint, including an end-  
9 point that can be measured earlier than irre-  
10 reversible morbidity or mortality, that is reason-  
11 ably likely to predict an effect on irreversible  
12 morbidity or mortality or other clinical benefit.

13 The evidence to support that an endpoint is reason-  
14 ably likely to predict clinical benefit may include epi-  
15 demiological, pathophysiologic, pharmacologic, thera-  
16 peutic or other evidence developed using, for exam-  
17 ple, biomarkers, or other scientific methods or tools.

18 “(2) LIMITATION.—Approval of a product  
19 under this subsection may, as determined by the  
20 Secretary, be subject to the following require-  
21 ments—

22 “(A) that the sponsor conduct appropriate  
23 post-approval studies to verify and describe the  
24 predicted effect of the product on irreversible

1 morbidity or mortality or other clinical benefit;  
2 and

3 “(B) that the sponsor submit copies of all  
4 promotional materials related to the product, at  
5 least 30 days prior to dissemination of the ma-  
6 terials during—

7 “(i) the preapproval review period;  
8 and

9 “(ii) following approval, for a period  
10 that the Secretary determines to be appro-  
11 priate.

12 “(3) EXPEDITED WITHDRAWAL OF AP-  
13 PROVAL.—The Secretary may withdraw approval of  
14 a product approved pursuant to this subsection  
15 using expedited procedures (as prescribed by the  
16 Secretary in regulations, which shall include an op-  
17 portunity for an informal hearing) if—

18 “(A) the sponsor fails to conduct any re-  
19 quired post-approval study of the product with  
20 due diligence;

21 “(B) a study required to verify and de-  
22 scribe the predicted effect on irreversible mor-  
23 bidity or mortality or other clinical benefit of  
24 the product fails to verify and describe such ef-  
25 fect or benefit;

1           “(C) other evidence demonstrates that the  
2           product is not safe or effective under the condi-  
3           tions of use; or

4           “(D) the sponsor disseminates false or  
5           misleading promotional materials with respect  
6           to the product.

7           “(c) REVIEW OF INCOMPLETE APPLICATIONS FOR  
8 APPROVAL OF A FAST TRACK PRODUCT.—

9           “(1) IN GENERAL.—If the Secretary deter-  
10          mines, after preliminary evaluation of clinical data  
11          submitted by the sponsor, that a fast track product  
12          may be effective, the Secretary shall evaluate for fil-  
13          ing, and may commence review of portions of, an ap-  
14          plication for the approval of the product before the  
15          sponsor submits a complete application. The Sec-  
16          retary shall commence such review only if the appli-  
17          cant—

18                 “(A) provides a schedule for submission of  
19                 information necessary to make the application  
20                 complete; and

21                 “(B) pays any fee that may be required  
22                 under section 736.

23           “(2) EXCEPTION.—Any time period for review  
24          of human drug applications that has been agreed to  
25          by the Secretary and that has been set forth in goals



1 identified in letters of the Secretary (relating to the  
2 use of fees collected under section 736 to expedite  
3 the drug development process and the review of  
4 human drug applications) shall not apply to an ap-  
5 plication submitted under paragraph (1) until the  
6 date on which the application is complete.

7 “(d) AWARENESS EFFORTS.—The Secretary shall—

8 “(1) develop and disseminate to physicians, pa-  
9 tient organizations, pharmaceutical and bio-  
10 technology companies, and other appropriate persons  
11 a description of the provisions of this section appli-  
12 cable to accelerated approval and fast track prod-  
13 ucts; and

14 “(2) establish a program to encourage the de-  
15 velopment of surrogate and clinical endpoints, in-  
16 cluding biomarkers, and other scientific methods and  
17 tools that can assist the Secretary in determining  
18 whether the evidence submitted in an application is  
19 reasonably likely to predict clinical benefit for seri-  
20 ous or life-threatening conditions for which there  
21 exist significant unmet medical needs.”.

22 **SEC. 4. GUIDANCE; AMENDED REGULATIONS.**

23 (a) INITIAL GUIDANCE.—Not later than one year  
24 after the date of enactment of this Act, the Secretary of  
25 Health and Human Services (hereinafter “the Secretary”)

1 shall issue draft guidance to implement the amendments  
2 made by section 3.

3 (b) FINAL GUIDANCE.—Not later than one year after  
4 the issuance of draft guidance under subsection (a), after  
5 an opportunity for public comment, the Secretary shall  
6 issue—

7 (1) final guidance to implement the amend-  
8 ments made by section 3; and

9 (2) amend the regulations governing accelerated  
10 approval in parts 314 and 601 of title 21, Code of  
11 Federal Regulations, as necessary to conform such  
12 regulations with the amendments made by section 3.

13 (c) CONSIDERATIONS.—In developing the guidance  
14 under subsections (a) and (b)(1) and the amendments  
15 under subsection (b)(2), the Secretary shall consider—

16 (1) issues arising under the accelerated ap-  
17 proval and fast track processes under section 506 of  
18 the Federal Food, Drug, and Cosmetic Act (as  
19 amended by section 3) for drugs designated for a  
20 rare disease or condition under section 526 of the  
21 Federal, Food, Drug, and Cosmetic Act; and

22 (2) how to incorporate novel approaches to the  
23 review of surrogate endpoints based on patho-  
24 physiologic and pharmacologic evidence in such guid-  
25 ance, especially in instances where the low preva-

1 lence of a disease renders the existence or collection  
2 of other types of data unlikely or impractical.

3 (d) NO DELAY IN REVIEW OR APPROVAL.—The  
4 issuance (or non-issuance) of guidance or conforming reg-  
5 ulations implementing the amendments made by section  
6 3 shall not preclude the review of, or action on, a request  
7 for designation or an application for approval submitted  
8 pursuant to section 506 of the Federal Food, Drug, and  
9 Cosmetic Act, as amended by section 3.

10 **SEC. 5. INDEPENDENT REVIEW.**

11 (a) IN GENERAL.—The Secretary shall, in conjunc-  
12 tion with other planned reviews of the new drug review  
13 process, contract with an independent entity with expertise  
14 in assessing the quality and efficiency of biopharma-  
15 ceutical development and regulatory review programs, to  
16 evaluate the Food and Drug Administration’s application  
17 of the processes described in section 506 of the Federal  
18 Food, Drug, and Cosmetic Act, as amended by section 3,  
19 and the impact of such processes on the development and  
20 timely availability of innovative treatments for patients  
21 suffering from serious or life-threatening conditions.

22 (b) CONSULTATION.—Any evaluation under sub-  
23 section (a) shall include consultation with regulated indus-  
24 tries, patient advocacy and disease research foundations,  
25 and relevant academic medical centers.

1 **SEC. 6. RULE OF CONSTRUCTION.**

2       The amendments made to section 506(b) of the Fed-  
3 eral Food, Drug and Cosmetic Act by this Act shall be  
4 construed in a manner that encourages the Secretary to  
5 utilize innovative approaches for the assessment of prod-  
6 ucts under accelerated approval while maintaining appro-  
7 priate safety and effectiveness standards for such prod-  
8 ucts.

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