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(Original Signature of Member)

112TH CONGRESS
2D SESSION

H. R. _____

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.

IN THE HOUSE OF REPRESENTATIVES

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

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.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Faster Access to Specialized Treatments Act'' or ''FAST Act''.

SEC. 2. FINDINGS; SENSE OF CONGRESS.

(a) FINDINGS.—Congress finds the following:

(1) The Food and Drug Administration (FDA) serves a critical role in helping to assure that new medicines are safe and effective. Regulatory innovation is one element of the nation's strategy to address serious and life-threatening diseases or conditions by promoting investment in and development of innovative treatments for unmet medical needs.

(2) Over the previous two decades, since the accelerated approval mechanism was established, advances in medical sciences, including genomics, molecular biology, and bioinformatics, have provided an unprecedented understanding of the underlying biological mechanism and pathogenesis of disease. A new generation of modern, targeted medicines is currently under development to treat serious and life-threatening diseases, some applying drug development strategies based on biomarkers or pharmacogenomics, predictive toxicology, clinical trial enrichment techniques, and novel clinical trial designs, such as adaptive clinical trials.

(3) As a result of these remarkable scientific and medical advances, FDA should be encouraged to implement more broadly effective processes for the expedited development and review of innovative new medicines intended to address unmet medical needs

for serious or life-threatening diseases or conditions, including those for rare diseases or conditions, using a broad range of surrogate or clinical endpoints and modern scientific tools earlier in the drug development cycle when appropriate. This may result in fewer, smaller, or shorter clinical trials for the intended patient population or targeted subpopulation without compromising or altering FDA's existing high standards for the approval of drugs.

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

(5) For these reasons, the existing statutory authority governing expedited approval of drugs or serious or life-threatening conditions should be amended in order to enhance FDA's authority to consider appropriate scientific data, methods, and tools, and to expedite development and access to novel treatments for patients with a broad range oofserious or life-threatening diseases or conditions.

(b) SENSE OF CONGRESS.—It is the sense of Congress that the Food and Drug Administration should apply the accelerated approval and the fast track provisions set forth in section 506 of the Federal Food, Drug,

and Cosmetic Act (21 U.S.C. 356), as amended by section 3, to the greatest extent possible to help expedite the development and availability to patients of treatments for serious or life-threatening diseases or conditions while maintaining appropriate safety and effectiveness standards for such treatments.

SEC. 3. EXPEDITED APPROVAL OF DRUGS FOR SERIOUS OR LIFE-THREATENING DISEASES OR CONDITIONS.

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

"SEC. 506. EXPEDITED APPROVAL OF DRUGS FOR SERIOUS OR LIFE-THREATENING DISEASES OR CONDITIONS.

''(a) DESIGNATION OF DRUG AS A FAST TRACK PRODUCT.—

''(1) IN GENERAL.—The Secretary shall, at the request of the sponsor of a new drug, facilitate the development and expedite the review of such drug if it is intended, whether alone or in combination with one or more other drugs, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. (In this sec-

tion, such a drug is referred to as a 'fast track product'.)

''(2) REQUEST FOR DESIGNATION.—The sponsor of a new drug may request the Secretary to designate the drug as a fast track product. A request for the designation may be made concurrently with, or at any time after, submission of an application for the investigation of the drug under section 505(i) or section 351(a)(3) of the Public Health Service Act.

''(3) DESIGNATION.—Within 60 calendar days after the receipt of a request under paragraph (2), the Secretary shall determine whether the drug that is the subject of the request meets the criteria described in paragraph (1). If the Secretary finds that the drug meets the criteria, the Secretary shall designate the drug as a fast track product and shall take such actions as are appropriate to expedite the development and review of the application for approval of such product.

''(b) ACCELERATED APPROVAL OF A DRUG FOR A SERIOUS OR LIFE-THREATENING DISEASE OR CONDITION, INCLUDING A FAST TRACK PRODUCT.—

''(1) IN GENERAL.—The Secretary may approve an application for approval of a product for a seri-

ous or life-threatening disease or condition, including a fast track product, under section 505(c) or section 351(a) of the Public Health Service Act upon making a determination (taking into account the severity or rarity of the disease or condition and the availability of alternative treatments) that the product has an effect on—

''(A) a surrogate endpoint that is reasonably likely to predict clinical benefit; or

''(B) a clinical endpoint, including an endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit.

The evidence to support that an endpoint is reasonably likely to predict clinical benefit may include epidemiological, pathophysiologic, pharmacologic, therapeutic or other evidence developed using, for example, biomarkers, or other scientific methods or tools.

''(2) LIMITATION.—Approval of a product under this subsection may, as determined by the Secretary, be subject to the following requirements—

''(A) that the sponsor conduct appropriate post-approval studies to verify and describe the

predicted effect of the product on irreversible morbidity or mortality or other clinical benefit; and

''(B) that the sponsor submit copies of all promotional materials related to the product, at least 30 days prior to dissemination of the materials during—

''(i) the preapproval review period; and

''(ii) following approval, for a period that the Secretary determines to be appropriate.

''(3) EXPEDITED WITHDRAWAL OF APPROVAL.—The Secretary may withdraw approval of a product approved pursuant to this subsection using expedited procedures (as prescribed by the Secretary in regulations, which shall include an opportunity for an informal hearing) if—

''(A) the sponsor fails to conduct any required post-approval study of the product with due diligence;

''(B) a study required to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical benefit of

the product fails to verify and describe such effect or benefit;

''(C) other evidence demonstrates that the product is not safe or effective under the conditions of use; or

''(D) the sponsor disseminates false or misleading promotional materials with respect to the product.

''(c) REVIEW OF INCOMPLETE APPLICATIONS FOR APPROVAL OF A FAST TRACK PRODUCT.—

''(1) IN GENERAL.—If the Secretary determines, after preliminary evaluation of clinical data submitted by the sponsor, that a fast track product may be effective, the Secretary shall evaluate for filing, and may commence review of portions of, an application for the approval of the product before the sponsor submits a complete application. The Secretary shall commence such review only if the applicant—

''(A) provides a schedule for submission of information necessary to make the application complete; and

''(B) pays any fee that may be required under section 736.

''(2) EXCEPTION.—Any time period for review of human drug applications that has been agreed to by the Secretary and that has been set forth in goals identified in letters of the Secretary (relating to the use of fees collected under section 736 to expedite the drug development process and the review of human drug applications) shall not apply to an application submitted under paragraph (1) until the date on which the application is complete.

''(d) AWARENESS EFFORTS.—The Secretary shall—

''(1) develop and disseminate to physicians, patient organizations, pharmaceutical and biotechnology companies, and other appropriate persons a description of the provisions of this section applicable to accelerated approval and fast track products; and

''(2) establish a program to encourage the development of surrogate and clinical endpoints, including biomarkers, and other scientific methods and tools that can assist the Secretary in determining whether the evidence submitted in an application is reasonably likely to predict clinical benefit for serious or life-threatening conditions for which there exist significant unmet medical needs.''.

SEC. 4. GUIDANCE; AMENDED REGULATIONS.

(a) INITIAL GUIDANCE.—Not later than one year after the date of enactment of this Act, the Secretary of Health and Human Services (hereinafter ''the Secretary'') shall issue draft guidance to implement the amendments made by section 3.

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

(1) final guidance to implement the amendments made by section 3; and

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

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

(1) issues arising under the accelerated approval and fast track processes under section 506 of the Federal Food, Drug, and Cosmetic Act (as amended by section 3) for drugs designated for a rare disease or condition under section 526 of the Federal, Food, Drug, and Cosmetic Act; and

(2) how to incorporate novel approaches to the review of surrogate endpoints based on pathophysiologic and pharmacologic evidence in such guidance, especially in instances where the low prevalence of a disease renders the existence or collection of other types of data unlikely or impractical.

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

SEC. 5. INDEPENDENT REVIEW.

(a) IN GENERAL.—The Secretary shall, in conjunction with other planned reviews of the new drug review process, contract with an independent entity with expertise in assessing the quality and efficiency of biopharmaceutical development and regulatory review programs, to evaluate the Food and Drug Administration's application of the processes described in section 506 of the Federal Food, Drug, and Cosmetic Act, as amended by section 3, and the impact of such processes on the development and timely availability of innovative treatments for patients suffering from serious or life-threatening conditions.

(b) CONSULTATION.—Any evaluation under subsection (a) shall include consultation with regulated industries, patient advocacy and disease research foundations, and relevant academic medical centers.

SEC. 6. RULE OF CONSTRUCTION.

The amendments made to section 506(b) of the Federal Food, Drug and Cosmetic Act by this Act shall be construed in a manner that encourages the Secretary to utilize innovative approaches for the assessment of products under accelerated approval while maintaining appropriate safety and effectiveness standards for such products.