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112TH CONGRESS
1ST SESSION

H. R. 3737

To amend the Federal Food, Drug, and Cosmetic Act with respect to fast track approval of certain orphan drugs.

IN THE HOUSE OF REPRESENTATIVES

DECEMBER 20, 2011

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 the Federal Food, Drug, and Cosmetic Act with respect to fast track approval of certain orphan drugs.

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 ''Unlocking Lifesaving Treatments for Rare-Diseases Act'' or ''ULTRA''.

SEC. 2. IMPROVING THE ACCELERATED APPROVAL PATHWAY FOR FAST TRACK PRODUCTS TO SERVE THE UNMET NEEDS OF INDIVIDUALS WITH ULTRA RARE DISEASES.

Section 506 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356) is amended by adding at the end the following:

''(e) SCIENTIFIC STANDARDS FOR APPROVAL OF CERTAIN ORPHAN DRUGS AS FAST TRACK PRODUCTS.—

''(1) IN GENERAL.—The Secretary may approve an application for a drug designated under section 526 for a rare disease or condition as a fast track product using a surrogate endpoint as described under paragraph (2) if—

''(A) the Secretary makes an initial determination that the drug is eligible for approval—

''(i) as a drug designated for a rare disease or condition under section 526; and

''(ii) as a fast track product under this section; and

''(B) the drug is a treatment for a disease or condition that affects a small number of patients in the United States, as determined by the Secretary in designating the drug for a rare disease or condition under section 526.

''(2) SURROGATE ENDPOINT DEFINITION FOR CERTAIN FAST TRACK PRODUCTS.—

''(A) IN GENERAL.—If a drug meets the criteria established in paragraph (1), the Secretary—

''(i) may use a surrogate endpoint for the approval of the drug as a fast track product based on the existence of reasonable scientific data that support and qualify the relevance of the surrogate endpoint to the disease state and treatment; and

''(ii) shall not require clinical treatment data or other historical clinical data on the surrogate endpoint as a prerequisite to assessment of the surrogate endpoint under this subsection if such data are not available.

''(B) USE OF CLINICAL DATA.—

''(i) Subject to subparagraph (A)(ii), in a surrogate endpoint assessment under this subsection, the Secretary may take into consideration any reliable clinical data that are readily available and published.

''(ii) For a surrogate endpoint which the Secretary decides to use in accordance

with subparagraph (A), nothing in this subsection shall preclude the Secretary from requiring clinical data that makes use of the surrogate endpoint as a condition of approval for the fast track product.

''(C) GUIDANCE AND CONSIDERATIONS.—Not later than 1 year after the date of enactment of the Unlocking Lifesaving Treatments for Rare-Diseases Act, the Secretary shall issue guidance providing details and options for qualifying surrogate endpoints without clinical data pursuant to this subsection. In qualifying a surrogate endpoint under this subsection, the Secretary shall take into account and balance the following considerations:

''(i) The unmet need served by the drug and the adverse effects of the rare disease or condition on quality of life and length of life.

''(ii) The very low likelihood that clinical data would exist or that clinical studies would be completed to support a surrogate endpoint due to the small size of the patient population in the United States and other significant barriers inherent in

performing such clinical studies due to the prevalence of the disease or related factors.

''(iii) The full scope of available basic scientific data and information describing the pathophysiology of the disease, mechanism of action of the drug, biology of the relevant disease pathway, information regarding the quality of the biomarker assay, model treatment data, or other supportive scientific information that the Secretary deems reasonably predictive of a clinical benefit in the absence of clinical data.''.

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