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(Original Signature of Member)

112TH CONGRESS  
1ST SESSION

**H. R.** \_\_\_\_\_

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

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IN THE HOUSE OF REPRESENTATIVES

Mr. STEARNS introduced the following bill; which was referred to the Committee on \_\_\_\_\_

\_\_\_\_\_  
**A BILL**

To amend the Federal Food, Drug, and Cosmetic Act with respect to fast track approval of certain orphan 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 “Unlocking Lifesaving  
5 Treatments for Rare-Diseases Act” or “ULTRA”.

1 **SEC. 2. IMPROVING THE ACCELERATED APPROVAL PATH-**  
2 **WAY FOR FAST TRACK PRODUCTS TO SERVE**  
3 **THE UNMET NEEDS OF INDIVIDUALS WITH**  
4 **ULTRA RARE DISEASES.**

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

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

10 “(1) IN GENERAL.—The Secretary may approve  
11 an application for a drug designated under section  
12 526 for a rare disease or condition as a fast track  
13 product using a surrogate endpoint as described  
14 under paragraph (2) if—

15 “(A) the Secretary makes an initial deter-  
16 mination that the drug is eligible for approval—

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

19 “(ii) as a fast track product under  
20 this section; and

21 “(B) the drug is a treatment for a disease  
22 or condition that affects a small number of pa-  
23 tients in the United States, as determined by  
24 the Secretary in designating the drug for a rare  
25 disease or condition under section 526.

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

3           “(A) IN GENERAL.—If a drug meets the  
4           criteria established in paragraph (1), the Sec-  
5           retary—

6                   “(i) may use a surrogate endpoint for  
7                   the approval of the drug as a fast track  
8                   product based on the existence of reason-  
9                   able scientific data that support and qual-  
10                  ify the relevance of the surrogate endpoint  
11                  to the disease state and treatment; and

12                   “(ii) shall not require clinical treat-  
13                   ment data or other historical clinical data  
14                   on the surrogate endpoint as a prerequisite  
15                   to assessment of the surrogate endpoint  
16                   under this subsection if such data are not  
17                   available.

18           “(B) USE OF CLINICAL DATA.—

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

24                   “(ii) For a surrogate endpoint which  
25                   the Secretary decides to use in accordance

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

6 “(C) GUIDANCE AND CONSIDERATIONS.—  
7 Not later than 1 year after the date of enact-  
8 ment of the Unlocking Lifesaving Treatments  
9 for Rare-Diseases Act, the Secretary shall issue  
10 guidance providing details and options for  
11 qualifying surrogate endpoints without clinical  
12 data pursuant to this subsection. In qualifying  
13 a surrogate endpoint under this subsection, the  
14 Secretary shall take into account and balance  
15 the following considerations:

16 “(i) The unmet need served by the  
17 drug and the adverse effects of the rare  
18 disease or condition on quality of life and  
19 length of life.

20 “(ii) The very low likelihood that clin-  
21 ical data would exist or that clinical stud-  
22 ies would be completed to support a surro-  
23 gate endpoint due to the small size of the  
24 patient population in the United States  
25 and other significant barriers inherent in

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

3 “(iii) The full scope of available basic  
4 scientific data and information describing  
5 the pathophysiology of the disease, mecha-  
6 nism of action of the drug, biology of the  
7 relevant disease pathway, information re-  
8 garding the quality of the biomarker assay,  
9 model treatment data, or other supportive  
10 scientific information that the Secretary  
11 deems reasonably predictive of a clinical  
12 benefit in the absence of clinical data.”.