

Calendar No. 416

114TH CONGRESS
2^D SESSION

S. 2030

To allow the sponsor of an application for the approval of a targeted drug to rely upon data and information with respect to such sponsor's previously approved targeted drugs.

IN THE SENATE OF THE UNITED STATES

SEPTEMBER 15, 2015

Mr. BENNET (for himself, Mr. BURR, Ms. WARREN, Mr. HATCH, and Mr. ENZI) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

APRIL 5, 2016

Reported by Mr. ALEXANDER, with an amendment

[Strike out all after the enacting clause and insert the part printed in *italic*]

A BILL

To allow the sponsor of an application for the approval of a targeted drug to rely upon data and information with respect to such sponsor's previously approved targeted drugs.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

1 **SECTION 1. SHORT TITLE.**

2 This Act may be cited as the “Advancing Targeted
3 Therapies for Rare Diseases Act of 2015”.

4 **SEC. 2. TARGETED DRUGS FOR RARE DISEASES.**

5 Title V of the Federal Food, Drug, and Cosmetic Act
6 (21 U.S.C. 351 et seq.) is amended by inserting after sec-
7 tion 506F the following:

8 **“SEC. 506G. TARGETED DRUGS FOR RARE DISEASES.**

9 “(a) **PURPOSE.**—The purpose of this section, through
10 the approach provided for in subsection (b), is to—

11 “(1) facilitate the development, review, and ap-
12 proval of genetically targeted drugs to address an
13 unmet medical need in one or more patient sub-
14 groups (or gene variant subpopulations) with respect
15 to rare diseases or conditions that are serious or life-
16 threatening; and

17 “(2) maximize the use of scientific tools or
18 methods, including surrogate endpoints and other
19 biomarkers for such purposes.

20 “(b) **LEVERAGING OF DATA FROM PREVIOUSLY AP-**
21 **PROVED DRUG APPLICATION OR APPLICATIONS.**—The
22 Secretary may, consistent with applicable standards for
23 approval under this Act or section 351 of the Public
24 Health Service Act, allow the sponsor of a genetically tar-
25 geted drug to rely upon data and information—

1 “(1) previously developed by the same sponsor
 2 (or another sponsor that has provided the sponsor
 3 with a contractual right of reference to such data
 4 and information); and

5 “(2) submitted by a sponsor described in para-
 6 graph (1) in support of one or more applications
 7 previously approved under this Act or section 351 of
 8 the Public Health Service Act,
 9 for a drug that incorporates or utilizes the same or similar
 10 genetically targeted technology, or the same variant pro-
 11 tein targeted technology, as the drug or drugs that are
 12 the subject of an application or applications described in
 13 paragraph (2).

14 “(e) DEFINITIONS.—For purposes of this section—

15 “(1) the term ‘genetically targeted drug’ means
 16 a drug which—

17 “(A) is the subject of an application under
 18 section 505(b)(1) of this Act or section 351(a)
 19 of the Public Health Service Act for the treat-
 20 ment of a rare disease or condition (as such
 21 term is defined in section 526) that is serious
 22 or life-threatening;

23 “(B) incorporates or utilizes a genetically
 24 targeted technology or a variant protein tar-
 25 geted technology; and

1 “(C) may result in the modulation (includ-
2 ing suppression, up-regulation, or activation) of
3 the function of a gene or its associated gene
4 product;

5 “(2) the term ‘genetically targeted technology’
6 means a technology comprising non-replicating nu-
7 cleic acid or analogous compounds with a common or
8 similar chemistry that is intended to treat one or
9 more subsets of patients with the same disease, in-
10 cluding due to other variants in the same gene; and

11 “(3) the term ‘variant protein targeted tech-
12 nology’ means a technology or compound that modu-
13 lates the function of a variant protein, due to a gene
14 variant, intended to treat one or more subsets of pa-
15 tients with the same disease, due to other variants
16 in the same gene.

17 “(d) RULE OF CONSTRUCTION.—Nothing in this sec-
18 tion shall be construed to—

19 “(1) alter the authority of the Secretary to ap-
20 prove drugs pursuant to this Act or section 351 of
21 the Public Health Service Act (as authorized prior
22 to the date of enactment of the Advancing Targeted
23 Therapies for Rare Diseases Act of 2015); including
24 the standards of evidence; and applicable conditions;
25 for approval under such Act; or

1 ~~“(2) confer any new rights, beyond those au-~~
 2 ~~thorized under this section, with respect to the per-~~
 3 ~~missibility of referencing information contained in~~
 4 ~~another application submitted under section~~
 5 ~~505(b)(1) of this Act or section 351(a) of the Public~~
 6 ~~Health Service Act.”.~~

7 **SECTION 1. SHORT TITLE.**

8 *This Act may be cited as the “Advancing Targeted*
 9 *Therapies for Rare Diseases Act of 2016”.*

10 **SEC. 2. TARGETED DRUGS FOR RARE DISEASES.**

11 *Subchapter B of chapter V of the Federal Food, Drug,*
 12 *and Cosmetic Act (21 U.S.C. 360aa et seq.) is amended by*
 13 *inserting after section 529 the following:*

14 **“SEC. 529A. TARGETED DRUGS FOR RARE DISEASES.**

15 ~~“(a) PURPOSE.—The purpose of this section, through~~
 16 ~~the approach provided for in subsection (b), is to—~~

17 ~~“(1) facilitate the development, review, and ap-~~
 18 ~~proval of genetically targeted drugs and variant pro-~~
 19 ~~tein targeted drugs to address an unmet medical need~~
 20 ~~in one or more patient subgroups, including sub-~~
 21 ~~groups of patients with different mutations of a gene,~~
 22 ~~with respect to rare diseases or conditions that are se-~~
 23 ~~rious or life-threatening; and~~

1 “(2) maximize the use of scientific tools or meth-
 2 ods, including surrogate endpoints and other bio-
 3 markers for such purposes.

4 “(b) *LEVERAGING OF DATA FROM PREVIOUSLY AP-*
 5 *PROVED DRUG APPLICATION OR APPLICATIONS.*—*The Sec-*
 6 *retary may, consistent with applicable standards for ap-*
 7 *proval under this Act or section 351(a) of the Public Health*
 8 *Service Act, allow the sponsor of an application under sec-*
 9 *tion 505(b)(1) of this Act or section 351(a) of the Public*
 10 *Health Service Act for a genetically targeted drug or a vari-*
 11 *ant protein targeted drug to rely upon data and informa-*
 12 *tion—*

13 “(1) previously developed by the same sponsor
 14 (or another sponsor that has provided the sponsor
 15 with a contractual right of reference to such data and
 16 information); and

17 “(2) submitted by a sponsor described in para-
 18 graph (1) in support of one or more previously ap-
 19 proved applications that were submitted under section
 20 505(b)(1) of this this Act or section 351(a) of the Pub-
 21 lic Health Service Act,

22 for a drug that incorporates or utilizes the same or similar
 23 genetically targeted technology as the drug or drugs that
 24 are the subject of an application or applications described
 25 in paragraph (2) or for a variant protein targeted drug

1 *that is the same or incorporates or utilizes the same variant*
 2 *protein targeted drug, as the drug or drugs that are the*
 3 *subject of an application or applications described in para-*
 4 *graph (2).*

5 “(c) *DEFINITIONS.*—*For purposes of this section—*

6 “(1) *the term ‘genetically targeted drug’ means a*
 7 *drug that—*

8 “(A) *is the subject of an application under*
 9 *section 505(b)(1) of this Act or section 351(a) of*
 10 *the Public Health Service Act for the treatment*
 11 *of a rare disease or condition (as such term is*
 12 *defined in section 526) that is serious or life-*
 13 *threatening;*

14 “(B) *may result in the modulation (includ-*
 15 *ing suppression, up-regulation, or activation) of*
 16 *the function of a gene or its associated gene*
 17 *product; and*

18 “(C) *incorporates or utilizes a genetically*
 19 *targeted technology;*

20 “(2) *the term ‘genetically targeted technology’*
 21 *means a technology comprising non-replicating nu-*
 22 *cleic acid or analogous compounds with a common or*
 23 *similar chemistry that is intended to treat one or*
 24 *more patient subgroups, including subgroups of pa-*
 25 *tients with different mutations of a gene, with the*

1 *same disease or condition, including a disease or con-*
 2 *dition due to other variants in the same gene; and*

3 *“(3) the term ‘variant protein targeted drug’*
 4 *means a drug that—*

5 *“(A) is the subject of an application under*
 6 *section 505(b)(1) of this Act or section 351(a) of*
 7 *the Public Health Service Act for the treatment*
 8 *of a rare disease or condition (as such term is*
 9 *defined in section 526) that is serious or life-*
 10 *threatening;*

11 *“(B) modulates the function of a product of*
 12 *a mutated gene where such mutation is respon-*
 13 *sible in whole or in part for a given disease or*
 14 *condition; and*

15 *“(C) is intended to treat one or more pa-*
 16 *tient subgroups, including subgroups of patients*
 17 *with different mutations of a gene, with the same*
 18 *disease or condition.*

19 *“(d) RULE OF CONSTRUCTION.—Nothing in this sec-*
 20 *tion shall be construed to—*

21 *“(1) alter the authority of the Secretary to ap-*
 22 *prove drugs pursuant to this Act or section 351 of the*
 23 *Public Health Service Act (as authorized prior to the*
 24 *date of enactment of this section), including the*

1 *standards of evidence, and applicable conditions, for*
2 *approval under such applicable Act; or*
3 *“(2) confer any rights, beyond those authorized*
4 *under this Act or the Public Health Service Act prior*
5 *to enactment of this section, with respect to the per-*
6 *missibility of referencing information contained in*
7 *another application submitted under section 505(b)(1)*
8 *of this Act or section 351(a) of the Public Health*
9 *Service Act.”.*

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