

114TH CONGRESS  
1ST 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.

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## IN THE SENATE OF THE UNITED STATES

SEPTEMBER 15, 2015

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

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## 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,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the "Advancing Targeted  
5 Therapies for Rare Diseases Act of 2015".

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

2 Title V of the Federal Food, Drug, and Cosmetic Act  
3 (21 U.S.C. 351 et seq.) is amended by inserting after sec-  
4 tion 506F the following:

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

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

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

14 “(2) maximize the use of scientific tools or  
15 methods, including surrogate endpoints and other  
16 biomarkers for such purposes.

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

23 “(1) previously developed by the same sponsor  
24 (or another sponsor that has provided the sponsor  
25 with a contractual right of reference to such data  
26 and information); and

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

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

11           “(1) the term ‘genetically targeted drug’ means  
12           a drug which—

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

19                   “(B) incorporates or utilizes a genetically  
20                   targeted technology or a variant protein tar-  
21                   geted technology; and

22                   “(C) may result in the modulation (includ-  
23                   ing suppression, up-regulation, or activation) of  
24                   the function of a gene or its associated gene  
25                   product;

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

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

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

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

22           “(2) confer any new rights, beyond those au-  
23 thorized under this section, with respect to the per-  
24 missibility of referencing information contained in  
25 another application submitted under section

1 505(b)(1) of this Act or section 351(a) of the Public  
2 Health Service Act.”.

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