114TH CONGRESS
1ST SESSION

S. _____

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

IN THE SENATE OF THE UNITED STATES

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 _____

A BILL

To allow the sponsor of an application for the approval of a targeted drug to rely upon data and information with respect such sponsor's previously approved targeted 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 ''Advancing Targeted Therapies for Rare Diseases Act of 2015''.

SEC. 2. TARGETED DRUGS FOR RARE DISEASES.

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

"SEC. 506G. TARGETED DRUGS FOR RARE DISEASES.

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

''(1) facilitate the development, review, and approval of genetically targeted drugs to address an unmet medical need in one or more patient subgroups (or gene variant subpopulations) with respect to rare diseases or conditions that are serious or life-threatening; and

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

''(b) LEVERAGING OF DATA FROM PREVIOUSLY APPROVED DRUG APPLICATION OR APPLICATIONS.—The Secretary may, consistent with applicable standards for approval under this Act or section 351 of the Public Health Service Act, allow the sponsor of a genetically targeted drug to rely upon data and information—

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

''(2) submitted by a sponsor described in paragraph (1) in support of one or more applications previously approved under this Act or section 351 of the Public Health Service Act,

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

''(c) DEFINITIONS.—For purposes of this section—

''(1) the term 'genetically targeted drug' means a drug which—

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

''(B) incorporates or utilizes a genetically targeted technology or a variant protein targeted technology; and

''(C) may result in the modulation (including suppression, up-regulation, or activation) of the function of a gene or its associated gene product;

''(2) the term 'genetically targeted technology' means a technology comprising non-replicating nucleic acid or analogous compounds with a common or similar chemistry that is intended to treat one or more subsets of patients with the same disease, including due to other variants in the same gene; and

''(3) the term 'variant protein targeted technology' means a technology or compound that modulates the function of a variant protein, due to a gene variant, intended to treat one or more subsets of patients with the same disease, due to other variants in the same gene.

''(d) RULE OF CONSTRUCTION.—Nothing in this section shall be construed to—

''(1) alter the authority of the Secretary to approve drugs pursuant to this Act or section 351 of the Public Health Service Act (as authorized prior to the date of enactment of the Advancing Targeted Therapies for Rare Diseases Act of 2015), including the standards of evidence, and applicable conditions, for approval under such Act; or

''(2) confer any new rights, beyond those authorized under this section, with respect to the permissibility of referencing information contained in another application submitted under section

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