112TH CONGRESS
2D SESSION

S. 2281

To amend the Federal Food, Drug, and Cosmetic Act to strengthen the ability of the Food and Drug Administration to seek advice from external experts regarding rare diseases, the burden of rare diseases, and the unmet medical needs of individuals with rare diseases.

IN THE SENATE OF THE UNITED STATES

MARCH 29, 2012

Mr. WHITEHOUSE 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 amend the Federal Food, Drug, and Cosmetic Act to strengthen the ability of the Food and Drug Administration to seek advice from external experts regarding rare diseases, the burden of rare diseases, and the unmet medical needs of individuals with rare diseases.

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 ''Expanding and Promoting Expertise in Rare Treatments Act of 2012'' or the ''ExPERT Act''.

SEC. 2. FINDINGS.

Congress finds as follows:

(1) Biomedical research is yielding discoveries that are leading to the development of new therapies that hold great promise for treating disease.

(2) Scientists are increasingly unlocking the potential for targeting treatments according to genetic defect.

(3) Many of the new therapies that are under development in laboratories across the Nation are targeted to rare diseases, small subsets of diseases of significant incidence, or even small subsets of rare diseases.

(4) Progress in the development of targeted therapies, while of great promise for those with disease or disability, requires the Food and Drug Administration to develop or obtain expertise in many diseases and disease subtypes.

(5) In previous circumstances when the Food and Drug Administration has consulted with rare drug experts, the agency has been able to move new therapies to market with greater efficiency.

(6) The Food and Drug Administration benefits from this type of consultation with external experts who have a deep understanding of the diseases or disease subtypes that are targeted by new therapies.

(7) Access to external experts provides valuable advice about rare diseases or disease subtypes, disease severity, and unmet medical needs.

SEC. 3. CONSULTATION WITH EXTERNAL EXPERTS.

Subchapter E of chapter V of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb et seq.) is amended by adding at the end the following:

"SEC. 568. CONSULTATION WITH EXTERNAL EXPERTS ON RARE DISEASES, TARGETED THERAPIES, AND GENETIC TARGETING OF TREATMENTS.

''(a) IN GENERAL.—

''(1) OPPORTUNITIES FOR CONSULTATION.—The Secretary shall ensure that opportunities exist, at a time the Secretary determines appropriate, for consultation with external experts on the topics described in subsection (c), for the purpose of promoting the efficiency of and informing the review by the Food and Drug Administration of drugs and biologic products for rare diseases and drugs and biologic products that are genetically targeted.

''(2) CONSULTATION.—The Center for Drug Evaluation and Research and the Center for Biologics Evaluation and Research shall, when appropriate, seek the opinion of external experts on any topic, including the topics described in subsection

(c), by initiating contact with such experts. External experts may also request the opportunity to meet with a review division regarding any topic described in subsection (c).

''(b) EXTERNAL EXPERTS.—The external experts under subsection (a) may include—

''(1) representatives of patient, consumer, research, and health professional organizations with expertise relevant to the review of rare disease products;

''(2) experts on rare diseases, rare subtypes of rare and other diseases, and genetic targeting of treatments, including experts from academia; and

''(3) experts in innovative clinical trial designs for small target populations.

''(c) TOPICS FOR CONSULTATION.—Topics for consultation may include—

''(1) rare diseases;

''(2) the severity of rare diseases;

''(3) the unmet medical need associated with rare diseases;

''(4) the willingness and ability of individuals with a rare disease to participate in clinical trials;

''(5) an assessment of the benefits and risks, including side effects, of current and investigational therapies;

''(6) the design of clinical trials for rare disease populations and subpopulations, including regulatory and scientific policies affecting the design of such trials; and

''(7) demographics and the clinical description of patient populations.

''(d) CLASSIFICATION AS SPECIAL GOVERNMENT EMPLOYEES.—The external experts who are consulted under this section may be considered special government employees, as defined under section 202 of title 18, United States Code.

''(e) PROPRIETARY INFORMATION.—Nothing in this section shall be construed to create a right for any external expert, as described in subsection (b), to obtain access to proprietary information of a sponsor without the permission of such sponsor.

''(f) NO RIGHT OR OBLIGATION.—Nothing in this section shall be construed to create a legal right for a consultation on any matter or require the Secretary to meet with any particular expert.''.

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