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112TH CONGRESS
2D SESSION

H. R. 4156

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 HOUSE OF REPRESENTATIVES

MARCH 7, 2012

Mr. MARKEY (for himself, Mr. MARINO, and Mr. STEARNS) introduced the following bill; which was referred to the Committee on Energy and Commerce

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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 Review of Rare Treatments Act of 2012'' or ''EXPERRT Act of 2012''.

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, poses challenges for the Food and Drug Administration, as the agency is asked to develop or obtain expertise in many diseases and disease subtypes.

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

(6) External experts could provide 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.—The Secretary shall establish a program for consultation with external experts on the topics described in subsection (d), for the purpose of informing and strengthening the Food and Drug Agency's review of drugs and biologic products for rare diseases and drugs and biologic products that are genetically targeted.

''(b) PROGRAM OPERATION.—Under the program established under subsection (a), each review division within 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 described in subsection (d) by initiating contact with such experts. The external experts may also request the opportunity to meet with a review division regarding any topic described in subsection (d).

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

''(1) representatives of patient, consumer, research, and health professional organizations;

''(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.

''(d) 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; and

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

''(e) TIMING OF CONSULTATION.—The Secretary may determine the timing of each consultation with the external experts, which may occur prior to, or following, the filing of an investigational new drug application under section 505(i), a new drug application under section

505(b), or a biologics license application under section 351 of the Public Health Service Act.

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

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