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113TH CONGRESS
2D SESSION

H. R. 5805

To amend the Federal Food, Drug, and Cosmetic Act with respect to expanding access for breakthrough drugs, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

DECEMBER 8, 2014

Mr. MCCAUL 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 with respect to expanding access for breakthrough drugs, and for other purposes.

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 ''Andrea Sloan Compassionate Use Reform and Enhancement Act'' or the ''Andrea Sloan CURE Act''.

SEC. 2. EXPANDED ACCESS POLICY AS CONDITION OF EXPEDITED APPROVAL.

Section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) is amended—

(1) by redesignating subsections (d) and (e) as subsections (e) and (f), respectively; and

(2) by inserting after subsection (c) the following new subsection:

''(d) EXPANDED ACCESS POLICY REQUIRED FOR COVERED BREAKTHROUGH DRUGS.—

''(1) IN GENERAL.—With respect to a qualified breakthrough drug, not later than 30 days after the date on which the drug meets the definition of a covered breakthrough drug (as specified in paragraph (2)), the sponsor of the covered breakthrough drug shall submit to the Secretary and make publicly available the policy of the sponsor with respect to requests submitted under subsection (b). In the case of such a policy under which the sponsor accepts such requests, such policy shall include—

''(A) a single point of contact who receives and processes such requests;

''(B) procedures for making such requests;

''(C) the minimum criteria for the sponsor's consideration or approval of such requests; and

''(D) the amount of time the sponsor anticipates will be necessary to make a decision on such requests.

''(2) COVERED BREAKTHROUGH DRUG.—In this subsection, the term 'covered breakthrough drug' means a drug—

''(A) that is designated as a breakthrough therapy or as a fast track product or is approved under accelerated approval under section 506;

''(B) that is designated under section 505E(d) as a qualified infectious disease product; or

''(C) the sponsor of which is awarded a priority review voucher under section 524 or 529.''.

SEC. 3. NOTIFICATION OF SUBMITTERS OF COMPASSIONATE USE REQUESTS.

Section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb), as amended by section 2, is further amended—

(1) by redesignating subsections (e) and (f) (as redesignated by section 2(1)) as subsections (f) and (g), respectively; and

(2) by inserting after subsection (d) (as inserted by section 2(2)) the following new subsection:

''(e) NOTIFICATION OF SUBMITTERS OF REQUESTS.—In the case of the denial by a manufacturer or

distributor of a request under subsection (b), not later than 5 days after the date of such denial, the manufacturer or distributor, as applicable, shall submit to the person (or physician) who made the request written notice of the denial, including an explanation for the denial.''.

SEC. 4. GAO QUALITATIVE ANALYSIS ON INDIVIDUAL PATIENT ACCESS TO UNAPPROVED THERAPIES AND DIAGNOSTICS.

Not later than 180 days after the date of the enactment of this Act and each year thereafter, the Comptroller General of the United States shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor and Pensions of the Senate a report containing a qualitative analysis of the extent to which individual patients have access to investigational drugs pursuant to subsection (b) of section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) and recommendations for improving such access. In preparing such report, the Comptroller General shall conduct a qualitative analysis of the following:

(1) Whether there are any identifiable patterns in requests submitted under subsection (b) of such section, such as the types of indications for which

requests for individual patient access are sought or the reasons for the denial of such requests.

(2) What the primary barriers are to drug sponsors granting requests for individual patient access.

(3) How the Secretary evaluates safety and efficacy data submitted in connection with such requests.

(4) The amount of time that—

(A) a physician typically takes to complete the paperwork necessary to make such a request;

(B) a drug sponsor takes to process such a request and to issue a decision with respect to the request; and

(C) the Secretary takes to process such a request and to issue a decision with respect to the request.

(5) How regulations, guidance, policies, or practices may be modified, streamlined, expanded, or discontinued to reduce or prevent delays in approving such requests.

(6) The number of such requests that, for the period covered by the report—

(A) were approved by drug sponsors and the Food and Drug Administration;

(B) were approved by drug sponsors but denied by the Food and Drug Administration; and

(C) were denied by drug sponsors.

(7) How to encourage drug sponsors to grant requests for expanded access under such section 561, including requests for emergency use, intermediate-size patient populations, and large patient populations under a specified indication.

(8) Whether and to what extent adverse events reported to the Secretary as a result of individual use of an investigational drug or investigational device under such section 561 affected the development or approval of any drug or device.

SEC. 5. EXPANDED ACCESS TASK FORCE.

(a) ESTABLISHMENT.—The Secretary of Health and Human Services shall establish a task force within the Department of Health and Human Services to explore mechanisms for improving the access individual patients have to investigational drugs pursuant to subsection (b) of section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb), to be known as the ''Expanded Access Task Force'' (in this section referred to as the ''Task

Force''). Not later than 90 days after the date on which the Comptroller General of the United States submits the first report required under section 4, the Task Force shall be convened.

(b) MEMBERSHIP.—

(1) COMPOSITION.—The Task Force shall be composed of not more than 9 voting members appointed as follows:

(A) One member to serve as Chairman of the Task Force, appointed by the Speaker of the House of Representatives.

(B) One representative from the Department of Health and Human Services, appointed by the Secretary of Health and Human Services.

(C) Four representatives appointed by the Majority Leader of the House of Representatives, in consultation with the Minority Leader of the House of Representatives, and the Chairman and the Ranking Member of the Committee on Energy and Commerce of the House of Representatives, including—

(i) one representative of a biopharmaceutical company of less than 250 full-time employees;

(ii) one representative of the rare disease patient community;

(iii) one representative of the health care provider community; and

(iv) one bioethicist.

(D) Three representatives appointed by Majority Leader of the Senate, in consultation with the Minority Leader of the Senate, and the Chairman and the Ranking Member of the Health, Education, Labor and Pensions Committee of the Senate, including—

(i) one representative of the biopharmaceutical industry;

(ii) one representative of the patient community; and

(iii) one representative of the health care payor community.

(2) COMPENSATION.—Members of the Task Force shall serve without compensation.

(c) DUTIES.—The Task Force shall comprehensively evaluate the access individual patients have to investigational drugs pursuant to subsection (b) of section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb), taking into account—

(1) the unique challenges faced by children with likely fatal diseases for which there is not a comparable or satisfactory alternative therapy available;

(2) possible incentives for biopharmaceutical companies and providers to approve requests submitted under such subsection;

(3) how the Secretary of Health and Human Services interprets and takes into consideration adverse event data reported in the case of data from use under a request submitted under such subsection;

(4) ways to streamline and standardize the process for submitting requests under such subsection; and

(5) the costs incurred by biopharmaceutical companies for the time, effort, and delivery of investigational drugs to patients for the diagnosis, monitoring, or treatment of a serious disease or condition under such subsection.

(d) REPORT.—Not later than 180 days after the date on which the Task Force is convened, the Task Force shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor and Pensions of the Senate a report in an electronic format describing the specific recommenda-

tions of the Task Force for improving the access individual patients have to investigational drugs pursuant to subsection (b) of section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb).

(e) TERMINATION.—The task force shall terminate upon submission of the report required under subsection (d).

SEC. 6. FINALIZING DRAFT GUIDANCE ON EXPANDED ACCESS.

(a) IN GENERAL.—Not later than 180 days after the date on which the Expanded Access Task Force established under section 5 submits the report under subsection (d) of such section, the Secretary of Health and Human Services shall finalize the draft guidance entitled ''Expanded Access to Investigational Drugs for Treatment Use—Qs & As'' and dated May 2013.

(b) CONTENTS.—The final guidance referred to in subsection (a) shall—

(1) clearly define how the Secretary interprets and uses adverse drug event data reported by investigators in the case of data reported from use under a request submitted under section 561(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb(b)); and

(2) take into account the report of the Expanded Access Task Force submitted under section 5(d) and the first report of the Comptroller General of the United States submitted under section 4.

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