

113TH CONGRESS
2^D 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

A BILL

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

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 “Andrea Sloan Compas-
5 sionate Use Reform and Enhancement Act” or the “An-
6 drea Sloan CURE Act”.

7 **SEC. 2. EXPANDED ACCESS POLICY AS CONDITION OF EX-**
8 **PEDITED APPROVAL.**

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

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

3 (2) by inserting after subsection (c) the fol-
4 lowing new subsection:

5 “(d) EXPANDED ACCESS POLICY REQUIRED FOR
6 COVERED BREAKTHROUGH DRUGS.—

7 “(1) IN GENERAL.—With respect to a qualified
8 breakthrough drug, not later than 30 days after the
9 date on which the drug meets the definition of a cov-
10 ered breakthrough drug (as specified in paragraph
11 (2)), the sponsor of the covered breakthrough drug
12 shall submit to the Secretary and make publicly
13 available the policy of the sponsor with respect to re-
14 quests submitted under subsection (b). In the case
15 of such a policy under which the sponsor accepts
16 such requests, such policy shall include—

17 “(A) a single point of contact who receives
18 and processes such requests;

19 “(B) procedures for making such requests;

20 “(C) the minimum criteria for the spon-
21 sor’s consideration or approval of such requests;

22 and

23 “(D) the amount of time the sponsor an-
24 ticipates will be necessary to make a decision on
25 such requests.

1 “(2) COVERED BREAKTHROUGH DRUG.—In this
2 subsection, the term ‘covered breakthrough drug’
3 means a drug—

4 “(A) that is designated as a breakthrough
5 therapy or as a fast track product or is ap-
6 proved under accelerated approval under section
7 506;

8 “(B) that is designated under section
9 505E(d) as a qualified infectious disease prod-
10 uct; or

11 “(C) the sponsor of which is awarded a
12 priority review voucher under section 524 or
13 529.”.

14 **SEC. 3. NOTIFICATION OF SUBMITTERS OF COMPAS-**
15 **SIONATE USE REQUESTS.**

16 Section 561 of the Federal Food, Drug, and Cosmetic
17 Act (21 U.S.C. 360bbb), as amended by section 2, is fur-
18 ther amended—

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

22 (2) by inserting after subsection (d) (as in-
23 serted by section 2(2)) the following new subsection:

24 “(e) NOTIFICATION OF SUBMITTERS OF RE-
25 QUESTS.—In the case of the denial by a manufacturer or

1 distributor of a request under subsection (b), not later
2 than 5 days after the date of such denial, the manufac-
3 turer or distributor, as applicable, shall submit to the per-
4 son (or physician) who made the request written notice
5 of the denial, including an explanation for the denial.”.

6 **SEC. 4. GAO QUALITATIVE ANALYSIS ON INDIVIDUAL PA-**
7 **TIENT ACCESS TO UNAPPROVED THERAPIES**
8 **AND DIAGNOSTICS.**

9 Not later than 180 days after the date of the enact-
10 ment of this Act and each year thereafter, the Comptroller
11 General of the United States shall submit to the Com-
12 mittee on Energy and Commerce of the House of Rep-
13 resentatives and the Committee on Health, Education,
14 Labor and Pensions of the Senate a report containing a
15 qualitative analysis of the extent to which individual pa-
16 tients have access to investigational drugs pursuant to
17 subsection (b) of section 561 of the Federal Food, Drug,
18 and Cosmetic Act (21 U.S.C. 360bbb) and recommenda-
19 tions for improving such access. In preparing such report,
20 the Comptroller General shall conduct a qualitative anal-
21 ysis of the following:

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

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

3 (2) What the primary barriers are to drug
4 sponsors granting requests for individual patient ac-
5 cess.

6 (3) How the Secretary evaluates safety and effi-
7 cacy data submitted in connection with such re-
8 quests.

9 (4) The amount of time that—

10 (A) a physician typically takes to complete
11 the paperwork necessary to make such a re-
12 quest;

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

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

19 (5) How regulations, guidance, policies, or prac-
20 tices may be modified, streamlined, expanded, or dis-
21 continued to reduce or prevent delays in approving
22 such requests.

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

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

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

6 (C) were denied by drug sponsors.

7 (7) How to encourage drug sponsors to grant
8 requests for expanded access under such section
9 561, including requests for emergency use, inter-
10 mediate-size patient populations, and large patient
11 populations under a specified indication.

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

17 **SEC. 5. EXPANDED ACCESS TASK FORCE.**

18 (a) ESTABLISHMENT.—The Secretary of Health and
19 Human Services shall establish a task force within the De-
20 partment of Health and Human Services to explore mech-
21 anisms for improving the access individual patients have
22 to investigational drugs pursuant to subsection (b) of sec-
23 tion 561 of the Federal Food, Drug, and Cosmetic Act
24 (21 U.S.C. 360bbb), to be known as the “Expanded Ac-
25 cess Task Force” (in this section referred to as the “Task

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

5 (b) MEMBERSHIP.—

6 (1) COMPOSITION.—The Task Force shall be
7 composed of not more than 9 voting members ap-
8 pointed as follows:

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

12 (B) One representative from the Depart-
13 ment of Health and Human Services, appointed
14 by the Secretary of Health and Human Serv-
15 ices.

16 (C) Four representatives appointed by the
17 Majority Leader of the House of Representa-
18 tives, in consultation with the Minority Leader
19 of the House of Representatives, and the Chair-
20 man and the Ranking Member of the Com-
21 mittee on Energy and Commerce of the House
22 of Representatives, including—

23 (i) one representative of a biopharma-
24 ceutical company of less than 250 full-time
25 employees;

1 (ii) one representative of the rare dis-
2 ease patient community;

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

5 (iv) one bioethicist.

6 (D) Three representatives appointed by
7 Majority Leader of the Senate, in consultation
8 with the Minority Leader of the Senate, and the
9 Chairman and the Ranking Member of the
10 Health, Education, Labor and Pensions Com-
11 mittee of the Senate, including—

12 (i) one representative of the bio-
13 pharmaceutical industry;

14 (ii) one representative of the patient
15 community; and

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

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

20 (c) DUTIES.—The Task Force shall comprehensively
21 evaluate the access individual patients have to investiga-
22 tional drugs pursuant to subsection (b) of section 561 of
23 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
24 360bbb), taking into account—

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

4 (2) possible incentives for biopharmaceutical
5 companies and providers to approve requests sub-
6 mitted under such subsection;

7 (3) how the Secretary of Health and Human
8 Services interprets and takes into consideration ad-
9 verse event data reported in the case of data from
10 use under a request submitted under such sub-
11 section;

12 (4) ways to streamline and standardize the
13 process for submitting requests under such sub-
14 section; and

15 (5) the costs incurred by biopharmaceutical
16 companies for the time, effort, and delivery of inves-
17 tigational drugs to patients for the diagnosis, moni-
18 toring, or treatment of a serious disease or condition
19 under such subsection.

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

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

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

8 **SEC. 6. FINALIZING DRAFT GUIDANCE ON EXPANDED AC-**
9 **CESS.**

10 (a) IN GENERAL.—Not later than 180 days after the
11 date on which the Expanded Access Task Force estab-
12 lished under section 5 submits the report under subsection
13 (d) of such section, the Secretary of Health and Human
14 Services shall finalize the draft guidance entitled “Ex-
15 panded Access to Investigational Drugs for Treatment
16 Use—Qs & As” and dated May 2013.

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

19 (1) clearly define how the Secretary interprets
20 and uses adverse drug event data reported by inves-
21 tigators in the case of data reported from use under
22 a request submitted under section 561(b) of the
23 Federal Food, Drug, and Cosmetic Act (21 U.S.C.
24 360bbb(b)); and

1 (2) take into account the report of the Ex-
2 panded Access Task Force submitted under section
3 5(d) and the first report of the Comptroller General
4 of the United States submitted under section 4.

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