

114TH CONGRESS
1ST SESSION

H. R. 909

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

FEBRUARY 12, 2015

Mr. McCAUL (for himself, Mr. BUTTERFIELD, Mr. BURGESS, Mr. GRIFFITH, Ms. MATSUI, and Mr. LANCE) 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 Compass-
5 sionate Use Reform and Enhancement Act” or the “An-
6 drea Sloan CURE Act”.

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

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

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

7 (2) by inserting after subsection (c) the fol-
8 lowing new subsection:

9 “(d) EXPANDED ACCESS POLICY REQUIRED FOR
10 COVERED INVESTIGATIONAL DRUGS.—

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

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

23 “(B) procedures for making such requests;

24 “(C) the general criteria for the sponsor’s
25 consideration or approval of such requests; and

1 “(D) the amount of time the sponsor an-
2 ticipates will be necessary to respond to such
3 requests.

4 “(2) COVERED INVESTIGATIONAL DRUG.—In
5 this subsection, the term ‘covered investigational
6 drug’ means a drug that—

7 “(A) is designated as a breakthrough ther-
8 apy or as a fast track product;

9 “(B) is designated under section 505E(d)
10 as a qualified infectious disease product; or

11 “(C) is designated under section 526 as a
12 drug for a rare disease or condition.”.

13 **SEC. 3. NOTIFICATION OF SUBMITTERS OF EXPANDED AC-**
14 **CESS REQUESTS.**

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

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

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

23 “(e) NOTIFICATION OF SUBMITTERS OF RE-
24 QUESTS.—In the case of the denial by a manufacturer or
25 distributor of a request under subsection (b), not later

1 than 5 days after the date of such denial, the manufac-
2 turer or distributor, as applicable, shall submit to the per-
3 son (or physician) who made the request written notice
4 of the denial, including an explanation for the denial.”.

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

8 Not later than 180 days after the date of the enact-
9 ment of this Act and every two years thereafter through
10 2023, the Comptroller General of the United States shall
11 submit to the Committee on Energy and Commerce of the
12 House of Representatives and the Committee on Health,
13 Education, Labor and Pensions of the Senate a report
14 containing a qualitative analysis of the extent to which in-
15 dividual patients have access to investigational drugs pur-
16 suant to subsection (b) of section 561 of the Federal Food,
17 Drug, and Cosmetic Act (21 U.S.C. 360bbb) and rec-
18 ommendations for improving such access. In preparing
19 such report, the Comptroller General shall conduct a qual-
20 itative analysis of the following:

21 (1) Whether there are any identifiable patterns
22 in requests submitted under subsection (b) of such
23 section, such as the types of indications for which
24 requests for individual patient access are sought or
25 the reasons for the denial of such requests.

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

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

7 (4) The amount of time that—

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

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

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

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

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

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

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

4 (C) were denied by drug sponsors.

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

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

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

16 (a) ESTABLISHMENT.—The Secretary of Health and
17 Human Services shall establish a task force within the De-
18 partment of Health and Human Services to explore mech-
19 anisms for improving the access individual patients have
20 to investigational drugs pursuant to subsection (b) of sec-
21 tion 561 of the Federal Food, Drug, and Cosmetic Act
22 (21 U.S.C. 360bbb), to be known as the “Expanded Ac-
23 cess Task Force” (in this section referred to as the “Task
24 Force”). Not later than 90 days after the date on which
25 the Comptroller General of the United States submits the

1 first report required under section 4, the Task Force shall
2 be convened.

3 (b) MEMBERSHIP.—

4 (1) COMPOSITION.—The Task Force shall be
5 composed of not more than 13 voting members ap-
6 pointed as follows:

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

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

14 (C) Six representatives appointed by the
15 majority leader of the House of Representa-
16 tives, in consultation with the minority leader of
17 the House of Representatives, and the chairman
18 and the ranking member of the Committee on
19 Energy and Commerce of the House of Rep-
20 resentatives, including—

21 (i) one current or former representa-
22 tive of the biopharmaceutical industry of
23 not less than 250 full-time employees;

1 (ii) one representative of a biopharma-
2 ceutical company of less than 250 full-time
3 employees;

4 (iii) one representative of the patient
5 community;

6 (iv) one representative of the rare dis-
7 ease patient community;

8 (v) one representative of the health
9 care provider community; and

10 (vi) one bioethicist.

11 (D) Five representatives appointed by ma-
12 jority leader of the Senate, in consultation with
13 the minority leader of the Senate, and the
14 chairman and the ranking member of the Com-
15 mittee on Health, Education, Labor and Pen-
16 sions of the Senate, including—

17 (i) one representative of the bio-
18 pharmaceutical industry of not less than
19 250 full-time employees;

20 (ii) one current or former representa-
21 tive of a biopharmaceutical company of
22 less than 250 full-time employees;

23 (iii) one representative of the patient
24 community;

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

3 (v) one representative of the health
4 care payor community.

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

7 (c) DUTIES.—The Task Force shall comprehensively
8 evaluate the access individual patients have to investiga-
9 tional drugs pursuant to subsection (b) of section 561 of
10 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.
11 360bbb), taking into account—

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

15 (2) possible incentives for biopharmaceutical
16 companies and providers to approve requests sub-
17 mitted under such subsection;

18 (3) ways to improve followup reporting of ad-
19 verse event data and compliance with such reporting
20 requirements;

21 (4) how the Secretary of Health and Human
22 Services interprets and takes into consideration ad-
23 verse event data reported in the case of data from
24 use under a request submitted under such sub-
25 section;

1 (5) ways to streamline and standardize the
2 process for submitting requests under such sub-
3 section; and

4 (6) the costs incurred by biopharmaceutical
5 companies for the time, effort, and delivery of inves-
6 tigational drugs to patients for the diagnosis, moni-
7 toring, or treatment of a serious disease or condition
8 under such subsection.

9 (d) REPORT.—Not later than 180 days after the date
10 on which the Task Force is convened, the Task Force shall
11 submit to the Committee on Energy and Commerce of the
12 House of Representatives and the Committee on Health,
13 Education, Labor and Pensions of the Senate a report in
14 an electronic format describing the specific recommenda-
15 tions of the Task Force for improving the access individual
16 patients have to investigational drugs pursuant to sub-
17 section (b) of section 561 of the Federal Food, Drug, and
18 Cosmetic Act (21 U.S.C. 360bbb).

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

22 **SEC. 6. FINALIZING DRAFT GUIDANCE ON EXPANDED AC-**
23 **CESS.**

24 (a) IN GENERAL.—Not later than 180 days after the
25 date on which the Expanded Access Task Force estab-

1 lished under section 5 submits the report under subsection
2 (d) of such section, the Secretary of Health and Human
3 Services shall finalize the draft guidance entitled “Ex-
4 panded Access to Investigational Drugs for Treatment
5 Use—Qs & As” and dated May 2013.

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

8 (1) clearly define how the Secretary interprets
9 and uses adverse drug event data reported by inves-
10 tigators in the case of data reported from use under
11 a request submitted under section 561(b) of the
12 Federal Food, Drug, and Cosmetic Act (21 U.S.C.
13 360bbb(b)); and

14 (2) take into account the report of the Ex-
15 panded Access Task Force submitted under section
16 5(d) and the first report of the Comptroller General
17 of the United States submitted under section 4.

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