

112TH CONGRESS
1ST SESSION

S. _____

To amend the Federal Food, Drug, and Cosmetic Act to improve the priority review voucher incentive program relating to tropical and rare pediatric diseases.

IN THE SENATE OF THE UNITED STATES

Mr. CASEY (for himself, Mr. BROWN of Massachusetts, Mr. BROWN of Ohio, Mr. ISAKSON, and Mr. FRANKEN) introduced the following bill; which was read twice and referred to the Committee on _____

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to improve the priority review voucher incentive program relating to tropical and rare pediatric diseases.

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; REFERENCES.**

4 (a) SHORT TITLE.—This Act may be cited as the
5 “Creating Hope Act of 2011”.

6 (b) REFERENCES.—Wherever in this Act an amend-
7 ment is expressed in terms of an amendment to a section
8 or other provision, the reference shall be considered to be

1 made to a section or other provision of the Federal Food,
2 Drug, and Cosmetic Act (21 U.S.C. 301 et seq.).

3 **SEC. 2. IMPROVEMENT OF THE TROPICAL DISEASE VOUCH-**
4 **ER PROGRAM.**

5 (a) **HEADING.**—The heading of section 524 (21
6 U.S.C. 360n) is amended to read as follows: “**PRIORITY**
7 **REVIEW TO ENCOURAGE INNOVATIVE TREATMENTS**
8 **FOR TROPICAL DISEASES AND RARE PEDIATRIC**
9 **DISEASES**”.

10 (b) **DEFINITIONS.**—Section 524(a) (21 U.S.C.
11 360n(a)) is amended—

12 (1) by redesignating paragraphs (3) and (4) as
13 paragraphs (6) and (7), respectively;

14 (2) by redesignating paragraphs (1) and (2) as
15 paragraphs (2) and (3), respectively;

16 (3) by inserting after “In this section:”, the fol-
17 lowing:

18 “(1) **INNOVATIVE TREATMENT.**—The term ‘in-
19 novative treatment’ means—

20 “(A) a human drug that is the subject of
21 an application submitted under section
22 505(b)(1), if that drug contains no active ingre-
23 dient (including any ester or salt of the active
24 ingredient) that has been previously approved
25 in any other application under section

1 505(b)(1), 505(b)(2), or 505(j) or section 351
2 of the Public Health Service Act; or

3 “(B) a biological product that is the sub-
4 ject of an application submitted under section
5 351(a) of the Public Health Service Act, if that
6 biological product—

7 “(i) does not have the same structure
8 as a biological product that has been pre-
9 viously licensed in any other application
10 under subsection (a) or (k) of section 351
11 of the Public Health Service Act or ap-
12 proved under section 505 of this Act; and

13 “(ii) is not biosimilar, within the
14 meaning of section 351(i) of the Public
15 Health Service Act, to a biological product
16 that has been previously licensed in any
17 other application under subsection (a) or
18 (k) of section 351 of the Public Health
19 Service Act or approved under section 505
20 of this Act.”.

21 (4) in paragraph (3), as so redesignated, by in-
22 serting “or rare pediatric disease product applica-
23 tion” after “tropical disease product application”
24 each place that phrase appears;

1 (5) by inserting after paragraph (3) the fol-
2 lowing:

3 “(4) RARE PEDIATRIC DISEASE.—The term
4 ‘rare pediatric disease’ means a disease that meets
5 each of the following criteria:

6 “(A) The disease is recognized in the med-
7 ical community as affecting a pediatric popu-
8 lation.

9 “(B) The disease is a rare disease or con-
10 dition, within the meaning of section 526.

11 “(5) RARE PEDIATRIC DISEASE PRODUCT AP-
12 PLICATION.—The term ‘rare pediatric disease prod-
13 uct application’ means a human drug application, as
14 defined in section 735(1)—

15 “(A) for prevention or treatment of a rare
16 pediatric disease;

17 “(B) that the Secretary deems eligible for
18 priority review;

19 “(C) that is for an innovative treatment;

20 “(D) that relies on clinical data derived
21 from studies examining a pediatric population
22 and dosages of the drug intended for that popu-
23 lation; and

1 “(E) that does not seek approval for an
2 adult indication in the original rare pediatric
3 disease product application.”;

4 (6) in paragraph (6), as so redesignated—

5 (A) by redesignating subparagraph (Q) as
6 subparagraph (R); and

7 (B) by inserting after subparagraph (P)
8 the following:

9 “(Q) Chagas Disease.”; and

10 (7) by amending paragraph (7), as so redesignated,
11 to read as follows:

12 “(7) TROPICAL DISEASE PRODUCT APPLICATION.—The term ‘tropical disease product application’ means a human drug application, as defined in
13 section 735(1)—
14 section 735(1)—
15 section 735(1)—

16 “(A) for prevention or treatment of a tropical
17 disease;

18 “(B) that the Secretary deems eligible for
19 priority review;

20 “(C) that is for an innovative treatment;
21 and

22 “(D) that the sponsor affirms in the application
23 is for a drug that has not been approved
24 for commercial marketing for any tropical disease
25 indication by a government authority out-

1 side of the United States for more than 24
2 months before the tropical disease product ap-
3 plication is submitted.”.

4 (c) RULES REGARDING USE AND TRANSFER OF PRI-
5 ORITY REVIEW VOUCHERS.—Section 524(b) (21 U.S.C.
6 360n(b)) is amended—

7 (1) in paragraph (1), by inserting “or rare pe-
8 diatric disease product application” after “tropical
9 disease product application” each place that phrase
10 appears;

11 (2) by amending paragraph (2) to read as fol-
12 lows:

13 “(2) TRANSFERABILITY.—

14 “(A) IN GENERAL.—The sponsor of a trop-
15 ical disease product application or rare pediatric
16 disease product application that receives a pri-
17 ority review voucher under this section may
18 transfer (including by sale) the entitlement to
19 such voucher. There is no limit on the number
20 of times a priority review voucher may be trans-
21 ferred before such voucher is used.

22 “(B) CONDITIONS OF TRANSFER.—If a
23 sponsor transfers a priority review voucher
24 after such sponsor has provided notification to
25 the Secretary under paragraph (4)(A) of the in-

1 tent of such sponsor to use the voucher, the
2 transfer shall be subject to the provisions of
3 subparagraphs (B) and (C) of paragraph (4).

4 “(C) NOTIFICATION OF TRANSFER.—The
5 person to whom a voucher is transferred under
6 paragraph (4)(B)(i) shall notify the Secretary
7 of such change in ownership of the voucher not
8 later than 30 days after such transfer.”;

9 (3) by amending paragraph (3) to read as fol-
10 lows:

11 “(3) LIMITATION FOR PRIOR APPLICATIONS.—

12 “(A) TROPICAL DISEASE PRODUCT APPLI-
13 CATIONS.—A sponsor of a tropical disease prod-
14 uct application may not receive a priority review
15 voucher under this section if the tropical dis-
16 ease product application was submitted to the
17 Secretary prior to September 27, 2007.

18 “(B) RARE PEDIATRIC DISEASE PRODUCT
19 APPLICATIONS.—A sponsor of a rare pediatric
20 disease product application may not receive a
21 priority review voucher under this section if the
22 rare pediatric disease product application was
23 submitted to the Secretary prior to the date
24 that is 90 days after the date of enactment of
25 the Creating Hope Act of 2011.”; and

1 (4) by amending paragraph (4) to read as fol-
2 lows:

3 “(4) NOTIFICATION.—

4 “(A) TIMING.—At least 90 days before the
5 date on which a human drug application for
6 which the sponsor intends to use a priority re-
7 view voucher is submitted, the sponsor of such
8 human drug application shall notify the Sec-
9 retary of the intent of such sponsor to submit
10 the human drug application.

11 “(B) TRANSFER OF VOUCHER AFTER NO-
12 TIFICATION.—

13 “(i) IN GENERAL.—The sponsor of a
14 human drug application that provides noti-
15 fication of the intent of such sponsor to
16 use the voucher for the human drug appli-
17 cation may transfer the voucher within 1
18 year after such notification is provided, if
19 such sponsor has not yet submitted the
20 human drug application described in the
21 notification.

22 “(ii) EXCEPTION.—The person to
23 whom a voucher is transferred under
24 clause (i) (referred to in this paragraph as
25 the ‘transferee’) shall give notification of

1 the intent of such transferee to use the
2 voucher in accordance with this subsection,
3 unless—

4 “(I) the transferee uses the
5 voucher for a human drug application
6 including the same indications as the
7 human drug application described in
8 the transferor’s notification; and

9 “(II) the transferee notifies the
10 Secretary within 30 days of the trans-
11 fer of the intent of such transferee to
12 use the voucher for such purpose.

13 “(iii) INTERNAL TRANSFER.—If the
14 sponsor transfers a voucher internally for
15 use with a drug application including one
16 or more indications that were not included
17 in the drug application that was the sub-
18 ject of the notification of such sponsor, the
19 sponsor shall notify the Secretary of the
20 transfer in accordance with this subsection.

21 “(C) FEE DUE UPON NOTIFICATION; CRED-
22 IT FOR TRANSFERRED VOUCHER.—

23 “(i) DUE UPON NOTIFICATION.—The
24 notification under this subsection shall be
25 a legally binding commitment to pay for

1 the user fee to be assessed in accordance
2 with this section. Such fee shall be payable
3 by the sponsor upon the submission by
4 such sponsor of such notification.

5 “(ii) CREDIT.—If a sponsor pays a
6 user fee upon providing notification of the
7 intent of such sponsor to use a priority re-
8 view voucher, but later transfers the vouch-
9 er for which such sponsor gave notifica-
10 tion, the Secretary shall credit the user
11 fees paid to the next human drug applica-
12 tion for which a sponsor provides notifica-
13 tion of the intent of such sponsor to use
14 the same transferred voucher.

15 “(iii) DIFFERENCE IN FEE.—The Sec-
16 retary may require a sponsor using a
17 transferred voucher to pay the difference
18 between the credit associated with the
19 transferred voucher and the user fee pre-
20 vailing at the time the sponsor submits no-
21 tification of the intent of such sponsor to
22 use the transferred voucher. This provision
23 does not apply in cases where a transferee
24 is exempted from submitting notification
25 under this paragraph.”.

1 (d) PAYMENT.—Section 524(c)(4) (21 U.S.C.
2 360n(c)(4)) is amended—

3 (1) in subparagraph (A), by striking “submis-
4 sion of a human drug application under section
5 505(b)(1) or section 351 of the Public Health Serv-
6 ices Act for which the priority review voucher is
7 used.” and inserting “notification by a sponsor of
8 the intent of such sponsor to use the voucher, as
9 specified in subsection (b)(4)(A). All other user fees
10 associated with the human drug application shall be
11 due as required by the Secretary or under applicable
12 law.”; and

13 (2) in subparagraph (C), by striking the period
14 at the end and inserting “, except as specified in
15 subsection (b)(4)(C).”.

16 (e) DESIGNATION PROCESS; PRODUCT IMPLEMENTA-
17 TION REQUIREMENT.—Section 524 (21 U.S.C. 360n) is
18 amended by adding at the end the following new sub-
19 sections:

20 “(e) DESIGNATION PROCESS.—

21 “(1) DESIGNATION OF RARE PEDIATRIC DIS-
22 EASES.—

23 “(A) IN GENERAL.—Upon the request of
24 the manufacturer or the sponsor of a new drug,
25 the Secretary may designate that the new drug

1 is for a rare pediatric disease. Such a request
2 for designation, if sought, shall be made when
3 requesting designation of orphan disease status
4 under section 526 or fast-track designation
5 under section 506. Requesting designation of
6 rare pediatric disease status under this para-
7 graph is not a prerequisite to receiving a pri-
8 ority review voucher.

9 “(B) DETERMINATION BY SECRETARY.—
10 Not later than 60 days after a request is sub-
11 mitted under subparagraph (A), the Secretary
12 shall determine whether the disease or condition
13 that is the subject of such request is a rare pe-
14 diatric disease.

15 “(2) DESIGNATION OF INNOVATIVE TREAT-
16 MENTS.—

17 “(A) IN GENERAL.—Upon the request of
18 the manufacturer or the sponsor of a new drug,
19 the Secretary may designate that a new drug is
20 an innovative treatment. Such a request for
21 designation, if sought, shall be made when re-
22 questing fast-track designation under section
23 506. Requesting designation that a new drug is
24 an innovative treatment is not a prerequisite to
25 receiving a priority review voucher.

1 “(B) DETERMINATION BY SECRETARY.—
2 Not later than 60 days after a request is sub-
3 mitted under subparagraph (A), the Secretary
4 shall determine whether the new drug that is
5 the subject of such request is an innovative
6 treatment.

7 “(f) PRODUCT IMPLEMENTATION FOR RARE PEDI-
8 ATRIC DISEASE PRODUCTS.—

9 “(1) IN GENERAL.—The Secretary shall deem a
10 rare pediatric disease product application incomplete
11 if such application does not contain a description of
12 the plan of the sponsor of such application to mar-
13 ket the product in the United States.

14 “(2) GOOD FAITH INTENT TO MARKET.—

15 “(A) GOOD FAITH INTENT.—The Sec-
16 retary may refuse to issue a priority review
17 voucher upon the approval of a rare pediatric
18 disease product application if the Secretary
19 finds that the sponsor of such application lacks
20 a good faith intention to market the product in
21 the United States. The Secretary may consider
22 any fact relevant to this determination, includ-
23 ing the history of such sponsor of producing
24 rare pediatric disease products for which such
25 sponsor received a priority review voucher, or-

1 phan drugs for which the sponsor received ex-
2 clusivity under section 527, or pediatric drugs
3 for which the sponsor received an additional 6
4 months of exclusivity under section 505A.

5 “(B) PRESUMPTION.—The sponsor may
6 establish a presumption of good faith by dem-
7 onstrating that such sponsor has allocated suffi-
8 cient resources or otherwise arranged for the
9 production (by the sponsor or by another manu-
10 facturer) of the rare pediatric disease product
11 in a manner sufficient to meet the expected de-
12 mand for the product during the 5-year period
13 following approval of the application.

14 “(C) GUIDANCE.—If the Secretary re-
15 quires sponsors seeking a priority review vouch-
16 er to demonstrate a good faith intent to market
17 the rare pediatric disease product in the United
18 States, the Secretary shall first issue a guid-
19 ance document setting forth the required evi-
20 dentiary support necessary to demonstrate such
21 a good faith intent.

22 “(3) POSTAPPROVAL PRODUCTION REPORT.—

23 “(A) REPORT REQUIRED.—The sponsor of
24 an approved rare pediatric disease product shall
25 submit a report to the Secretary not later than

1 5 years after the approval of the applicable rare
2 pediatric disease product application. Such re-
3 port shall provide the following information,
4 with respect to each of the first 4 years after
5 approval of such product:

6 “(i) The estimated population in the
7 United States suffering from the rare pedi-
8 atric disease.

9 “(ii) The estimated demand in the
10 United States for such rare pediatric dis-
11 ease product.

12 “(iii) The actual amount of such rare
13 pediatric disease product distributed in the
14 United States.

15 “(B) PUBLICATION UPON FAILURE TO
16 DEMONSTRATE GOOD FAITH EFFORT TO MAR-
17 KET.—The Secretary may publish the results of
18 a report submitted under subparagraph (A) in
19 the Federal Register if the Secretary finds that
20 the sponsor that submitted such report has not
21 made a good faith effort to meet the demand in
22 the United States for the product that is the
23 subject of such report during each of the first
24 4 years after approval of such product.

1 “(g) PRODUCTION REPORT FOR TROPICAL DISEASE
2 PRODUCTS.—

3 “(1) REPORT REQUIRED.—The sponsor of an
4 approved tropical disease product shall submit a re-
5 port to the Secretary not later than 5 years after the
6 approval of the applicable rare tropical disease prod-
7 uct application. Such report shall provide the fol-
8 lowing information, with respect to each of the first
9 4 years after approval of such product:

10 “(A) The estimated global population suf-
11 fering from the tropical disease.

12 “(B) The estimated global demand for
13 such tropical disease product.

14 “(C) The actual amount of such tropical
15 disease product distributed globally.

16 “(2) PUBLICATION UPON FAILURE TO DEM-
17 ONSTRATE GOOD FAITH EFFORT TO MARKET.—The
18 Secretary may publish the results of a report sub-
19 mitted under paragraph (1) in the Federal Register
20 if the Secretary finds that the sponsor that sub-
21 mitted such report has not made a good faith effort
22 to meet the global demand for the product that is
23 the subject of such report during each of the first
24 4 years after approval of such product.

1 “(h) NOTICE OF ISSUANCE AND USE OF VOUCHER.—

2 The Secretary shall publish a notice in the Federal Reg-

3 ister and on the Web site of the Food and Drug Adminis-

4 tration not later than 30 days after the occurrence of each

5 of the following:

6 “(1) The Secretary issues a priority review

7 voucher under this section.

8 “(2) A sponsor submits a human drug applica-

9 tion for which such sponsor uses a priority review

10 voucher.

11 “(i) ELIGIBILITY FOR OTHER PROGRAMS.—A spon-

12 sor who seeks a priority review voucher under this section

13 may participate in any other incentive program, including

14 the programs the Secretary has implemented under this

15 Act, if the sponsor meets the applicable criteria of such

16 other incentive program.

17 “(j) RELATION TO OTHER PROVISIONS.—This provi-

18 sions of this section shall supplement, not supplant, any

19 other provisions of this Act or the Public Health Service

20 Act that encourage the development of drugs for tropical

21 diseases and rare pediatric diseases.”.

22 (f) CONFORMING AMENDMENT.—Section 740(b) of

23 the Agricultural, Rural Development, Food and Drug Ad-

24 ministration, and Related Agencies Appropriations Act,

25 2010 (21 U.S.C. 360aa note) is amended by striking

1 “(a)(3)” each place such term appears and inserting

2 “(a)(6)”.

3 **SEC. 3. EFFECTIVE DATE.**

4 This Act (and the amendments made by this Act)

5 shall take effect on the date that is 90 days after the date

6 of enactment of this Act.