

112TH CONGRESS
1ST SESSION

H. R. 3059

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 HOUSE OF REPRESENTATIVES

SEPTEMBER 23, 2011.

Mr. McCAUL (for himself, Mr. BUTTERFIELD, Mrs. MYRICK, Mr. VAN HOLLEN, Mr. BURGESS, Ms. SPEIER, Mr. KELLY, Mr. JOHNSON of Georgia, Mr. DAVIS of Illinois, Mr. TOWNS, Mrs. CHRISTENSEN, Mr. RUSH, Mr. CUELLAR, Mr. BILBRAY, Mr. WOLF, Mrs. McMORRIS RODGERS, Mr. KEATING, Mr. OLSON, Mr. CANSECO, Mr. ROGERS of Alabama, Mr. BOUSTANY, Mr. DAVIS of Kentucky, Ms. ROS-LEHTINEN, Ms. PELOSI, and Mr. ROTIMAN of New Jersey) 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 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”.

1 (b) REFERENCES.—Wherever in this Act an amend-
2 ment is expressed in terms of an amendment to a section
3 or other provision, the reference shall be considered to be
4 made to a section or other provision of the Federal Food,
5 Drug, and Cosmetic Act (21 U.S.C. 301 et seq.).

6 **SEC. 2. IMPROVEMENT OF THE TROPICAL DISEASE VOUCH-**
7 **ER PROGRAM.**

8 (a) HEADING.—The heading of section 524 (21
9 U.S.C. 360n) is amended to read as follows: “**PRIORITY**
10 **REVIEW TO ENCOURAGE TREATMENTS FOR TROP-**
11 **ICAL DISEASES AND RARE PEDIATRIC DISEASES”.**

12 (b) DEFINITIONS.—Section 524(a) (21 U.S.C.
13 360n(a)) is amended—

14 (1) by redesignating paragraphs (3) and (4) as
15 paragraphs (6) and (7), respectively;

16 (2) by redesignating paragraphs (1) and (2) as
17 paragraphs (2) and (3), respectively;

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

20 “(1) **ELIGIBLE TREATMENT.**—The term ‘eligi-
21 ble treatment’ means a new drug, including a bio-
22 logical product that is a new drug, that is the sub-
23 ject of an application submitted under section
24 505(b)(1) of this Act or section 351(a) of the Public
25 Health Service Act, if that drug contains no active

1 ingredient (including any ester or salt of the active
2 ingredient) that has been previously approved in any
3 other application under section 505(b)(1), 505(b)(2),
4 or 505(j) of this Act or section 351(a) or 351(k) of
5 the Public Health Service Act.”;

6 (4) in paragraph (3), as so redesignated, by in-
7 serting “or rare pediatric disease product applica-
8 tion” after “tropical disease product application”
9 each place that phrase appears;

10 (5) by inserting after paragraph (3) the fol-
11 lowing:

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

15 “(A) The disease primarily affects individ-
16 uals aged from birth to 18 years, including age
17 groups often called neonates, infants, children,
18 and adolescents.

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

21 “(5) RARE PEDIATRIC DISEASE PRODUCT AP-
22 PPLICATION.—The term ‘rare pediatric disease prod-
23 uct application’ means a human drug application, as
24 defined in section 735(1)—

1 “(A) for prevention or treatment of a rare
2 pediatric disease;

3 “(B) that the Secretary deems eligible for
4 priority review;

5 “(C) that is for an eligible treatment;

6 “(D) that relies on clinical data derived
7 from studies examining a pediatric population
8 and dosages of the drug intended for that popu-
9 lation; and

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

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

14 (A) by redesignating subparagraph (Q) as
15 subparagraph (R); and

16 (B) by inserting after subparagraph (P)
17 the following:

18 “(Q) Chagas Disease.”; and

19 (7) by amending paragraph (7), as so redesign-
20 ated, to read as follows:

21 “(7) TROPICAL DISEASE PRODUCT APPLICA-
22 TION.—The term ‘tropical disease product applica-
23 tion’ means a human drug application, as defined in
24 section 735(1)—

1 “(A) for prevention or treatment of a trop-
2 ical disease;

3 “(B) that the Secretary deems eligible for
4 priority review;

5 “(C) that is for an eligible treatment; and

6 “(D) that the sponsor affirms in the appli-
7 cation is for a drug that has not been approved
8 for commercial marketing for any tropical dis-
9 ease indication by a government authority out-
10 side of the United States for more than 24
11 months before the tropical disease product ap-
12 plication is submitted.”.

13 (c) RULES REGARDING USE AND TRANSFER OF PRI-
14 ORITY REVIEW VOUCHERS.—Section 524(b) (21 U.S.C.
15 360n(b)) is amended—

16 (1) in paragraph (1), by inserting “or rare pe-
17 diatric disease product application” after “tropical
18 disease product application” each place that phrase
19 appears;

20 (2) by amending paragraph (2) to read as fol-
21 lows:

22 “(2) TRANSFERABILITY.—

23 “(A) IN GENERAL.—The sponsor of a trop-
24 ical disease product application or rare pediatric
25 disease product application that receives a pri-

1 ority review voucher under this section may
2 transfer (including by sale) the entitlement to
3 such voucher. There is no limit on the number
4 of times a priority review voucher may be trans-
5 ferred before such voucher is used.

6 “(B) CONDITIONS OF TRANSFER.—If a
7 sponsor transfers a priority review voucher
8 after such sponsor has provided notification to
9 the Secretary under paragraph (4)(A) of the in-
10 tent of such sponsor to use the voucher, the
11 transfer shall be subject to the provisions of
12 subparagraphs (B) and (C) of paragraph (4).

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

18 (3) by amending paragraph (3) to read as fol-

19 lows:

20 “(3) LIMITATION FOR PRIOR APPLICATIONS.—

21 “(A) TROPICAL DISEASE PRODUCT APPLI-
22 CATIONS.—A sponsor of a tropical disease prod-
23 uct application may not receive a priority review
24 voucher under this section if the tropical dis-

1 ease product application was submitted to the
2 Secretary prior to September 27, 2007.

3 “(B) RARE PEDIATRIC DISEASE PRODUCT
4 APPLICATIONS.—A sponsor of a rare pediatric
5 disease product application may not receive a
6 priority review voucher under this section if the
7 rare pediatric disease product application was
8 submitted to the Secretary prior to the date
9 that is 90 days after the date of enactment of
10 the Creating Hope Act of 2011.”; and

11 (4) by amending paragraph (4) to read as fol-
12 lows:

13 “(4) NOTIFICATION.—

14 “(A) TIMING.—At least 90 days before the
15 date on which a human drug application for
16 which the sponsor intends to use a priority re-
17 view voucher is submitted, the sponsor of such
18 human drug application shall notify the Sec-
19 retary of the intent of such sponsor to submit
20 the human drug application.

21 “(B) TRANSFER OF VOUCHER AFTER NO-
22 TIFICATION.—

23 “(i) IN GENERAL.—The sponsor of a
24 human drug application that provides noti-
25 fication of the intent of such sponsor to

1 use the voucher for the human drug appli-
2 cation may transfer the voucher after such
3 notification is provided, if such sponsor has
4 not yet submitted the human drug applica-
5 tion described in the notification.

6 “(ii) EXCEPTION.—The person to
7 whom a voucher is transferred under
8 clause (i) (referred to in this paragraph as
9 the ‘transferee’) shall give notification of
10 the intent of such transferee to use the
11 voucher in accordance with this subsection,
12 unless—

13 “(I) the transferee uses the
14 voucher for a human drug application
15 including the same indications as the
16 human drug application described in
17 the transferor’s notification; and

18 “(II) the transferee notifies the
19 Secretary within 30 days of the trans-
20 fer of the intent of such transferee to
21 use the voucher for such purpose.

22 “(iii) INTERNAL TRANSFER.—If the
23 sponsor transfers a voucher internally for
24 use with a drug application including one
25 or more indications that were not included

1 in the drug application that was the sub-
2 ject of the notification of such sponsor, the
3 sponsor shall notify the Secretary of the
4 transfer in accordance with this subsection.

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

7 “(i) DUE UPON NOTIFICATION.—The
8 notification under this subsection shall be
9 a legally binding commitment to pay for
10 the user fee to be assessed in accordance
11 with this section. Such fee shall be payable
12 by the sponsor upon the submission by
13 such sponsor of such notification.

14 “(ii) CREDIT.—If a sponsor pays a
15 user fee upon providing notification of the
16 intent of such sponsor to use a priority re-
17 view voucher, but later transfers the vouch-
18 er for which such sponsor gave notifica-
19 tion, the Secretary shall credit the user
20 fees paid to the next human drug applica-
21 tion for which a sponsor provides notifica-
22 tion of the intent of such sponsor to use
23 the same transferred voucher.

24 “(iii) DIFFERENCE IN FEE.—The Sec-
25 retary may require a sponsor using a

1 transferred voucher to pay the difference
2 between the credit associated with the
3 transferred voucher and the user fee pre-
4 vailing at the time the sponsor submits no-
5 tification of the intent of such sponsor to
6 use the transferred voucher. This provision
7 does not apply in cases where a transferee
8 is exempted from submitting notification
9 under this paragraph.”.

10 (d) PAYMENT.—Section 524(c)(4) (21 U.S.C.
11 360n(c)(4)) is amended—

12 (1) in subparagraph (A), by striking “submis-
13 sion of a human drug application under section
14 505(b)(1) or section 351 of the Public Health Serv-
15 ices Act for which the priority review voucher is
16 used.” and inserting “notification by a sponsor of
17 the intent of such sponsor to use the voucher, as
18 specified in subsection (b)(4)(A). All other user fees
19 associated with the human drug application shall be
20 due as required by the Secretary or under applicable
21 law.”; and

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

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

5 “(d) DESIGNATION PROCESS.—

6 “(1) DESIGNATION OF RARE PEDIATRIC DIS-
7 EASES.—

8 “(A) IN GENERAL.—Upon the request of
9 the manufacturer or the sponsor of a new drug,
10 the Secretary may designate that the new drug
11 is for a rare pediatric disease. Such a request
12 for designation, if sought, shall be made when
13 requesting designation of orphan disease status
14 under section 526 or fast-track designation
15 under section 506. Requesting designation of
16 rare pediatric disease status under this para-
17 graph is not a prerequisite to receiving a pri-
18 ority review voucher.

19 “(B) DETERMINATION BY SECRETARY.—
20 Not later than 60 days after a request is sub-
21 mitted under subparagraph (A), the Secretary
22 shall determine whether the disease or condition
23 that is the subject of such request is a rare pe-
24 diatric disease.

1 “(2) DESIGNATION OF ELIGIBLE TREAT-
2 MENTS.—

3 “(A) IN GENERAL.—Upon the request of
4 the manufacturer or the sponsor of a new drug,
5 the Secretary may designate that a new drug is
6 an eligible treatment. Such a request for des-
7 ignation, if sought, shall be made when request-
8 ing fast-track designation under section 506.
9 Requesting designation that a new drug is an
10 eligible treatment is not a prerequisite to receiv-
11 ing a priority review voucher.

12 “(B) DETERMINATION BY SECRETARY.—
13 Not later than 60 days after a request is sub-
14 mitted under subparagraph (A), the Secretary
15 shall determine whether the new drug that is
16 the subject of such request is an eligible treat-
17 ment.

18 “(c) PRODUCT IMPLEMENTATION FOR RARE PEDI-
19 ATRIC DISEASE PRODUCTS.—

20 “(1) IN GENERAL.—The Secretary shall deem a
21 rare pediatric disease product application incomplete
22 if such application does not contain a description of
23 the plan of the sponsor of such application to mar-
24 ket the product in the United States.

25 “(2) GOOD FAITH INTENT TO MARKET.—

1 “(A) GOOD FAITH INTENT.—The Sec-
2 retary may refuse to issue a priority review
3 voucher upon the approval of a rare pediatric
4 disease product application if the Secretary
5 finds that the sponsor of such application lacks
6 a good faith intention to market the product in
7 the United States. The Secretary may consider
8 any fact relevant to this determination, includ-
9 ing the history of such sponsor of producing
10 rare pediatric disease products for which such
11 sponsor received a priority review voucher, or-
12 phan drugs for which the sponsor received ex-
13 clusivity under section 527, or pediatric drugs
14 for which the sponsor received an additional 6
15 months of exclusivity under section 505A.

16 “(B) PRESUMPTION.—The sponsor may
17 establish a presumption of good faith by dem-
18 onstrating that such sponsor has allocated suffi-
19 cient resources or otherwise arranged for the
20 production (by the sponsor or by another manu-
21 facturer) of the rare pediatric disease product
22 in a manner sufficient to meet the expected de-
23 mand for the product during the 5-year period
24 following approval of the application.

1 “(C) GUIDANCE.—If the Secretary re-
2 quires sponsors seeking a priority review vouch-
3 er to demonstrate a good faith intent to market
4 the rare pediatric disease product in the United
5 States, the Secretary shall first issue a guid-
6 ance document setting forth the required evi-
7 dentiary support necessary to demonstrate such
8 a good faith intent.

9 “(3) POSTAPPROVAL PRODUCTION REPORT.—

10 “(A) REPORT REQUIRED.—The sponsor of
11 an approved rare pediatric disease product shall
12 submit a report to the Secretary not later than
13 5 years after the approval of the applicable rare
14 pediatric disease product application. Such re-
15 port shall provide the following information,
16 with respect to each of the first 4 years after
17 approval of such product:

18 “(i) The estimated population in the
19 United States suffering from the rare pedi-
20 atric disease.

21 “(ii) The estimated demand in the
22 United States for such rare pediatric dis-
23 ease product.

1 “(iii) The actual amount of such rare
2 pediatric disease product distributed in the
3 United States.

4 “(B) PUBLICATION UPON FAILURE TO
5 DEMONSTRATE GOOD FAITH EFFORT TO MAR-
6 KET.—The Secretary may publish the results of
7 a report submitted under subparagraph (A) in
8 the Federal Register if the Secretary finds that
9 the sponsor that submitted such report has not
10 made a good faith effort to meet the demand in
11 the United States for the product that is the
12 subject of such report during each of the first
13 4 years after approval of such product.

14 “(f) PRODUCTION REPORT FOR TROPICAL DISEASE
15 PRODUCTS.—

16 “(1) REPORT REQUIRED.—The sponsor of an
17 approved tropical disease product shall submit a re-
18 port to the Secretary not later than 5 years after the
19 approval of the applicable rare tropical disease prod-
20 uct application. Such report shall provide the fol-
21 lowing information, with respect to each of the first
22 4 years after approval of such product:

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

1 “(B) The estimated global demand for
2 such tropical disease product.

3 “(C) The actual amount of such tropical
4 disease product distributed globally.

5 “(2) PUBLICATION UPON FAILURE TO DEM-
6 ONSTRATE GOOD FAITH EFFORT TO MARKET.—The
7 Secretary may publish the results of a report sub-
8 mitted under paragraph (1) in the Federal Register
9 if the Secretary finds that the sponsor that sub-
10 mitted such report has not made a good faith effort
11 to meet the global demand for the product that is
12 the subject of such report during each of the first
13 4 years after approval of such product.

14 “(g) NOTICE OF ISSUANCE AND USE OF VOUCHER.—
15 The Secretary shall publish a notice in the Federal Reg-
16 ister and on the Web site of the Food and Drug Adminis-
17 tration not later than 30 days after the occurrence of each
18 of the following:

19 “(1) The Secretary issues a priority review
20 voucher under this section.

21 “(2) A sponsor submits a human drug applica-
22 tion for which such sponsor uses a priority review
23 voucher.

24 “(h) ELIGIBILITY FOR OTHER PROGRAMS.—A spon-
25 sor who seeks a priority review voucher under this section

1 may participate in any other incentive program, including
2 the programs the Secretary has implemented under this
3 Act, if the sponsor meets the applicable criteria of such
4 other incentive program.

5 “(i) RELATION TO OTHER PROVISIONS.—The provi-
6 sions of this section shall supplement, not supplant, any
7 other provisions of this Act or the Public Health Service
8 Act that encourage the development of drugs for tropical
9 diseases and rare pediatric diseases.”.

10 (f) CONFORMING AMENDMENT.—Section 740(b) of
11 the Agricultural, Rural Development, Food and Drug Ad-
12 ministration, and Related Agencies Appropriations Act,
13 2010 (21 U.S.C. 360aa note) is amended by striking
14 “(a)(3)” each place such term appears and inserting
15 “(a)(6)”.

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

17 This Act (and the amendments made by this Act)
18 shall take effect on the date that is 90 days after the date
19 of enactment of this Act.

○