

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.

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## 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. ROTHMAN of New Jersey) 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 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          nated, 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 pedi-  
17                 atric 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            priority 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          “(e) 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.

○