

# Calendar No. 415

114<sup>TH</sup> CONGRESS  
2<sup>D</sup> SESSION

# S. 1878

To extend the pediatric priority review voucher program.

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## IN THE SENATE OF THE UNITED STATES

JULY 28, 2015

Mr. CASEY (for himself, Mr. ISAKSON, Mr. BROWN, and Mr. KIRK) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

APRIL 5, 2016

Reported by Mr. ALEXANDER, with an amendment

[Strike out all after the enacting clause and insert the part printed in *italie*]

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## A BILL

To extend the pediatric priority review voucher program.

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 “~~Advancing Hope Act~~  
5 ~~of 2015~~”.

1 **SEC. 2. REAUTHORIZATION OF PROGRAM FOR PRIORITY**  
 2 **REVIEW TO ENCOURAGE TREATMENTS FOR**  
 3 **RARE PEDIATRIC DISEASES.**

4 Section 529 of the Federal Food, Drug, and Cosmetic  
 5 Act (21 U.S.C. 360ff) is amended—

6 (1) in subsection (a)—

7 (A) by amending paragraph (3) to read as  
 8 follows:

9 “(3) RARE PEDIATRIC DISEASE.—The term  
 10 ‘rare pediatric disease’ means any of the following:

11 “(A) A disease that meets each of the fol-  
 12 lowing criteria:

13 “(i) The disease primarily affects indi-  
 14 viduals aged from birth to 18 years, in-  
 15 cluding age groups often called neonates,  
 16 infants, children, and adolescents.

17 “(ii) The disease is a rare disease or  
 18 condition, within the meaning of section  
 19 526.

20 “(B) Any form of sickle cell disease.

21 “(C) Any pediatric cancers.”; and

22 (B) in paragraph (4)—

23 (i) in subparagraph (E), by striking

24 “and” at the end;

1 (ii) in subparagraph (F), by striking  
2 the period at the end and inserting “;  
3 and”; and

4 (iii) by adding at the end the fol-  
5 lowing:

6 “(G) is for a drug or biological product for  
7 which a priority review voucher has not been  
8 issued under section 524 (relating to tropical  
9 disease products).”;

10 (2) in subsection (b), by striking paragraph (5);  
11 and

12 (3) in subsection (d)(2), in the second sentence,  
13 by striking the period and inserting “, but the spon-  
14 sor of a drug that intends to request a priority re-  
15 view voucher under this section shall notify the Sec-  
16 retary of such intent upon submission of an applica-  
17 tion under section 505 or section 351 of the Public  
18 Health Service Act that is the basis of such re-  
19 quest.”.

20 **SECTION 1. SHORT TITLE.**

21 *This Act may be cited as the “Advancing Hope Act*  
22 *of 2016”.*

1 **SEC. 2. REAUTHORIZATION OF PROGRAM FOR PRIORITY RE-**  
 2 **VIEW TO ENCOURAGE TREATMENTS FOR**  
 3 **RARE PEDIATRIC DISEASES.**

4 (a) *IN GENERAL.*—Section 529 of the Federal Food,  
 5 Drug, and Cosmetic Act (21 U.S.C. 360ff) is amended—

6 (1) in subsection (a)—

7 (A) in paragraph (3), by amending sub-  
 8 paragraph (A) to read as follows:

9 “(A) The disease is a serious or life-threat-  
 10 ening disease in which the serious or life-threat-  
 11 ening manifestations primarily affect individ-  
 12 uals aged from birth to 18 years, including age  
 13 groups often called neonates, infants, children,  
 14 and adolescents.”; and

15 (B) in paragraph (4)(F), by striking “Pre-  
 16 scription Drug User Fee Amendments of 2012”  
 17 and inserting “Advancing Hope Act of 2016”;

18 (2) in subsection (b)—

19 (A) by striking paragraph (4) and inserting  
 20 the following:

21 “(4) NOTIFICATION.—

22 “(A) SPONSOR OF A RARE PEDIATRIC DIS-  
 23 EASE PRODUCT.—

24 “(i) *IN GENERAL.*—Beginning on the  
 25 date that is 90 days after the date of enact-  
 26 ment of the Advancing Hope Act of 2016,

1           *the sponsor of a rare pediatric disease prod-*  
2           *uct application that intends to request a*  
3           *priority review voucher under this section*  
4           *shall notify the Secretary of such intent*  
5           *upon submission of the rare pediatric dis-*  
6           *ease product application that is the basis of*  
7           *the request for a priority review voucher.*

8           “(ii) *APPLICATIONS SUBMITTED BUT*  
9           *NOT YET APPROVED.—The sponsor of a rare*  
10          *pediatric disease product application that*  
11          *was submitted and that has not been ap-*  
12          *proved as of the date of enactment of the*  
13          *Advancing Hope Act of 2016 shall be con-*  
14          *sidered eligible for a priority review vouch-*  
15          *er, if—*

16                “(I) *such sponsor has submitted*  
17                *such rare pediatric disease product ap-*  
18                *plication—*

19                    “(aa) *on or after the date*  
20                    *that is 90 days after the date of*  
21                    *enactment of the Prescription*  
22                    *Drug User Fee Amendments of*  
23                    *2012; and*

1                   “(bb) on or before the date of  
2                   enactment of the Advancing Hope  
3                   Act of 2016; and

4                   “(II) such application otherwise  
5                   meets the criteria for a priority review  
6                   voucher under this section.

7                   “(B) SPONSOR OF A DRUG APPLICATION  
8                   USING A PRIORITY REVIEW VOUCHER.—

9                   “(i) IN GENERAL.—The sponsor of a  
10                  human drug application shall notify the  
11                  Secretary not later than 90 days prior to  
12                  submission of the human drug application  
13                  that is the subject of a priority review  
14                  voucher of an intent to submit the human  
15                  drug application, including the date on  
16                  which the sponsor intends to submit the ap-  
17                  plication. Such notification shall be a le-  
18                  gally binding commitment to pay the user  
19                  fee to be assessed in accordance with this  
20                  section.

21                  “(ii) TRANSFER AFTER NOTICE.—The  
22                  sponsor of a human drug application that  
23                  provides notification of the intent of such  
24                  sponsor to use the voucher for the human  
25                  drug application under clause (i) may

1           *transfer the voucher after such notification*  
2           *is provided, if such sponsor has not yet sub-*  
3           *mitted the human drug application de-*  
4           *scribed in the notification.”; and*

5           *(B) by striking paragraph (5) and inserting*  
6           *the following:*

7           “(5) *TERMINATION OF AUTHORITY.—The Sec-*  
8           *retary may not award any priority review vouchers*  
9           *under paragraph (1) after September 30, 2022, unless*  
10          *the rare pediatric disease product application—*

11           *“(A) is for a drug that, not later than Sep-*  
12          *tember 30, 2022, is designated under subsection*  
13          *(d) as a drug for a rare pediatric disease; and*

14           *“(B) is, not later than September 30, 2027,*  
15          *approved under section 505(b)(1) of this Act or*  
16          *section 351(a) of the Public Health Service Act.”;*  
17          *and*

18          *(3) in subsection (g), by inserting before the pe-*  
19          *riod “, except that no sponsor of a rare pediatric dis-*  
20          *ease product application may receive more than one*  
21          *priority review voucher issued under any section of*  
22          *this Act with respect to the drug for which the appli-*  
23          *cation is made.”*

24          *(b) RULE OF CONSTRUCTION.—Nothing in this Act, or*  
25          *the amendments made by this Act, shall be construed to af-*

1 *fect the validity of a priority review voucher that was issued*  
2 *under section 529 of the Federal Food, Drug, and Cosmetic*  
3 *Act (21 U.S.C. 360ff) before the date of enactment of this*  
4 *Act.*

5 **SEC. 3. GAO REPORT.**

6 *(a) STUDY.—The Comptroller General of the United*  
7 *States shall conduct a study on the effectiveness of awarding*  
8 *priority review vouchers under section 529 of the Federal*  
9 *Food, Drug, and Cosmetic Act (21 U.S.C. 360ff) in pro-*  
10 *viding incentives for the development of drugs that treat*  
11 *or prevent rare pediatric diseases (as defined in subsection*  
12 *(a)(3) of such section) that would not otherwise have been*  
13 *developed. In conducting such study, the Comptroller Gen-*  
14 *eral shall examine the following:*

15 *(1) The indications for which each drug for*  
16 *which a priority review voucher was awarded under*  
17 *such section 529 was approved under section*  
18 *505(b)(1) of the Federal Food, Drug, and Cosmetic*  
19 *Act (21 U.S.C. 355(b)(1)) or section 351(a) of the*  
20 *Public Health Service Act (42 U.S.C. 262(a)).*

21 *(2) Whether the priority review voucher im-*  
22 *acted sponsors' decisions to invest in developing a*  
23 *drug to treat or prevent a rare pediatric disease.*



1           (3) *An analysis of the drugs for which such pri-*  
2 *ority review vouchers were used, which shall in-*  
3 *clude—*

4           (A) *the indications for which such drugs*  
5 *were approved under section 505(b)(1) of the*  
6 *Federal Food, Drug, and Cosmetic Act (21*  
7 *U.S.C. 355(b)(1)) or section 351(a) of the Public*  
8 *Health Service Act (42 U.S.C. 262(a));*

9           (B) *whether unmet medical needs were ad-*  
10 *dressed through the approval of such drugs, in-*  
11 *cluding, for each such drug—*

12           (i) *if an alternative therapy was pre-*  
13 *viously available to treat the indication;*  
14 *and*

15           (ii) *if the drug provided a benefit or*  
16 *advantage over another available therapy;*

17           (C) *the number of patients potentially treat-*  
18 *ed by such drugs;*

19           (D) *the value of the priority review voucher*  
20 *if transferred; and*

21           (E) *the length of time between the date on*  
22 *which a priority review voucher was awarded*  
23 *and the date on which it was used.*

1           (4) *With respect to the priority review voucher*  
2 *program under section 529 of the Federal Food, Drug,*  
3 *and Cosmetic Act (21 U.S.C. 360ff)—*

4           (A) *the resources used by the Food and*  
5 *Drug Administration in implementing such pro-*  
6 *gram, including the effect of such program on the*  
7 *Food and Drug Administration’s review of drugs*  
8 *for which a priority review voucher was not*  
9 *awarded or used;*

10          (B) *the impact of the program on the public*  
11 *health as a result of the review and approval of*  
12 *drugs that received a priority review voucher*  
13 *and products that were the subject of a redeemed*  
14 *priority review voucher; and*

15          (C) *alternative approaches to improving*  
16 *such program so that the program is appro-*  
17 *priately targeted toward providing incentives for*  
18 *the development of clinically important drugs*  
19 *that—*

20           (i) *prevent or treat rare pediatric dis-*  
21 *eases; and*

22           (ii) *would likely not otherwise have*  
23 *been developed to prevent or treat such dis-*  
24 *eases.*

1       **(b) REPORT.**—*Not later than January 31, 2022, the*  
2 *Comptroller General of the United States shall submit to*  
3 *the Committee on Health, Education, Labor, and Pensions*  
4 *of the Senate and the Committee on Energy and Commerce*  
5 *of the House of Representatives a report containing the re-*  
6 *sults of the study of conducted under subsection (a).*

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