

Calendar No. 415

114TH CONGRESS
2^D SESSION**S. 1878**

To extend the pediatric priority review voucher program.

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*]

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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