# Calendar No. 415

114TH CONGRESS
2D 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 italic]

---

# A BILL

To extend the pediatric priority review voucher program.

*Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,*

~~**SECTION 1. SHORT TITLE.**~~

~~This Act may be cited as the "Advancing Hope Act of 2015".~~

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

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

(1) in subsection (a)—

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

"(3) RARE PEDIATRIC DISEASE.—The term 'rare pediatric disease' means any of the following:

"(A) A disease that meets each of the following criteria:

"(i) The disease primarily affects individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents.

"(ii) The disease is a rare disease or condition, within the meaning of section 526.

"(B) Any form of sickle cell disease.

"(C) Any pediatric cancers."; and

(B) in paragraph (4)—

(i) in subparagraph (E), by striking "and" at the end;

(ii) in subparagraph (F), by striking the period at the end and inserting ''; and''; and

(iii) by adding at the end the following:

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

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

(3) in subsection (d)(2), in the second sentence, by striking the period and inserting '', but the sponsor of a drug that intends to request a priority review voucher under this section shall notify the Secretary of such intent upon submission of an application under section 505 or section 351 of the Public Health Service Act that is the basis of such request.''.

***SECTION 1. SHORT TITLE.***

*This Act may be cited as the ''Advancing Hope Act of 2016''.*

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

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

*(1) in subsection (a)—*

*(A) in paragraph (3), by amending subparagraph (A) to read as follows:*

*"(A) The disease is a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents."; and*

*(B) in paragraph (4)(F), by striking "Prescription Drug User Fee Amendments of 2012" and inserting "Advancing Hope Act of 2016";*

*(2) in subsection (b)—*

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

*"(4) NOTIFICATION.—*

*"(A) SPONSOR OF A RARE PEDIATRIC DISEASE PRODUCT.—*

*"(i) IN GENERAL.—Beginning on the date that is 90 days after the date of enactment of the Advancing Hope Act of 2016,*

*the sponsor of a rare pediatric disease product application that intends to request a priority review voucher under this section shall notify the Secretary of such intent upon submission of the rare pediatric disease product application that is the basis of the request for a priority review voucher.*

*"(ii) APPLICATIONS SUBMITTED BUT NOT YET APPROVED.—The sponsor of a rare pediatric disease product application that was submitted and that has not been approved as of the date of enactment of the Advancing Hope Act of 2016 shall be considered eligible for a priority review voucher, if—*

*"(I) such sponsor has submitted such rare pediatric disease product application—*

*"(aa) on or after the date that is 90 days after the date of enactment of the Prescription Drug User Fee Amendments of 2012; and*

*"(bb) on or before the date of enactment of the Advancing Hope Act of 2016; and*

*"(II) such application otherwise meets the criteria for a priority review voucher under this section.*

*"(B) SPONSOR OF A DRUG APPLICATION USING A PRIORITY REVIEW VOUCHER.—*

*"(i) IN GENERAL.—The sponsor of a human drug application shall notify the Secretary not later than 90 days prior to submission of the human drug application that is the subject of a priority review voucher of an intent to submit the human drug application, including the date on which the sponsor intends to submit the application. Such notification shall be a legally binding commitment to pay the user fee to be assessed in accordance with this section.*

*"(ii) TRANSFER AFTER NOTICE.—The sponsor of a human drug application that provides notification of the intent of such sponsor to use the voucher for the human drug application under clause (i) may*

*transfer the voucher after such notification is provided, if such sponsor has not yet submitted the human drug application described in the notification.''; and*

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

*''(5) TERMINATION OF AUTHORITY.—The Secretary may not award any priority review vouchers under paragraph (1) after September 30, 2022, unless the rare pediatric disease product application—*

*''(A) is for a drug that, not later than September 30, 2022, is designated under subsection (d) as a drug for a rare pediatric disease; and*

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

*(3) in subsection (g), by inserting before the period '', except that no sponsor of a rare pediatric disease product application may receive more than one priority review voucher issued under any section of this Act with respect to the drug for which the application is made.''*

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

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

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

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

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

*(2) Whether the priority review voucher impacted sponsors' decisions to invest in developing a drug to treat or prevent a rare pediatric disease.*

*(3) An analysis of the drugs for which such priority review vouchers were used, which shall include—*

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

*(B) whether unmet medical needs were addressed through the approval of such drugs, including, for each such drug—*

*(i) if an alternative therapy was previously available to treat the indication; and*

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

*(C) the number of patients potentially treated by such drugs;*

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

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

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

*(A) the resources used by the Food and Drug Administration in implementing such program, including the effect of such program on the Food and Drug Administration's review of drugs for which a priority review voucher was not awarded or used;*

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

*(C) alternative approaches to improving such program so that the program is appropriately targeted toward providing incentives for the development of clinically important drugs that—*

*(i) prevent or treat rare pediatric diseases; and*

*(ii) would likely not otherwise have been developed to prevent or treat such diseases.*

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

**Calendar No. 415**

114TH CONGRESS
2D SESSION

# S. 1878

# A BILL

To extend the pediatric priority review voucher program.

APRIL 5, 2016

Reported with an amendment