

CREATING HOPE REAUTHORIZATION ACT

SEPTEMBER 29, 2020.—Committed to the Committee of the Whole House on the State of the Union and ordered to be printed

Mr. PALLONE, from the Committee on Energy and Commerce, submitted the following

R E P O R T

[To accompany H.R. 4439]

The Committee on Energy and Commerce, to whom was referred the bill (H.R. 4439) to amend the Federal Food, Drug, and Cosmetic Act to make permanent the authority of the Secretary of Health and Human Services to issue priority review vouchers to encourage treatments for rare pediatric diseases, having considered the same, reports favorably thereon with amendments and recommends that the bill as amended do pass.

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The amendments are as follows:
 Strike all after the enacting clause and insert the following:

SECTION 1. SHORT TITLE.

This Act may be cited as the “Creating Hope Reauthorization Act”.

SEC. 2. EXTENSION OF AUTHORITY TO ISSUE PRIORITY REVIEW VOUCHERS TO ENCOURAGE TREATMENTS FOR RARE PEDIATRIC DISEASES.

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

(1) by striking “September 30, 2020” each place it appears and inserting “September 30, 2024”; and

(2) by striking “September 30, 2022” and inserting “September 30, 2026”.

Amend the title so as to read:

A bill to amend the Federal Food, Drug, and Cosmetic Act to extend the authority of the Secretary of Health and Human Services to issue priority review vouchers to encourage treatments for rare pediatric diseases.

I. PURPOSE AND SUMMARY

H.R. 4439, the “Creating Hope Reauthorization Act”, was introduced on September 20, 2019, by Representative G.K. Butterfield (D–NC) and referred to the Committee on Energy and Commerce. H.R. 4439, as reported, will extend the Food and Drug Administration (FDA) Pediatric Rare Disease Priority Review Voucher (PRV) program for four years. Under this program, certain manufacturers of rare pediatric disease drugs can be eligible for a voucher that can be used or transferred to obtain a priority review for a subsequent drug after the date of approval of the rare pediatric disease drug.

II. BACKGROUND AND NEED FOR THE LEGISLATION

The Pediatric Rare Disease PRV program was originally enacted in 2012 with the intent to create an incentive for drug manufacturers to develop therapies for rare pediatric diseases that affect neonates, infants, children, and adolescents. The program requires the FDA to award a PRV to the sponsor of a rare pediatric disease product application that receives approval as a drug or biological product to treat a rare pediatric disease, defined as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. The incentive value is premised on the ability for the holder of a PRV to shorten the FDA review time on another drug application from the standard ten months to six months, or for the holder of a PRV to sell the voucher to another manufacturer to be used on a drug application of the manufacturer’s choosing.

To date, FDA has awarded 22 PRVs for rare pediatric diseases.¹ PRVs have been redeemed to accelerate review of applications for treatments of a variety of conditions, including human immunodeficiency virus (HIV) and type 2 diabetes, and have been sold for prices that range between \$65 million and \$350 million.² While these facts indicate that PRVs carry some level of value, at least one study has shown that the Pediatric Rare Disease PRV program has not improved the rate of new pediatric drugs starting or completing clinical testing, suggesting that the value of PRVs is an in-

¹Michael Mezher, Zachary Brennan, and Alexander Gaffney, *Everything You Need to Know about FDA’s Priority Review Vouchers*, Regulatory Affairs Professionals Society (Feb. 25, 2020) (www.raps.org/regulatory-focus/news-articles/2017/12/regulatory-explainer-everything-you-need-to-know-about-fdas-priority-review-vouchers).

²*Id.*

sufficient incentive for drug development.³ Furthermore, observers have noted that as more PRVs have been awarded, the price of PRVs has reduced, suggesting that additional awards reduce the PRV value to developers.⁴ Additionally, FDA has said that the program strains agency resources and impairs the agency’s ability to set public health priorities.⁵

Proponents of H.R. 4439 argue that the program is an important consideration for drug manufacturers when deciding where to invest research and development dollars. According to a review of PRV programs conducted by the U.S. Government Accountability Office (GAO), six of seven drug manufacturers interviewed by the GAO indicated that the PRV was one of a number of factors in making drug development decisions, with the seventh indicating that the PRV was “pivotal” in its development of a drug.⁶ Proponents have also suggested that the increase in the number of drug applications receiving pediatric rare disease designation—from 24 in 2016 to 64 in 2019—indicates an increased level of drug development for pediatric rare diseases under the program.⁷

H.R. 4439, as introduced, would remove the sunset on the authority of the Secretary of the U.S. Department of Health and Human Services (the Secretary) to issue new pediatric rare disease designations and PRVs, which is set to expire on September 30, 2020. So as not to foreclose on the potential for development of additional treatments for pediatric rare diseases, but in recognition of concerns about effectiveness of the program, the Committee adopted an amendment in the nature of a substitute (AINS) that would extend the Pediatric Rare Disease PRV program for four years.

III. COMMITTEE HEARINGS

For the purposes of section 103(i) of H. Res. 6 of the 116th Congress, the following hearing was used to develop or consider H.R. 4439:

The Subcommittee on Health held a legislative hearing on July 29, 2020, entitled “Improving Access to Care: Legislation to Reauthorize Key Public Health Programs” to consider H.R. 4439, the “Creating Hope Reauthorization Act” and four other bills. The Subcommittee received testimony from the following witnesses:

- Robert Boyd, M.C.R.P., M. Div., President, School-Based Health Alliance;
- Linda Goler Blount, M.P.H., President and CEO, Black Women’s Health Imperative;
- Nancy Goodman, M.P.P., J.D., Founder and Executive Director, Kids v. Cancer;

³Thomas J. Hwang et al., *Impact of the Priority Review Voucher Program on Drug Development for Rare Pediatric Diseases*, Health Affairs (Feb. 2019).

⁴House Committee on Energy and Commerce, Subcommittee on Health, Testimony of Aaron S. Kesselheim, M.D., J.D., M.P.H., Professor of Medicine, Harvard Medical School, *Legislative Hearing on Improving Access to Care: Legislation to Reauthorize Key Public Health Programs*, 116th Cong. (July 29, 2020).

⁵Government Accountability Office, *Rare Diseases: Too Early to Gauge Effectiveness of FDA’s Pediatric Voucher Program* (Mar. 2016) (GAO-16-319).

⁶Government Accountability Office, *Drug Development: FDA’s Priority Review Voucher Programs* (Jan. 2020) (GAO-20-251).

⁷House Committee on Energy and Commerce, Subcommittee on Health, Testimony of Nancy Goodman, M.P.P., J.D., Founder and Executive Director, Kids v. Cancer, *Legislative Hearing on Improving Access to Care: Legislation to Reauthorize Key Public Health Programs*, 116th Cong. (July 29, 2020).

- Aaron Seth Kesselheim, M.D., J.D., M.P.H., Professor of Medicine, Harvard Medical School;
- Brian Lindbergh, Chief Legal Officer and General Counsel, National Bone Marrow Donor Program; and
- Travis T. Tygart, CEO, U.S. Anti-doping Agency.

IV. COMMITTEE CONSIDERATION

Representative Butterfield (D–NC) introduced H.R. 4439, the “Creating Hope Reauthorization Act”, on September 20, 2019, and the bill was referred to the Committee on Energy and Commerce. The bill was then referred to the Subcommittee on Health on September 23, 2019. A legislative hearing was held on the bill on July 29, 2020.

On September 9, 2020, H.R. 4439 was discharged from further consideration by the Subcommittee on Health as the bill was called up for markup by the full Committee. The Committee on Energy and Commerce met in virtual open markup session, pursuant to notice, to consider the bill H.R. 4439. During consideration of the bill, an amendment in the nature of a substitute offered by Mr. Pallone of New Jersey was agreed to by a voice vote. Upon conclusion of consideration of the bill, the full Committee agreed to a motion on final passage by Mr. Pallone, Chairman of the committee, to order H.R. 4439 reported favorably to the House, amended, by a voice vote, a quorum being present.

V. COMMITTEE VOTES

Clause 3(b) of rule XIII of the Rules of the House of Representatives requires the Committee to list each record vote on the motion to report legislation and amendments thereto. The Committee advises that there were no record votes taken on H.R. 4439, including the motion for final passage of the bill.

VI. OVERSIGHT FINDINGS

Pursuant to clause 3(c)(1) of rule XIII and clause 2(b)(1) of rule X of the Rules of the House of Representatives, the oversight findings and recommendations of the Committee are reflected in the descriptive portion of the report.

VII. NEW BUDGET AUTHORITY, ENTITLEMENT AUTHORITY, AND TAX EXPENDITURES

Pursuant to 3(c)(2) of rule XIII of the Rules of the House of Representatives, the Committee adopts as its own the estimate of new budget authority, entitlement authority, or tax expenditures or revenues contained in the cost estimate prepared by the Director of the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974.

The Committee has requested but not received from the Director of the Congressional Budget Office a statement as to whether this bill contains any new budget authority, spending authority, credit authority, or an increase or decrease in revenues or tax expenditures.

VIII. FEDERAL MANDATES STATEMENT

The Committee adopts as its own the estimate of Federal mandates prepared by the Director of the Congressional Budget Office pursuant to section 423 of the Unfunded Mandates Reform Act.

IX. STATEMENT OF GENERAL PERFORMANCE GOALS AND OBJECTIVES

Pursuant to clause 3(c)(4) of rule XIII, the general performance goal or objective of this legislation is to extend the authority of the Secretary of Health and Human Services to issue PRVs to encourage the development of treatments for rare pediatric diseases.

X. DUPLICATION OF FEDERAL PROGRAMS

Pursuant to clause 3(c)(5) of rule XIII, no provision of H.R. 4439 is known to be duplicative of another Federal program, including any program that was included in a report to Congress pursuant to section 21 of Public Law 111–139 or the most recent Catalog of Federal Domestic Assistance.

XI. COMMITTEE COST ESTIMATE

Pursuant to clause 3(d)(1) of rule XIII, the Committee adopts as its own the cost estimate prepared by the Director of the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974.

XII. EARMARKS, LIMITED TAX BENEFITS, AND LIMITED TARIFF BENEFITS

Pursuant to clause 9(e), 9(f), and 9(g) of rule XXI, the Committee finds that H.R. 4439 contains no earmarks, limited tax benefits, or limited tariff benefits.

XIII. ADVISORY COMMITTEE STATEMENT

No advisory committee within the meaning of section 5(b) of the Federal Advisory Committee Act was created by this legislation.

XIV. APPLICABILITY TO LEGISLATIVE BRANCH

The Committee finds that the legislation does not relate to the terms and conditions of employment or access to public services or accommodations within the meaning of section 102(b)(3) of the Congressional Accountability Act.

XV. SECTION-BY-SECTION ANALYSIS OF THE LEGISLATION

Section 1. Short title

Section 1 designates that the bill may be cited as the “Creating Hope Reauthorization Act”.

Sec. 2. Extension of authority to issue priority review vouchers to encourage treatments for rare pediatric diseases

Section 2 amends the Federal Food, Drug, and Cosmetic Act to extend the sunset on the Secretary’s authority to issue new Pediatric Rare Disease PRVs to September 30, 2024, provided that the PRV is issued for a drug that, not later than September 30, 2024,

is designated as a drug for a rare pediatric disease; and is, not later than September 30, 2026, approved under section 351(b)(1) of the Federal Food, Drug, and Cosmetic Act, or section 351(a) of the Public Health Service Act.

XVI. CHANGES IN EXISTING LAW MADE BY THE BILL, AS REPORTED

In compliance with clause 3(e) of rule XIII of the Rules of the House of Representatives, changes in existing law made by the bill, as reported, are shown as follows (existing law proposed to be omitted is enclosed in black brackets, new matter is printed in italics, and existing law in which no change is proposed is shown in roman):

FEDERAL FOOD, DRUG, AND COSMETIC ACT

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CHAPTER V—DRUGS AND DEVICES

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SUBCHAPTER B—DRUGS FOR RARE DISEASES OR CONDITIONS

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SEC. 529. PRIORITY REVIEW TO ENCOURAGE TREATMENTS FOR RARE PEDIATRIC DISEASES.

(a) DEFINITIONS.—In this section:

(1) PRIORITY REVIEW.—The term “priority review”, with respect to a human drug application as defined in section 735(1), means review and action by the Secretary on such application not later than 6 months after receipt by the Secretary of such application, as described in the Manual of Policies and Procedures of the Food and Drug Administration and goals identified in the letters described in section 101(b) of the Prescription Drug User Fee Amendments of 2012.

(2) PRIORITY REVIEW VOUCHER.—The term “priority review voucher” means a voucher issued by the Secretary to the sponsor of a rare pediatric disease product application that entitles the holder of such voucher to priority review of a single human drug application submitted under section 505(b)(1) or section 351(a) of the Public Health Service Act after the date of approval of the rare pediatric disease product application.

(3) RARE PEDIATRIC DISEASE.—The term “rare pediatric disease” means a disease that meets each of the following criteria:

(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.

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

(4) RARE PEDIATRIC DISEASE PRODUCT APPLICATION.—The term “rare pediatric disease product application” means a human drug application, as defined in section 735(1), that—

(A) is for a drug or biological product—

(i) that is for the prevention or treatment of a rare pediatric disease; and

(ii) that contains no active ingredient (including any ester or salt of the active ingredient) that has been previously approved in any other application under section 505(b)(1), 505(b)(2), or 505(j) of this Act or section 351(a) or 351(k) of the Public Health Service Act;

(B) is submitted under section 505(b)(1) of this Act or section 351(a) of the Public Health Service Act;

(C) the Secretary deems eligible for priority review;

(D) that relies on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population;

(E) that does not seek approval for an adult indication in the original rare pediatric disease product application; and

(F) is approved after the date of the enactment of the Advancing Hope Act of 2016.

(b) PRIORITY REVIEW VOUCHER.—

(1) IN GENERAL.—The Secretary shall award a priority review voucher to the sponsor of a rare pediatric disease product application upon approval by the Secretary of such rare pediatric disease product application.

(2) TRANSFERABILITY.—

(A) IN GENERAL.—The sponsor of a rare pediatric disease product application that receives a priority review voucher under this section may transfer (including by sale) the entitlement to such voucher. There is no limit on the number of times a priority review voucher may be transferred before such voucher is used.

(B) NOTIFICATION OF TRANSFER.—Each person to whom a voucher is transferred shall notify the Secretary of such change in ownership of the voucher not later than 30 days after such transfer.

(3) LIMITATION.—A sponsor of a rare pediatric disease product application may not receive a priority review voucher under this section if the rare pediatric disease product application was submitted to the Secretary prior to the date that is 90 days after the date of enactment of the Prescription Drug User Fee Amendments of 2012.

(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 Ad-

vancing 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.

(5) TERMINATION OF AUTHORITY.—The Secretary may not award any priority review vouchers under paragraph (1) after **September 30, 2020** *September 30, 2024*, unless the rare pediatric disease product application—

(A) is for a drug that, not later than **September 30, 2020** *September 30, 2024*, is designated under subsection (d) as a drug for a rare pediatric disease; and

(B) is, not later than **September 30, 2022** *September 30, 2026*, approved under section 505(b)(1) of this Act or section 351(a) of the Public Health Service Act.

(c) PRIORITY REVIEW USER FEE.—

(1) IN GENERAL.—The Secretary shall establish a user fee program under which a sponsor of a human drug application that is the subject of a priority review voucher shall pay to the Secretary a fee determined under paragraph (2). Such fee shall be in addition to any fee required to be submitted by the sponsor under chapter VII.

(2) FEE AMOUNT.—The amount of the priority review user fee shall be determined each fiscal year by the Secretary, based on the difference between—

(A) the average cost incurred by the Food and Drug Administration in the review of a human drug application subject to priority review in the previous fiscal year; and

(B) the average cost incurred by the Food and Drug Administration in the review of a human drug application

that is not subject to priority review in the previous fiscal year.

(3) ANNUAL FEE SETTING.—The Secretary shall establish, before the beginning of each fiscal year beginning after September 30, 2012, the amount of the priority review user fee for that fiscal year.

(4) PAYMENT.—

(A) IN GENERAL.—The priority review user fee required by this subsection shall be due upon the notification by a sponsor of the intent of such sponsor to use the voucher, as specified in subsection (b)(4)(A). All other user fees associated with the human drug application shall be due as required by the Secretary or under applicable law.

(B) COMPLETE APPLICATION.—An application described under subparagraph (A) for which the sponsor requests the use of a priority review voucher shall be considered incomplete if the fee required by this subsection and all other applicable user fees are not paid in accordance with the Secretary's procedures for paying such fees.

(C) NO WAIVERS, EXEMPTIONS, REDUCTIONS, OR REFUNDS.—The Secretary may not grant a waiver, exemption, reduction, or refund of any fees due and payable under this section.

(5) OFFSETTING COLLECTIONS.—Fees collected pursuant to this subsection for any fiscal year—

(A) shall be deposited and credited as offsetting collections to the account providing appropriations to the Food and Drug Administration; and

(B) shall not be collected for any fiscal year except to the extent provided in advance in appropriations Acts.

(d) DESIGNATION PROCESS.—

(1) IN GENERAL.—Upon the request of the manufacturer or the sponsor of a new drug, the Secretary may designate—

(A) the new drug as a drug for a rare pediatric disease; and

(B) the application for the new drug as a rare pediatric disease product application.

(2) REQUEST FOR DESIGNATION.—The request for a designation under paragraph (1) shall be made at the same time a request for designation of orphan disease status under section 526 or fast-track designation under section 506 is made. Requesting designation under this subsection is not a prerequisite to receiving a priority review voucher under this section.

(3) DETERMINATION BY SECRETARY.—Not later than 60 days after a request is submitted under paragraph (1), the Secretary shall determine whether—

(A) the disease or condition that is the subject of such request is a rare pediatric disease; and

(B) the application for the new drug is a rare pediatric disease product application.

(e) MARKETING OF RARE PEDIATRIC DISEASE PRODUCTS.—

(1) REVOCATION.—The Secretary may revoke any priority review voucher awarded under subsection (b) if the rare pediatric disease product for which such voucher was awarded is not

marketed in the United States within the 365-day period beginning on the date of the approval of such drug under section 505 of this Act or section 351 of the Public Health Service Act.

(2) POSTAPPROVAL PRODUCTION REPORT.—The sponsor of an approved rare pediatric disease product shall submit a report to the Secretary not later than 5 years after the approval of the applicable rare pediatric disease product application. Such report shall provide the following information, with respect to each of the first 4 years after approval of such product:

(A) The estimated population in the United States suffering from the rare pediatric disease.

(B) The estimated demand in the United States for such rare pediatric disease product.

(C) The actual amount of such rare pediatric disease product distributed in the United States.

(f) NOTICE AND REPORT.—

(1) NOTICE OF ISSUANCE OF VOUCHER AND APPROVAL OF PRODUCTS UNDER VOUCHER.—The Secretary shall publish a notice in the Federal Register and on the Internet Web site of the Food and Drug Administration not later than 30 days after the occurrence of each of the following:

(A) The Secretary issues a priority review voucher under this section.

(B) The Secretary approves a drug pursuant to an application submitted under section 505(b) of this Act or section 351(a) of the Public Health Service Act for which the sponsor of the application used a priority review voucher under this section.

(2) NOTIFICATION.—If, after the last day of the 1-year period that begins on the date that the Secretary awards the third rare pediatric disease priority voucher under this section, a sponsor of an application submitted under section 505(b) of this Act or section 351(a) of the Public Health Service Act for a drug uses a priority review voucher under this section for such application, the Secretary shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate a document—

(A) notifying such Committees of the use of such voucher; and

(B) identifying the drug for which such priority review voucher is used.

(g) ELIGIBILITY FOR OTHER PROGRAMS.—Nothing in this section precludes a sponsor who seeks a priority review voucher under this section from participating in any other incentive program, including under this Act, 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.

(h) RELATION TO OTHER PROVISIONS.—The provisions of this section shall supplement, not supplant, any other provisions of this Act or the Public Health Service Act that encourage the development of drugs for tropical diseases and rare pediatric diseases.

(i) GAO STUDY AND REPORT.—

(1) STUDY.—

(A) IN GENERAL.—Beginning on the date that the Secretary awards the third rare pediatric disease priority voucher under this section, the Comptroller General of the United States shall conduct a study of the effectiveness of awarding rare pediatric disease priority vouchers under this section in the development of human drug products that treat or prevent such diseases.

(B) CONTENTS OF STUDY.—In conducting the study under subparagraph (A), the Comptroller General shall examine the following:

(i) The indications for which each rare disease product for which a priority review voucher was awarded was approved under section 505 or section 351 of the Public Health Service Act.

(ii) Whether, and to what extent, an unmet need related to the treatment or prevention of a rare pediatric disease was met through the approval of such a rare disease product.

(iii) The value of the priority review voucher if transferred.

(iv) Identification of each drug for which a priority review voucher was used.

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

(2) REPORT.—Not later than 1 year after the date under paragraph (1)(A), the Comptroller General shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate, a report containing the results of the study under paragraph (1).

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