section (a) [amending this section] shall apply to human drug applications submitted after September 30, 2017.''

§ 360n–1. Priority review for qualified infectious disease products

(a) In general

If the Secretary designates a drug under section 355f(d) of this title as a qualified infectious disease product, then the Secretary shall give priority review to the first application submitted for approval for such drug under section 355(b) of this title.

(b) Construction

Nothing in this section shall prohibit the Secretary from giving priority review to a human drug application or efficacy supplement submitted for approval under section 355(b) of this title that otherwise meets the criteria for the Secretary to grant priority review.

(June 25, 1938, ch. 675, § 524A, as added Pub. L. 112–144, title VIII, § 802(a), July 9, 2012, 126 Stat. 1079; amended Pub. L. 114–255, div. A, title III, § 3101(a)(2)(N), Dec. 13, 2016, 130 Stat. 1154.)

Editorial Notes

AMENDMENTS

2016—Pub. L. 114–255 designated existing provisions as subsec. (a), inserted heading, substituted ''the first application'' for ''any application'', and added subsec. (b).

Statutory Notes and Related Subsidiaries

EFFECTIVE DATE

Pub. L. 112–144, title VIII, § 802(b), July 9, 2012, 126 Stat. 1079, provided that: ''Section 524A of the Federal Food, Drug, and Cosmetic Act [21 U.S.C. 360n–1], as added by subsection (a), applies only with respect to an application that is submitted under section 505(b) of such Act (21 U.S.C. 355(b)) on or after the date of the enactment of this Act [July 9, 2012].''

PART B—DRUGS FOR RARE DISEASES OR CONDITIONS

§ 360aa. Recommendations for investigations of drugs for rare diseases or conditions

(a) Request by sponsor; response by Secretary

The sponsor of a drug for a disease or condition which is rare in the States may request the Secretary to provide written recommendations for the non-clinical and clinical investigations which must be conducted with the drug before—

(1) it may be approved for such disease or condition under section 355 of this title, or

(2) if the drug is a biological product, it may be licensed for such disease or condition under section 262 of title 42.

If the Secretary has reason to believe that a drug for which a request is made under this section is a drug for a disease or condition which is rare in the States, the Secretary shall provide the person making the request written recommendations for the non-clinical and clinical investigations which the Secretary believes, on the basis of information available to the Secretary at the time of the request under this section, would be necessary for approval of such drug for such disease or condition under section 355 of this title or licensing of such drug for such disease or condition under section 262 of title 42.

(b) Regulations

The Secretary shall by regulation promulgate procedures for the implementation of subsection (a).

(June 25, 1938, ch. 675, § 525, as added Pub. L. 97–414, § 2(a), Jan. 4, 1983, 96 Stat. 2049; amended Pub. L. 99–91, § 3(a)(1), Aug. 15, 1985, 99 Stat. 387; Pub. L. 105–115, title I, § 125(b)(2)(F), (G), Nov. 21, 1997, 111 Stat. 2325, 2326.)

Editorial Notes

AMENDMENTS

1997—Subsec. (a). Pub. L. 105–115, § 125(b)(2)(G), struck out '', certification of such drug for such disease or condition under section 357 of this title,'' before ''or licensing of such drug'' in closing provisions.

Subsec. (a)(1) to (3). Pub. L. 105–115, § 125(b)(2)(F), inserted ''or'' at end of par. (1), redesignated par. (3) as (2), and struck out former par. (2), which read as follows: ''if the drug is an antibiotic, it may be certified for such disease or condition under section 357 of this title, or''.

1985—Subsec. (a). Pub. L. 99–91 struck out ''or'' at end of par. (1), inserted par. (2), redesignated former par. (2) as (3) and struck out ''before'' after ''product,'', and in last sentence inserted provisions relating to certification of such drug for disease or condition under section 357 of this title and substituted ''licensing of such drug for such disease or condition under section 262 of title 42'' for ''licensing under section 262 of title 42 for such disease or condition''.

Statutory Notes and Related Subsidiaries

EFFECTIVE DATE OF 1985 AMENDMENT

Pub. L. 99–91, § 8, Aug. 15, 1985, 99 Stat. 392, provided that:

''(a) GENERAL RULE.—Except as provided in subsection (b), this Act and the amendments made by this Act [amending this section, sections 360bb, 360cc, and 360ee of this title, and sections 295g–1 and 6022 of Title 42, The Public Health and Welfare, and enacting provisions set out as notes under section 301 of this title and section 236 of Title 42] shall take effect October 1, 1985.

''(b) EXCEPTION.—The amendments made by sections 2, 3, and 6(a) [amending this section and sections 360bb and 360cc of this title] shall take effect on the date of the enactment of this Act [Aug. 15, 1985]. The amendment made by section 6(b) [amending section 6022 of Title 42] shall take effect October 19, 1984. The amendments made by section 7 [amending section 295g–1 of Title 42] shall take effect October 1, 1984 and shall cease to be in effect after September 30, 1985.''

ALS AND OTHER RARE NEURODEGENERATIVE DISEASE ACTION PLAN

Pub. L. 117–79, § 4, Dec. 23, 2021, 135 Stat. 1536, provided that:

''(a) IN GENERAL.—Not later than 6 months after the date of enactment of this Act [Dec. 23, 2021], the Commissioner of Food and Drugs shall publish on the website of the Food and Drug Administration an action plan describing actions the Food and Drug Administration intends to take during the 5-year period following publication of the plan with respect to program enhancements, policy development, regulatory science initiatives, and other appropriate initiatives to—

''(1) foster the development of safe and effective drugs that improve or extend, or both, the lives of people living with amyotrophic lateral sclerosis and other rare neurodegenerative diseases; and

''(2) facilitate access to investigational drugs for amyotrophic lateral sclerosis and other rare neurodegenerative diseases.

''(b) CONTENTS.—The initial action plan published under subsection (a) shall—

''(1) identify appropriate representation from within the Food and Drug Administration to be responsible for implementation of such action plan;

''(2) include elements to facilitate—

''(A) interactions and collaboration between the Food and Drug Administration, including the review centers thereof, and stakeholders including patients, sponsors, and the external biomedical research community;

''(B) consideration of cross-cutting clinical and regulatory policy issues, including consistency of regulatory advice and decisionmaking;

''(C) identification of key regulatory science and policy issues critical to advancing development of safe and effective drugs; and

''(D) enhancement of collaboration and engagement of the relevant centers and offices of the Food and Drug Administration with other operating divisions within the Department of Health and Human Services, the Partnership, and the broader neurodegenerative disease community; and

''(3) be subject to revision, as determined appropriate by the Secretary of Health and Human Services.''

REVIEW GROUPS ON RARE DISEASES AND NEGLECTED DISEASES OF THE DEVELOPING WORLD; REPORT; GUIDANCE; STANDARDS

Pub. L. 111–80, title VII, § 740, Oct. 21, 2009, 123 Stat. 2127, provided that:

''(a) The Commissioner of Food and Drugs shall establish within the Food and Drug Administration a review group which shall recommend to the Commissioner of Food and Drugs appropriate preclinical, trial design, and regulatory paradigms and optimal solutions for the prevention, diagnosis, and treatment of rare diseases: *Provided*, That the Commissioner of Food and Drugs shall appoint individuals employed by the Food and Drug Administration to serve on the review group: *Provided further*, That members of the review group shall have specific expertise relating to the development of articles for use in the prevention, diagnosis, or treatment of rare diseases, including specific expertise in developing or carrying out clinical trials.

''(b) The Commissioner of Food and Drugs shall establish within the Food and Drug Administration a review group which shall recommend to the Commissioner of Food and Drugs appropriate preclinical, trial design, and regulatory paradigms and optimal solutions for the prevention, diagnosis, and treatment of neglected diseases of the developing world: *Provided*, That the Commissioner of Food and Drugs shall appoint individuals employed by the Food and Drug Administration to serve on the review group: *Provided further*, That members of the review group shall have specific expertise relating to the development of articles for use in the prevention, diagnosis, or treatment of neglected diseases of the developing world, including specific expertise in developing or carrying out clinical trials: *Provided further*, That for the purposes of this section the term 'neglected disease of the developing world' means a tropical disease, as defined in section 524(a)(3) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360n(a)(3)).

''(c) The Commissioner of Food and Drugs shall—

''(1) submit, not later than 1 year after the date of the establishment of review groups under subsections (a) and (b), a report to Congress that describes both the findings and recommendations made by the review groups under subsections (a) and (b);

''(2) issue, not later than 180 days after submission of the report to Congress under paragraph (1), guidance based on such recommendations for articles for use in the prevention, diagnosis, and treatment of rare diseases and for such uses in neglected diseases of the developing world; and

''(3) develop, not later than 180 days after submission of the report to Congress under paragraph (1), internal review standards based on such recommendations for articles for use in the prevention, diagnosis, and treatment of rare diseases and for such uses in neglected diseases of the developing world.''

STUDY

Pub. L. 100–290, § 3(d), Apr. 18, 1988, 102 Stat. 91, directed Secretary of Health and Human Services to conduct a study to determine whether the application of subchapter B of chapter V of the Federal Food, Drug, and Cosmetic Act, 21 U.S.C. 360aa et seq. (relating to drugs for rare diseases and conditions), and 26 U.S.C. 28 (relating to tax credit) to medical devices or medical foods for rare diseases or conditions or to both was needed to encourage development of such devices and foods and report results of the study to Congress not later than one year after Apr. 18, 1988.

CONGRESSIONAL FINDINGS

Pub. L. 97–414, § 1(b), Jan. 4, 1983, 96 Stat. 2049, provided that: ''The Congress finds that—

''(1) there are many diseases and conditions, such as Huntington's disease, myoclonus, ALS (Lou Gehrig's disease), Tourette syndrome, and muscular dystrophy which affect such small numbers of individuals residing in the United States that the diseases and conditions are considered rare in the United States;

''(2) adequate drugs for many of such diseases and conditions have not been developed;

''(3) drugs for these diseases and conditions are commonly referred to as 'orphan drugs';

''(4) because so few individuals are affected by any one rare disease or condition, a pharmaceutical company which develops an orphan drug may reasonably expect the drug to generate relatively small sales in comparison to the cost of developing the drug and consequently to incur a financial loss;

''(5) there is reason to believe that some promising orphan drugs will not be developed unless changes are made in the applicable Federal laws to reduce the costs of developing such drugs and to provide financial incentives to develop such drugs; and

''(6) it is in the public interest to provide such changes and incentives for the development of orphan drugs.''

§ 360bb. Designation of drugs for rare diseases or conditions

(a) Request by sponsor; preconditions; "rare disease or condition" defined

(1) The manufacturer or the sponsor of a drug may request the Secretary to designate the drug as a drug for a rare disease or condition. A request for designation of a drug shall be made before the submission of an application under section 355(b) of this title for the drug, or the submission of an application for licensing of the drug under section 262 of title 42. If the Secretary finds that a drug for which a request is submitted under this subsection is being or will be investigated for a rare disease or condition and—

(A) if an application for such drug is approved under section 355 of this title, or

(B) if a license for such drug is issued under section 262 of title 42,

the approval, certification, or license would be for use for such disease or condition, the Secretary shall designate the drug as a drug for such disease or condition. A request for a designation of a drug under this subsection shall contain the consent of the applicant to notice being given by the Secretary under subsection (b) respecting the designation of the drug.

(2) For purposes of paragraph (1), the term ''rare disease or condition'' means any disease or condition which (A) affects less than 200,000 persons in the United States, or (B) affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from sales in the United States of such drug. Determinations under the preceding sentence with respect to any drug shall be made on the basis of the facts and circumstances as of the date the request for designation of the drug under this subsection is made.

(b) Notification of discontinuance of drug or application as condition

A designation of a drug under subsection (a) shall be subject to the condition that—

(1) if an application was approved for the drug under section 355(b) of this title or a license was issued for the drug under section 262 of title 42, the manufacturer of the drug will notify the Secretary of any discontinuance of the production of the drug at least one year before discontinuance, and

(2) if an application has not been approved for the drug under section 355(b) of this title or a license has not been issued for the drug under section 262 of title 42 and if preclinical investigations or investigations under section 355(i) of this title are being conducted with the drug, the manufacturer or sponsor of the drug will notify the Secretary of any decision to discontinue active pursuit of approval of an application under section 355(b) of this title or approval of a license under section 262 of title 42.

(c) Notice to public

Notice respecting the designation of a drug under subsection (a) shall be made available to the public.

(d) Regulations

The Secretary shall by regulation promulgate procedures for the implementation of subsection (a).

(June 25, 1938, ch. 675, § 526, as added Pub. L. 97–414, § 2(a), Jan. 4, 1983, 96 Stat. 2050; amended Pub. L. 98–551, § 4(a), Oct. 30, 1984, 98 Stat. 2817; Pub. L. 99–91, § 3(a)(2), Aug. 15, 1985, 99 Stat. 387; Pub. L. 100–290, § 2, Apr. 18, 1988, 102 Stat. 90; Pub. L. 105–115, title I, § 125(b)(2)(H), (I), Nov. 21, 1997, 111 Stat. 2326.)

Editorial Notes

AMENDMENTS

1997—Subsec. (a)(1). Pub. L. 105–115, § 125(b)(2)(H), struck out ''the submission of an application for certification of the drug under section 357 of this title,'' before ''or the submission of an application for licensing of the drug'' in introductory provisions, inserted ''or'' at end of subpar. (A), redesignated subpar. (C) as (B), and struck out former subpar. (B) which read as follows: ''if a certification for such drug is issued under section 357 of this title, or''.

Subsec. (b)(1). Pub. L. 105–115, § 125(b)(2)(I)(i), struck out '', a certificate was issued for the drug under section 357 of this title,'' before ''or a license was issued''.

Subsec. (b)(2). Pub. L. 105–115, § 125(b)(2)(I)(ii), struck out '', a certificate has not been issued for the drug under section 357 of this title,'' before ''or a license has not been issued'' and '', approval of an application for certification under section 357 of this title,'' before ''or approval of a license''.

1988—Subsec. (a)(1). Pub. L. 100–290, § 2(a), inserted after first sentence ''A request for designation of a drug shall be made before the submission of an application under section 355(b) of this title for the drug, the submission of an application for certification of the drug under section 357 of this title, or the submission of an application for licensing of the drug under section 262 of title 42.''

Subsecs. (b) to (d). Pub. L. 100–290, § 2(b), added subsec. (b) and redesignated former subsecs. (b) and (c) as (c) and (d), respectively.

1985—Subsec. (a)(1). Pub. L. 99–91 struck out ''or'' at end of subpar. (A), struck out subpar. (B) and substituted subpars. (B) and (C), and inserted '', certification,'' after ''approval''.

1984—Subsec. (a)(2). Pub. L. 98–551 substituted ''which (A) affects less than 200,000 persons in the United States, or (B) affects more than 200,000 in the United States and for which'' for ''which occurs so infrequently in the United States that''.

Statutory Notes and Related Subsidiaries

EFFECTIVE DATE OF 1985 AMENDMENT

Amendment by Pub. L. 99–91 effective Aug. 15, 1985, see section 8(b) of Pub. L. 99–91, set out as a note under section 360aa of this title.

§ 360cc. Protection for drugs for rare diseases or conditions

(a) Exclusive approval, certification, or license

Except as provided in subsection (b), if the Secretary—

(1) approves an application filed pursuant to section 355 of this title, or

(2) issues a license under section 262 of title 42

for a drug designated under section 360bb of this title for a rare disease or condition, the Secretary may not approve another application under section 355 of this title or issue another license under section 262 of title 42 for the same drug for the same disease or condition for a person who is not the holder of such approved application or of such license until the expiration of seven years from the date of the approval of the approved application or the issuance of the license. Section 355(c)(2)[1] of this title does not apply to the refusal to approve an application under the preceding sentence.

(b) Exceptions

During the 7-year period described in subsection (a) for an approved application under section 355 of this title or license under section 262 of title 42, the Secretary may approve an application or issue a license for a drug that is otherwise the same, as determined by the Secretary, as the already approved drug for the same rare disease or condition if—

(1) the Secretary finds, after providing the holder of exclusive approval or licensure notice and opportunity for the submission of views, that during such period the holder of the exclusive approval or licensure cannot ensure the availability of sufficient quantities of the drug to meet the needs of persons with the

[1] See References in Text note below.

disease or condition for which the drug was designated; or

(2) the holder provides the Secretary in writing the consent of such holder for the approval of other applications or the issuance of other licenses before the expiration of such seven-year period.

(c) Condition of clinical superiority

(1) In general

If a sponsor of a drug that is designated under section 360bb of this title and is otherwise the same, as determined by the Secretary, as an already approved or licensed drug is seeking exclusive approval or exclusive licensure described in subsection (a) for the same rare disease or condition as the already approved drug, the Secretary shall require such sponsor, as a condition of such exclusive approval or licensure, to demonstrate that such drug is clinically superior to any already approved or licensed drug that is the same drug.

(2) Definition

For purposes of paragraph (1), the term ''clinically superior'' with respect to a drug means that the drug provides a significant therapeutic advantage over and above an already approved or licensed drug in terms of greater efficacy, greater safety, or by providing a major contribution to patient care.

(3) Applicability

This subsection applies to any drug designated under section 360bb of this title for which an application was approved under section 355 of this title or licensed under section 262 of title 42 after August 18, 2017, regardless of the date on which such drug was designated under section 360bb of this title.

(d) Regulations

The Secretary may promulgate regulations for the implementation of subsection (c). Beginning on August 18, 2017, until such time as the Secretary promulgates regulations in accordance with this subsection, the Secretary may apply any definitions set forth in regulations that were promulgated prior to such date, to the extent such definitions are not inconsistent with the terms of this section, as amended by such Act.

(e) Demonstration of clinical superiority standard

To assist sponsors in demonstrating clinical superiority as described in subsection (c), the Secretary—

(1) upon the designation of any drug under section 360bb of this title, shall notify the sponsor of such drug in writing of the basis for the designation, including, as applicable, any plausible hypothesis offered by the sponsor and relied upon by the Secretary that the drug is clinically superior to a previously approved drug; and

(2) upon granting exclusive approval or licensure under subsection (a) on the basis of a demonstration of clinical superiority as described in subsection (c), shall publish a summary of the clinical superiority findings.

(June 25, 1938, ch. 675, § 527, as added Pub. L. 97–414, § 2(a), Jan. 4, 1983, 96 Stat. 2050; amended Pub. L. 98–417, title I, § 102(b)(6), Sept. 24, 1984, 98 Stat. 1593; Pub. L. 99–91, §§ 2, 3(a)(3), Aug. 15, 1985, 99 Stat. 387, 388; Pub. L. 103–80, § 3(v), Aug. 13, 1993, 107 Stat. 778; Pub. L. 105–115, title I, § 125(b)(2)(J), (K), Nov. 21, 1997, 111 Stat. 2326; Pub. L. 107–281, § 4, Nov. 6, 2002, 116 Stat. 1993; Pub. L. 115–52, title VI, § 607(a), Aug. 18, 2017, 131 Stat. 1049; Pub. L. 116–260, div. BB, title III, § 323, Dec. 27, 2020, 134 Stat. 2933.)

Editorial Notes

REFERENCES IN TEXT

Section 355(c)(2) of this title, referred to in subsec. (a), was redesignated as section 355(c)(1)(B) of this title by Pub. L. 98–417, title I, § 102(a)(2), Sept. 24, 1984, 98 Stat. 1592.

This section, as amended by such Act, referred to in subsec. (d), means this section as amended by the FDA Reauthorization Act of 2017, Pub. L. 115–52.

AMENDMENTS

2020—Subsec. (c)(3). Pub. L. 116–260 added par. (3).

2017—Subsec. (a). Pub. L. 115–52, § 607(a)(1), substituted ''the same drug for the same disease or condition'' for ''such drug for such disease or condition'' in concluding provisions.

Subsec. (b). Pub. L. 115–52, § 607(a)(2)(A), in introductory provisions, substituted ''During the 7-year period described in subsection (a) for an approved application under section 355 of this title or license under section 262 of title 42, the Secretary may approve an application or issue a license for a drug that is otherwise the same, as determined by the Secretary, as the already approved drug for the same rare disease or condition if'' for ''If an application filed pursuant to section 355 of this title is approved for a drug designated under section 360bb of this title for a rare disease or condition or if a license is issued under section 262 of title 42 for such a drug, the Secretary may, during the seven-year period beginning on the date of the application approval or of the issuance of the license, approve another application under section 355 of this title or issue a license under section 262 of title 42, for such drug for such disease or condition for a person who is not the holder of such approved application or of such license if''.

Subsec. (b)(1). Pub. L. 115–52, § 607(a)(2)(B), substituted ''of exclusive approval or licensure notice and opportunity for the submission of views, that during such period the holder of the exclusive approval or licensure cannot ensure'' for ''notice and opportunity for the submission of views, that in such period the holder of the approved application or of the license cannot assure''.

Subsec. (b)(2). Pub. L. 115–52, § 607(a)(2)(C), substituted ''the holder provides'' for ''such holder provides''.

Subsecs. (c) to (e). Pub. L. 115–52, § 607(a)(3), added subsecs. (c) to (e).

2002—Subsec. (a). Pub. L. 107–281, in concluding provisions, struck out '', of such certification,'' after ''such approved application'' and '', the issuance of the certification,'' after ''approval of the approved application''.

1997—Subsec. (a). Pub. L. 105–115, § 125(b)(2)(J), struck out '', issue another certification under section 357 of this title,'' before ''or issue another license'' in closing provisions, inserted ''or'' at end of par. (1), redesignated par. (3) as (2), and struck out former par. (2) which read as follows: ''issues a certification under section 357 of this title, or''.

Subsec. (b). Pub. L. 105–115, § 125(b)(2)(K), in introductory provisions, struck out '', if a certification is issued under section 357 of this title for such a drug,'' after ''rare disease or condition'', '', of the issuance of the certification under section 357 of this title,'' after ''application approval'', '', issue another certification

under section 357 of this title,'' after ''application under section 355 of this title'', and '', of such certification,'' after ''approved application''.

Subsec. (b)(1). Pub. L. 105–115, § 125(b)(2)(K), struck out '', of the certification,'' after ''holder of the approved application''.

Subsec. (b)(2). Pub. L. 105–115, § 125(b)(2)(K), struck out '', issuance of other certifications,'' after ''approval of other applications''.

1993—Subsec. (b). Pub. L. 103–80 struck out extraneous comma before ''or issue a license under section 262'' in introductory provisions and substituted ''the'' for ''The'' at beginning of par. (1).

1985—Pub. L. 99–91, § 2(3), struck out ''unpatented'' before ''drugs'' in section catchline.

Subsec. (a). Pub. L. 99–91, §§ 2(1), 3(a)(3)(A)–(D), struck out ''or'' at end of par. (1), added par. (2), redesignated former par. (2) as (3), struck out ''and for which a United States Letter of Patent may not be issued'' after ''rare disease or condition'', inserted in first sentence '', issue another certification under section 357 of this title,'' after ''section 355 of this title'' the second time it appeared, inserted '', of such certification,'' after ''holder of such approved application'', and inserted '', the issuance of the certification,'' after ''approval of the approved application''.

Subsec. (b). Pub. L. 99–91, §§ 2(2), 3(a)(3)(E)–(K), struck out ''and if a United States Letter of Patent may not be issued for the drug'' after ''such a drug'', substituted '', if a certification is issued under section 357 of this title for such a drug, or if a license'' for ''or a license'', inserted '', of the issuance of the certification under section 357 of this title,'' after ''application approval'', struck out '', if the drug is a biological product,'' before ''issue a license'', inserted '', issue another certification under section 357 of this title,'' after ''section 355 of this title'', inserted '', of such certification,'' after ''holder of such approved application'', inserted '', of such certification,'' after ''application'' in par. (1), and inserted '', issuance of other certifications,'' after ''other applications'' in par. (2).

1984—Subsecs. (a), (b). Pub. L. 98–417 substituted ''section 355'' for ''section 355(b)'' wherever appearing.

Statutory Notes and Related Subsidiaries

EFFECTIVE DATE OF 1985 AMENDMENT

Amendment by Pub. L. 99–91 effective Aug. 15, 1985, see section 8(b) of Pub. L. 99–91, set out as a note under section 360aa of this title.

CONSTRUCTION

Pub. L. 115–52, title VI, § 607(b), Aug. 18, 2017, 131 Stat. 1050, provided that: ''Nothing in the amendments made by subsection (a) [amending this section] shall affect any determination under sections 526 and 527 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb, 360cc) made prior to the date of enactment of the FDA Reauthorization Act of 2017 [Aug. 18, 2017].''

§ 360dd. Open protocols for investigations of drugs for rare diseases or conditions

If a drug is designated under section 360bb of this title as a drug for a rare disease or condition and if notice of a claimed exemption under section 355(i) of this title or regulations issued thereunder is filed for such drug, the Secretary shall encourage the sponsor of such drug to design protocols for clinical investigations of the drug which may be conducted under the exemption to permit the addition to the investigations of persons with the disease or condition who need the drug to treat the disease or condition and who cannot be satisfactorily treated by available alternative drugs.

(June 25, 1938, ch. 675, § 528, as added Pub. L. 97–414, § 2(a), Jan. 4, 1983, 96 Stat. 2051.)

§ 360ee. Grants and contracts for development of drugs for rare diseases and conditions

(a) Authority of Secretary

The Secretary may make grants to and enter into contracts with public and private entities and individuals to assist in (1) defraying the costs of developing drugs for rare diseases or conditions, including qualified testing expenses, (2) defraying the costs of developing medical devices for rare diseases or conditions, and (3) defraying the costs of developing medical foods for rare diseases or conditions.

(b) Definitions

For purposes of subsection (a):

(1) The term ''qualified testing'' means—

(A) human clinical testing—

(i) which is carried out under an exemption for a drug for a rare disease or condition under section 355(i) of this title (or regulations issued under such section); and

(ii) which occurs before the date on which an application with respect to such drug is submitted under section 355(b) of this title or under section 262 of title 42;

(B) preclinical testing involving a drug for a rare disease or condition which occurs after the date such drug is designated under section 360bb of this title and before the date on which an application with respect to such drug is submitted under section 355(b) of this title or under section 262 of title 42; and

(C) prospectively planned and designed observational studies and other analyses conducted to assist in the understanding of the natural history of a rare disease or condition and in the development of a therapy, including studies and analyses to—

(i) develop or validate a drug development tool related to a rare disease or condition; or

(ii) understand the full spectrum of the disease manifestations, including describing genotypic and phenotypic variability and identifying and defining distinct subpopulations affected by a rare disease or condition.

(2) The term ''rare disease or condition'' means (1) in the case of a drug, any disease or condition which (A) affects less than 200,000 persons in the United States, or (B) affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from sales in the United States of such drug, (2) in the case of a medical device, any disease or condition that occurs so infrequently in the United States that there is no reasonable expectation that a medical device for such disease or condition will be developed without assistance under subsection (a), and (3) in the case of a medical food, any disease or condition that occurs so infrequently in the United States that there is no reasonable expectation that a medical food for such disease or condition will be developed without assistance under subsection (a). Determinations under the preceding sentence with respect to any drug shall be made on the

basis of the facts and circumstances as of the date the request for designation of the drug under section 360bb of this title is made.

(3) The term ''medical food'' means a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation.

(c) Authorization of appropriations

For grants and contracts under subsection (a), there is authorized to be appropriated $30,000,000 for each of fiscal years 2018 through 2022.

(Pub. L. 97–414, § 5, Jan. 4, 1983, 96 Stat. 2056; Pub. L. 98–551, § 4(b), Oct. 30, 1984, 98 Stat. 2817; Pub. L. 99–91, § 5, Aug. 15, 1985, 99 Stat. 391; Pub. L. 100–290, § 3(a)–(c), Apr. 18, 1988, 102 Stat. 90, 91; Pub. L. 105–115, title I, § 125(b)(2)(N), Nov. 21, 1997, 111 Stat. 2326; Pub. L. 107–281, § 3, Nov. 6, 2002, 116 Stat. 1993; Pub. L. 110–85, title XI, § 1112(b), Sept. 27, 2007, 121 Stat. 976; Pub. L. 112–144, title IX, § 906, July 9, 2012, 126 Stat. 1092; Pub. L. 114–255, div. A, title III, § 3015, Dec. 13, 2016, 130 Stat. 1094; Pub. L. 115–52, title VI, § 603, Aug. 18, 2017, 131 Stat. 1048.)

Editorial Notes

CODIFICATION

Section was enacted as part of the Orphan Drug Act, and not as part of the Federal Food, Drug, and Cosmetic Act which comprises this chapter.

AMENDMENTS

2017—Subsec. (c). Pub. L. 115–52 substituted ''2018 through 2022'' for ''2013 through 2017''.

2016—Subsec. (a)(1). Pub. L. 114–255, § 3015(1), added par. (1) and struck out former par. (1) which read as follows: ''defraying the costs of qualified testing expenses incurred in connection with the development of drugs for rare diseases and conditions,''.

Subsec. (b)(1)(C). Pub. L. 114–255, § 3015(2), added subpar. (C).

2012—Subsec. (b)(1)(A)(ii). Pub. L. 112–144, § 906(a), struck out ''after the date such drug is designated under section 360bb of this title and'' after ''which occurs''.

Subsec. (c). Pub. L. 112–144, § 906(b), amended subsec. (c) generally. Prior to amendment, text read as follows: ''For grants and contracts under subsection (a), there is authorized to be appropriated $30,000,000 for each of fiscal years 2008 through 2012.''

2007—Subsec. (c). Pub. L. 110–85 amended subsec. (c) generally. Prior to amendment, subsec. (c) read as follows: ''For grants and contracts under subsection (a) of this section, there are authorized to be appropriated such sums as already have been appropriated for fiscal year 2002, and $25,000,000 for each of the fiscal years 2003 through 2006.''

2002—Subsec. (c). Pub. L. 107–281 amended subsec. (c) generally. Prior to amendment, subsec. (c) read as follows: ''For grants and contracts under subsection (a) of this section there are authorized to be appropriated $10,000,000 for fiscal year 1988, $12,000,000 for fiscal year 1989, $14,000,000 for fiscal year 1990.''

1997—Subsec. (b)(1)(A)(ii), (B). Pub. L. 105–115 struck out ''or 357'' after ''355(b)''.

1988—Subsec. (a). Pub. L. 100–290, § 3(a)(1), (b)(1), inserted ''(1)'' after ''assist in'' and added pars. (2) and (3).

Subsec. (b)(2). Pub. L. 100–290, § 3(a)(2), (b)(2), inserted ''(1) in the case of a drug,'' after ''means'', added cls. (2) and (3), and substituted ''under section 360bb of this title'' for ''under this subsection'' in last sentence.

Subsec. (b)(3). Pub. L. 100–290, § 3(b)(3), added par. (3).

Subsec. (c). Pub. L. 100–290, § 3(c), amended subsec. (c) generally. Prior to amendment, subsec. (c) read as follows: ''For grants and contracts under subsection (a) of this section there are authorized to be appropriated $4,000,000 for fiscal year 1986, $4,000,000 for fiscal year 1987, and $4,000,000 for fiscal year 1988.''

1985—Subsec. (a). Pub. L. 99–91, § 5(a)(1), struck out ''clinical'' before ''testing''.

Subsec. (b)(1). Pub. L. 99–91, § 5(a)(2), substituted provisions defining ''qualified testing'' for provisions defining ''qualified clinical testing''.

Subsec. (c). Pub. L. 99–91, § 5(b), substituted provisions authorizing appropriations for fiscal years 1986 to 1988, for provisions authorizing appropriations for fiscal years 1983 and the two succeeding fiscal years.

1984—Subsec. (b)(2). Pub. L. 98–551 substituted ''which (A) affects less than 200,000 persons in the United States, or (B) affects more than 200,000 in the United States and for which'' for ''which occurs so infrequently in the United States that''.

Statutory Notes and Related Subsidiaries

EFFECTIVE DATE OF 1985 AMENDMENT

Amendment by Pub. L. 99–91 effective Oct. 1, 1985, see section 8(a) of Pub. L. 99–91, set out as a note under section 360aa of this title.

GRANTS FOR RESEARCH ON THERAPIES FOR ALS

Pub. L. 117–79, § 2, Dec. 23, 2021, 135 Stat. 1533, provided that:

''(a) IN GENERAL.—The Secretary of Health and Human Services (referred to in this section as the 'Secretary') shall award grants to participating entities for purposes of scientific research utilizing data from expanded access to investigational drugs for individuals who are not otherwise eligible for clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis. In the case of a participating entity seeking such a grant, an expanded access request must be submitted, and allowed to proceed by the Secretary, under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) and part 312 of title 21, Code of Federal Regulations (or any successor regulations), before the application for such grant is submitted.

''(b) APPLICATION.—

''(1) IN GENERAL.—A participating entity seeking a grant under this section shall submit to the Secretary an application at such time, in such manner, and containing such information as the Secretary shall specify.

''(2) USE OF DATA.—An application submitted under paragraph (1) shall include a description of how data generated through an expanded access request under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) with respect to the investigational drug involved will be used to support research or development related to the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis.

''(3) NONINTERFERENCE WITH CLINICAL TRIALS.—An application submitted under paragraph (1) shall include a description of how the proposed expanded access program will be designed so as not to interfere with patient enrollment in ongoing clinical trials for investigational therapies for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis.

''(c) SELECTION.—Consistent with sections 406 and 492 of the Public Health Service Act (42 U.S.C. 284a, 289a), the Secretary shall, in determining whether to award a grant under this section, confirm that—

''(1) such grant will be used to support a scientific research objective relating to the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis (as described in subsection (a));

''(2) such grant shall not have the effect of diminishing eligibility for, or impeding enrollment of, ongoing clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis by determining that individuals who receive expanded access to investigational drugs through such a grant are not eligible for enrollment in—

''(A) ongoing clinical trials that are registered on ClinicalTrials.gov (or successor website), with respect to a drug for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis; or

''(B) clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis for which an exemption under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) has been granted by the Food and Drug Administration and which are expected to begin enrollment within one year; and

''(3) the resulting project funded by such grant will allow for equitable access to investigational drugs by minority and underserved populations.

''(d) USE OF FUNDS.—A participating entity shall use funds received through the grant—

''(1) to pay the manufacturer or sponsor for the direct costs of the investigational drug, as authorized under section 312.8(d) of title 21, Code of Federal Regulations (or successor regulations), to prevent, diagnose, mitigate, treat, or cure amyotrophic lateral sclerosis that is the subject of an expanded access request described in subsection (a), if such costs are justified as part of peer review of the grant;

''(2) for the entity's direct costs incurred in providing such drug consistent with the research mission of the grant; or

''(3) for the direct and indirect costs of the entity in conducting research with respect to such drug.

''(e) DEFINITIONS.—In this section:

''(1) The term 'participating entity' means a participating clinical trial site or sites sponsored by a small business concern (as defined in section 3(a) of the Small Business Act (15 U.S.C. 632(a))) that is the sponsor of a drug that is the subject of an investigational new drug application under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) to prevent, diagnose, mitigate, treat, or cure amyotrophic lateral sclerosis.

''(2) The term 'participating clinical trial' means a phase 3 clinical trial conducted pursuant to an exemption under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) or section 351(a) of the Public Health Service Act (42 U.S.C. 262(a)) to investigate a drug intended to prevent, diagnose, mitigate, treat, or cure amyotrophic lateral sclerosis.

''(3) The term 'participating clinical trial site' means a health care facility, or network of facilities, at which patients participating in a participating clinical trial receive an investigational drug through such trial.

''(f) SUNSET.—The Secretary may not award grants under this section on or after September 30, 2026.''

FINDINGS AND PURPOSES

Pub. L. 107–281, § 2, Nov. 6, 2002, 116 Stat. 1992, provided that:

''(a) FINDINGS.—Congress makes the following findings:

''(1) Rare diseases and disorders are those which affect small patient populations, typically populations smaller than 200,000 individuals in the United States. Such diseases and conditions include Huntington's disease, amyotrophic lateral sclerosis (Lou Gehrig's disease), Tourette syndrome, Crohn's disease, cystic fibrosis, cystinosis, and Duchenne muscular dystrophy.

''(2) For many years, the 25,000,000 Americans suffering from the over 6,000 rare diseases and disorders were denied access to effective medicines because prescription drug manufacturers could rarely make a profit from marketing drugs for such small groups of patients. The prescription drug industry did not adequately fund research into such treatments. Despite the urgent health need for these medicines, they came to be known as 'orphan drugs' because no companies would commercialize them.

''(3) During the 1970s, an organization called the National Organization for Rare Disorders (NORD) was founded to provide services and to lobby on behalf of patients with rare diseases and disorders. NORD was instrumental in pressing Congress for legislation to encourage the development of orphan drugs.

''(4) The Orphan Drug Act [see Short Title of 1983 Amendments note set out under section 301 of this title] created financial incentives for the research and production of such orphan drugs. New Federal programs at the National Institutes of Health and the Food and Drug Administration encouraged clinical research and commercial product development for products that target rare diseases. An Orphan Products Board was established to promote the development of drugs and devices for rare diseases or disorders.

''(5) Before 1983, some 38 orphan drugs had been developed. Since the enactment of the Orphan Drug Act [Jan. 4, 1983], more than 220 new orphan drugs have been approved and marketed in the United States and more than 800 additional drugs are in the research pipeline.

''(6) Despite the tremendous success of the Orphan Drug Act, rare diseases and disorders deserve greater emphasis in the national biomedical research enterprise.

''(7) The Food and Drug Administration supports small clinical trials through Orphan Products Research Grants. Such grants embody successful partnerships of government and industry, and have led to the development of at least 23 drugs and four medical devices for rare diseases and disorders. Yet the appropriations in fiscal year 2001 for such grants were less than in fiscal year 1995.

''(b) PURPOSES.—The purpose of this Act [see Short Title of 2002 Amendments note set out under section 301 of this title] is to increase the national investment in the development of diagnostics and treatments for patients with rare diseases and disorders.''

§ 360ee–1. FDA rare neurodegenerative disease grant program

The Secretary of Health and Human Services, acting through the Commissioner of Food and Drugs, shall award grants and contracts to public and private entities to cover the costs of research on, and development of interventions intended to prevent, diagnose, mitigate, treat, or cure, amyotrophic lateral sclerosis and other rare neurodegenerative diseases in adults and children, including costs incurred with respect to the development and critical evaluation of tools, methods, and processes—

(1) to characterize such neurodegenerative diseases and their natural history;

(2) to identify molecular targets for such neurodegenerative diseases; and

(3) to increase efficiency and productivity of clinical development of therapies, including through—

(A) the use of master protocols and adaptive and add-on clinical trial designs; and

(B) efforts to establish new or leverage existing clinical trial networks.

(Pub. L. 117–79, § 5, Dec. 23, 2021, 135 Stat. 1537.)

Editorial Notes

CODIFICATION

Section was enacted as part of the Accelerating Access to Critical Therapies for ALS Act, and not as part

of the Federal Food, Drug, and Cosmetic Act which comprises this chapter.

§ 360ff. 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 379g(1) of this title, 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 355(b)(1) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] 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 360bb of this title.

(4) Rare pediatric disease product application

The term ''rare pediatric disease product application'' means a human drug application, as defined in section 379g(1) of this title, that—

(A) is for a drug or biological product that is for the prevention or treatment of a rare pediatric disease;

(B)(i) is for such a drug—

(I) that contains no active moiety (as defined by the Secretary in section 314.3 of title 21, Code of Federal Regulations (or any successor regulations)) that has been previously approved in any other application under subsection (b)(1), (b)(2), or (j) of section 355 of this title; and

(II) that is the subject of an application submitted under section 355(b)(1) of this title; or

(ii) is for such a biological product—

(I) that contains no active ingredient that has been previously approved in any other application under section 351(a) or 351(k) of the Public Health Service Act [42 U.S.C. 262(a), 262(k)]; and

(II) that is the subject of an application submitted under section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)];

(C) the Secretary deems eligible for priority review;

(D) that[1] relies on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population;

(E) that[1] does not seek approval for an adult indication in the original rare pediatric disease product application; and

(F) is approved after September 30, 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 July 9, 2012.

(4) Notification

(A) Sponsor of a rare pediatric disease product

(i) In general

Beginning on the date that is 90 days after September 30, 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 September 30, 2016, shall be considered eligible for a priority review voucher, if—

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

[1] So in original. The word ''that'' probably should not appear.

(aa) on or after the date that is 90 days after July 9, 2012; and

(bb) on or before September 30, 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, 2024, unless the rare pediatric disease product application—

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

(B) is, not later than September 30, 2026, approved under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)].

(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 subchapter 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 360bb of this title or fast-track designation under section 356 of this title 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 355 of this title or section 351 of the Public Health Service Act [42 U.S.C. 262].

(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 355(b) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] 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 355(b) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] 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 chapter, 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 chapter with respect to the drug for which the application is made..[2]

(h) Relation to other provisions

The provisions of this section shall supplement, not supplant, any other provisions of this chapter or the Public Health Service Act [42 U.S.C. 201 et seq.] 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 355 of this title or section 351 of the Public Health Service Act [42 U.S.C. 262].

(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).

(June 25, 1938, ch. 675, § 529, as added Pub. L. 112–144, title IX, § 908, July 9, 2012, 126 Stat. 1094; amended Pub. L. 114–113, div. A, title VII, § 765, Dec. 18, 2015, 129 Stat. 2286; Pub. L. 114–229, § 2(a), Sept. 30, 2016, 130 Stat. 943; Pub. L. 114–255, div. A, title III, § 3013(a), Dec. 13, 2016, 130 Stat. 1093; Pub. L. 116–159, div. C, title I, § 2105, Oct. 1, 2020, 134 Stat. 729; Pub. L. 116–215, div. B, title II, § 1211, Dec. 11, 2020, 134 Stat. 1045; Pub. L. 116–260, div. BB, title III, § 321, Dec. 27, 2020, 134 Stat. 2932; Pub. L. 117–9, § 1(a)(4), Apr. 23, 2021, 135 Stat. 257.)

[2] So in original.

Editorial Notes

REFERENCES IN TEXT

Section 101(b) of the Prescription Drug User Fee Amendments of 2012, referred to in subsec. (a)(1), is section 101(b) of Pub. L. 112–144, which is set out as a note under section 379g of this title.

The Public Health Service Act, referred to in subsec. (h), is act July 1, 1944, ch. 373, 58 Stat. 682, which is classified generally to chapter 6A (§ 201 et seq.) of Title 42, The Public Health and Welfare. For complete classification of this Act to the Code, see Short Title note set out under section 201 of Title 42 and Tables.

AMENDMENTS

2021—Subsec. (a)(4)(A), (B). Pub. L. 117–9 added subpars. (A) and (B) and struck out former subpars. (A) and (B) which read as follows:

''(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 355(b)(1), 355(b)(2), or 355(j) of this title or section 351(a) or 351(k) of the Public Health Service Act;

''(B) is submitted under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act;''.

2020—Subsec. (b)(5). Pub. L. 116–260 substituted ''September 30, 2024'' for ''December 18, 2020'' in introductory provisions and in subpar. (A) and substituted ''September 30, 2026'' for ''December 18, 2022'' in subpar. (B).

Pub. L. 116–215 substituted ''December 18, 2020'' for ''December 11, 2020'' in introductory provisions and in subpar. (A) and substituted ''December 18, 2022'' for ''December 11, 2022'' in subpar. (B).

Pub. L. 116–159 substituted ''December 11, 2020'' for ''September 30, 2020'' in introductory provisions and in subpar. (A) and substituted ''December 11, 2022'' for ''September 30, 2022'' in subpar. (B).

2016—Subsec. (a)(3)(A). Pub. L. 114–229, § 2(a)(1)(A), amended subpar. (A) generally. Prior to amendment, subpar. (A) read as follows: ''The disease primarily affects individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents.''

Subsec. (a)(4)(F). Pub. L. 114–229, § 2(a)(1)(B), substituted ''September 30, 2016'' for ''July 9, 2012''.

Subsec. (b)(4). Pub. L. 114–229, § 2(a)(2)(A), added par. (4) and struck out former par. (4). Prior to amendment, text read as follows:

''(A) 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 for the user fee to be assessed in accordance with this section.

''(B) 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 subparagraph (A) may transfer the voucher after such notification is provided, if such sponsor has not yet submitted the human drug application described in the notification.''

Subsec. (b)(5). Pub. L. 114–255 added par. (5) and struck out former par. (5). Prior to amendment, text read as follows: ''The Secretary may not award any priority review vouchers under paragraph (1) after December 31, 2016.''

Pub. L. 114–229, § 2(a)(2)(B), added par. (5) and struck out former par. (5). Prior to amendment, text read as follows: ''The Secretary may not award any priority review vouchers under paragraph (1) after September 30, 2016.''

Subsec. (g). Pub. L. 114–229, § 2(a)(3), inserted before period at end '', 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 chapter with respect to the drug for which the application is made.''

2015—Subsec. (b)(5). Pub. L. 114–113 substituted ''September 30, 2016.'' for ''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.''

Statutory Notes and Related Subsidiaries

CONSTRUCTION

Pub. L. 114–229, § 2(b), Sept. 30, 2016, 130 Stat. 944, provided that: ''Nothing in this Act [amending this section and enacting provisions set out as a note under section 301 of this title], or the amendments made by this Act, shall be construed to affect 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 [Sept. 30, 2016].''

§ 360ff–1. Targeted drugs for rare diseases

(a) Purpose

The purpose of this section, through the approach provided for in subsection (b), is to—

(1) facilitate the development, review, and approval of genetically targeted drugs and variant protein targeted drugs to address an unmet medical need in one or more patient subgroups, including subgroups of patients with different mutations of a gene, with respect to rare diseases or conditions that are serious or life-threatening; and

(2) maximize the use of scientific tools or methods, including surrogate endpoints and other biomarkers, for such purposes.

(b) Leveraging of data from previously approved drug application or applications

The Secretary may, consistent with applicable standards for approval under this chapter or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)], allow the sponsor of an application under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act for a genetically targeted drug or a variant protein targeted drug to rely upon data and information—

(1) previously developed by the same sponsor (or another sponsor that has provided the sponsor with a contractual right of reference to such data and information); and

(2) submitted by a sponsor described in paragraph (1) in support of one or more previously approved applications that were submitted under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act,

for a drug that incorporates or utilizes the same or similar genetically targeted technology as the drug or drugs that are the subject of an application or applications described in paragraph (2) or for a variant protein targeted drug that is the same or incorporates or utilizes the same variant protein targeted drug, as the drug or drugs that are the subject of an application or applications described in paragraph (2).

(c) Definitions

For purposes of this section—

(1) the term ''genetically targeted drug'' means a drug that—

(A) is the subject of an application under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] for the treatment of a rare disease or condition (as such term is defined in section 360bb of this title) that is serious or life-threatening;

(B) may result in the modulation (including suppression, up-regulation, or activation) of the function of a gene or its associated gene product; and

(C) incorporates or utilizes a genetically targeted technology;

(2) the term ''genetically targeted technology'' means a technology comprising non-replicating nucleic acid or analogous compounds with a common or similar chemistry that is intended to treat one or more patient subgroups, including subgroups of patients with different mutations of a gene, with the same disease or condition, including a disease or condition due to other variants in the same gene; and

(3) the term ''variant protein targeted drug'' means a drug that—

(A) is the subject of an application under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] for the treatment of a rare disease or condition (as such term is defined in section 360bb of this title) that is serious or life-threatening;

(B) modulates the function of a product of a mutated gene where such mutation is responsible in whole or in part for a given disease or condition; and

(C) is intended to treat one or more patient subgroups, including subgroups of patients with different mutations of a gene, with the same disease or condition.

(d) Rule of construction

Nothing in this section shall be construed to—

(1) alter the authority of the Secretary to approve drugs pursuant to this chapter or section 351 of the Public Health Service Act [42 U.S.C. 262] (as authorized prior to December 13, 2016), including the standards of evidence, and applicable conditions, for approval under such applicable chapter or Act; or

(2) confer any new rights, beyond those authorized under this chapter or the Public Health Service Act [42 U.S.C. 201 et seq.] prior to December 13, 2016, with respect to the permissibility of a sponsor referencing information contained in another application submitted under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)].

(June 25, 1938, ch. 675, § 529A, as added Pub. L. 114–255, div. A, title III, § 3012, Dec. 13, 2016, 130 Stat. 1091.)

Editorial Notes

REFERENCES IN TEXT

The Public Health Service Act, referred to in subsec. (d)(2), is act July 1, 1944, ch. 373, 58 Stat. 682, which is classified generally to chapter 6A (§ 201 et seq.) of Title 42, The Public Health and Welfare. For complete classification of this Act to the Code, see Short Title note set out under section 201 of Title 42 and Tables.

PART C—ELECTRONIC PRODUCT RADIATION CONTROL

Editorial Notes

CODIFICATION

This part was classified to subpart 3 (§ 263c et seq.) of part F of subchapter II of chapter 6A of Title 42, The Public Health and Welfare, prior to its renumbering by Pub. L. 101–629, § 19(a)(4), Nov. 28, 1990, 104 Stat. 4530, as amended by Pub. L. 103–80, § 4(a)(2), Aug. 13, 1993, 107 Stat. 779.

§ 360hh. Definitions

As used in this part—

(1) the term ''electronic product radiation'' means—

(A) any ionizing or non-ionizing electromagnetic or particulate radiation, or

(B) any sonic, infrasonic, or ultrasonic wave, which is emitted from an electronic product as the result of the operation of an electronic circuit in such product;

(2) the term ''electronic product'' means (A) any manufactured or assembled product which, when in operation, (i) contains or acts as part of an electronic circuit and (ii) emits (or in the absence of effective shielding or other controls would emit) electronic product radiation, or (B) any manufactured or assembled article which is intended for use as a component, part, or accessory of a product described in clause (A) and which when in operation emits (or in the absence of effective shielding or other controls would emit) such radiation;

(3) the term ''manufacturer'' means any person engaged in the business of manufacturing, assembling, or importing of electronic products;

(4) the term ''commerce'' means (A) commerce between any place in any State and any place outside thereof; and (B) commerce wholly within the District of Columbia; and

(5) the term ''State'' includes the District of Columbia, the Commonwealth of Puerto Rico, the Northern Mariana Islands, the Virgin Islands, Guam, and American Samoa.

(June 25, 1938, ch. 675, § 531, formerly act July 1, 1944, ch. 373, title III, § 531, formerly § 355, as added Pub. L. 90–602, § 2(3), Oct. 18, 1968, 82 Stat. 1174; amended Pub. L. 94–484, title IX, § 905(b)(1), Oct. 12, 1976, 90 Stat. 2325; renumbered § 531 and amended Pub. L. 101–629, § 19(a)(1)(B), (3), (4), Nov. 28, 1990, 104 Stat. 4529, 4530; Pub. L. 103–80, § 4(a)(2), Aug. 13, 1993, 107 Stat. 779.)

Editorial Notes

CODIFICATION

Section was classified to section 263c of Title 42, The Public Health and Welfare, prior to renumbering by Pub. L. 101–629.

AMENDMENTS

1993—Pub. L. 103–80 amended directory language of Pub. L. 101–629, § 19(a)(4), which renumbered section 263c of Title 42, The Public Health and Welfare, as this section.