Secretary [of Health and Human Services], in consultation with the Director of the Cybersecurity and Infrastructure Security Agency, shall review and, as appropriate and after soliciting and receiving feedback from device manufacturers, health care providers, third-party-device servicers, patient advocates, and other appropriate stakeholders, update the guidance entitled 'Content of Premarket Submissions for Management of Cybersecurity in Medical Devices' (or a successor document).''

[For definition of ''device'' as used in section 3305(e) of Pub. L. 117–328, set out above, see section 321(h) of this title, as made applicable by section 3305(h) of Pub. L. 117–328, which is set out below.]

RESOURCES REGARDING CYBERSECURITY OF DEVICES

Pub. L. 117–328, div. FF, title III, §3305(f), Dec. 29, 2022, 136 Stat. 5834, provided that: ''Not later than 180 days after the date of enactment of this Act [Dec. 29, 2022], and not less than annually thereafter, the Secretary [of Health and Human Services] shall update public information provided by the Food and Drug Administration, including on the website of the Food and Drug Administration, with information regarding improving cybersecurity of devices. Such information shall include information on identifying and addressing cyber vulnerabilities for health care providers, health systems, and device manufacturers, and how such entities may access support through the Cybersecurity and Infrastructure Security Agency and other Federal entities, including the Department of Health and Human Services, to improve the cybersecurity of devices.''

[For definition of ''device'' as used in section 3305(f) of Pub. L. 117–328, set out above, see section 321(h) of this title, as made applicable by section 3305(h) of Pub. L. 117–328, which is set out below.]

DEFINITION

Pub. L. 117–328, div. FF, title III, §3305(h), Dec. 29, 2022, 136 Stat. 5834, provided that: ''In this section [enacting this section, amending section 331 of this title, and enacting provisions set out as notes under this section and section 331 of this title], the term 'device' has the meaning given such term in section 201(h) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 321(h)).''

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.''

RARE DISEASE ENDPOINT ADVANCEMENT PILOT PROGRAM

Pub. L. 117–328, div. FF, title III, §3208, Dec. 29, 2022, 136 Stat. 5821, provided that:

''(a) IN GENERAL.—The Secretary [of Health and Human Services] shall establish a pilot program under which the Secretary establishes procedures to provide increased interaction with sponsors of rare disease drug development programs for purposes of advancing the development of efficacy endpoints, including surrogate and intermediate endpoints, for drugs intended to treat rare diseases, including through—

''(1) determining eligibility of participants for such program; and

''(2) developing and implementing a process for applying to, and participating in, such a program.

''(b) PUBLIC WORKSHOPS.—The Secretary shall conduct up to 3 public workshops, which shall be completed not later than September 30, 2026, to discuss topics relevant to the development of endpoints for rare diseases, which may include discussions about—

''(1) novel endpoints developed through the pilot program established under this section; and

''(2) as appropriate, the use of real world evidence and real world data to support the validation of efficacy endpoints, including surrogate and intermediate endpoints, for rare diseases.

''(c) REPORTS.—

''(1) INTERIM REPORT.—Not later than September 30, 2026, the Secretary shall submit to the Committee on Health, Education, Labor, and Pensions of the Senate and the Committee on Energy and Commerce of the House of Representatives a report describing the completed and ongoing activities in the pilot program established under this section and public workshops described in subsection (b).

''(2) FINAL REPORT.—Not later than September 30, 2027, the Secretary shall submit to the Committee on Health, Education, Labor, and Pensions of the Senate and the Committee on Energy and Commerce of the House of Representatives a report describing the outcomes of the pilot program established under this section.

''(d) GUIDANCE.—Not later than September 30, 2027, the Secretary shall issue guidance describing best practices and strategies for development of efficacy endpoints, including surrogate and intermediate endpoints, for rare diseases.

''(e) SUNSET.—The Secretary may not accept any new application or request to participate in the program established by this section on or after October 1, 2027.''

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)[1] 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

REFERENCES IN TEXT

Subsection (b), referred to in subsec. (a)(1), was redesignated as subsec. (c) of this section by Pub. L. 100–290, §2(b), Apr. 18, 1988, 102 Stat. 90.

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''.

[1] See References in Text note below.