

“(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), in-

ternal 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.