

## For Industry

# Orphan Drug Act

### CONGRESSIONAL FINDINGS FOR THE ORPHAN DRUG ACT

#### 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.

### RECOMMENDATIONS FOR INVESTIGATIONS OF DRUGS FOR RARE

## DISEASES OR CONDITIONS

### SEC. 525 [360aa].

(a) 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 nonclinical and clinical investigations which must be conducted with the drug before---

(1) it may be approved for such disease or condition under section 505,

(2) if the drug is an antibiotic, it may be certified for such disease or condition under section 507, or

(3) if the drug is a biological product, it may be licensed for such disease or condition under section 351 of the Public Health Service Act.

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 nonclinical 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 505, certification of such drug for such disease or condition under section 507, or licensing of such drug for such disease or condition under section 351 of the Public Health Service Act.

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

## DESIGNATION OF DRUGS FOR RARE DISEASES OR CONDITIONS

SEC. 526 [360bb]. (a)(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 505(b) for the drug, the submission of an application for certification of the drug under section 507, or the submission of an application for licensing of the drug under section 351 of the Public Health Service Act. 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 505,

(B) if a certification for such drug is issued under section 507, or

(C) if a license for such drug is issued under section 351 of the Public Health Service Act, 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) 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 505(b), a certificate was issued for the drug under section 507, or a license was issued for the drug under section 351 of the Public Health Service Act, 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 505(b), a certificate has not been issued for the drug under section 507, or a license has not been issued for the drug under section 351 of the Public Health Service Act and if preclinical investigations or investigations under section 505(i) 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 505(b), approval of an application for certification under section 507, or approval of a license under section 351 of the Public Health Service Act.

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

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

#### **PROTECTION FOR DRUGS FOR RARE DISEASES OR CONDITIONS**

SEC. 527 [360cc]. (a) Except as provided in subsection (b), if the Secretary---

(1) approves an application filed pursuant to section 505(b),

(2) issues a certification under section 507, or

(3) issues a license under section 351 of the Public Health Service Act for a drug designated under section 526 for a rare disease or condition, the Secretary may not approve another application under section 505(b), issue another certification under section 507, or issue another license under section 351 of the Public Health Service Act for such drug for such disease or condition for a person who is not the holder of such approved application, of such certification, or of such license until the expiration seven years from the date of the approval of the approved application, the issuance of the certification or the issuance of the license. Section

505(c)(2) does not apply to the refusal to approve an application under the preceding sentence.

(b) If an application filed pursuant to section 505(b) is approved for a drug designated under section 526 for a rare disease or condition, if a certification is issued under section 507 for such a drug or if a license is issued under section 351 of the Public Health Service Act for such a drug, the Secretary may, during the seven-year period beginning on the date of the application approval, of the issuance of the certification under section 507, or of the issuance of the license, approve another application under section 505(b), issue another certification under section 507, or, issue a license under section 351 of the Public Health Service Act, for such drug for such disease or condition for a person who is not the holder of such approved application, of such certification, or of such license if---

(1) the Secretary finds, after providing the holder notice and opportunity for the submission of views, that in such period the holder of the approved application, of the certification, or of the license cannot assure the availability of sufficient quantities of the drug to meet the needs of persons with the disease or condition for which the drug was designated; or

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

#### OPEN PROTOCOLS FOR INVESTIGATIONS OF DRUGS FOR RARE DISEASES OR CONDITIONS

SEC. 528 [360dd]. If a drug is designated under section 526 as a drug for a rare disease or condition and if notice of a claimed exemption under section 505(i) 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.

## GRANTS AND CONTRACTS FOR DEVELOPMENT OF DRUGS FOR RARE DISEASES AND CONDITIONS

SEC. 5. [360ee](a) 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 qualified clinical testing expenses incurred in connection with the development of drugs for rare diseases and conditions, (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) 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 505(i) of the Federal Food, Drug, and Cosmetic Act (or regulations issued under such section);

(ii) which occurs after the date such drug is designated under section 526 of such Act and before the date on which an application with respect to such drug is submitted under section 506(b) or 507 of such Act or under section 351 of the Public Health Service Act; and

(B) preclinical testing involving a drug is designated under section 526 of such Act and before the date on which an application with respect to such drug is submitted under section 505(b) or 507 of such Act or under section 351 of the Public Health Service Act.

(2) The term "rare disease or condition" means

(A) in the case of a drug, any disease or conditions 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,

(B) 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

(C) 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 526 of the Federal Food, Drug, and Cosmetic Act is made.

(D) 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) For grants and contracts under subsection (a) 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.

(d) STUDY.---The Secretary of Health and Human Services shall conduct a study to determine whether the application of subchapter B of chapter V of the Federal Food, Drug, and Cosmetic Act (relating to drugs for rare diseases and conditions) and section 28 of the Internal Revenue Code of 1986 (relating to tax credit) to medical devices or medical foods for rare diseases or conditions or to both is needed to encourage the development of such devices and foods. The Secretary shall report the results of the study to the Committee on Energy and Commerce of the House of Representatives and the Committee on Labor and Human Resources of the Senate not later than one year after the date of the enactment of this Act. For purposes of this section, the term "rare diseases or conditions" has the meaning prescribed by section 5 of the

Orphan Drug Act (21 U.S.C. 360ee).

## ORPHAN PRODUCTS BOARD

SEC. 227 [236]. (a) There is established in the Department of Health and Human Services a board for the development of drugs (including Biologics) and devices (including diagnostic products) for rare diseases or conditions to be known as the Orphan Products Board. The Board shall be comprised of the Assistant Secretary for Health of the Department of Health and Human Services and representatives, selected by the Secretary, of the Food and Drug Administration, the National Institutes of Health, the Centers for Disease Control and, any other Federal department or agency which the Secretary determines has activities relating to drugs and devices for rare diseases or conditions. The Assistant Secretary for Health shall chair the Board.

(b) The function of the Board shall be to promote the development of drugs and devices for rare diseases or conditions and the coordination among Federal, other public, and private agencies in carrying out their respective functions relating to the development of such articles for such diseases or conditions.

(c) In the case of drugs for rare diseases or conditions the Board shall---

(1) evaluate---

(A) the effect of subchapter B of the Federal Food, Drug, and Cosmetic Act on the development of such drugs, and

(B) the implementation of such subchapter;

(2) evaluate the activities of the National Institutes of Health and the Alcohol, Drug Abuse, and Mental Health Administration for the development of drugs for such diseases or conditions,

(3) assure appropriate coordination among the Food and Drug Administration, the National Institutes of Health, the Alcohol, Drug

Abuse, and Mental Health Administration, and the Centers for Disease Control in the carrying out of their respective functions relating to the development of drugs for such diseases or conditions to assure that the activities of each agency are complementary,

(4) assure appropriate coordination among all interested Federal agencies, manufacturers, and organizations representing patients, in their activities relating to such drugs,

(5) with the consent of the sponsor of a drug for a rare disease or condition exempt under section 505(i) of the Federal Food, Drug, and Cosmetic Act or regulations issued under such section, inform physicians and the public respecting the availability of such drug for such disease or condition and inform physicians and the public respecting the availability of drugs approved under section 505(c) of such Act or licensed under section 351 of this Act for rare diseases or conditions,

(6) seek business entities and others to undertake the sponsorship of drugs for rare diseases or conditions, seek investigators to facilitate the development of such drugs, and seek business entities to participate in the distribution of such drugs, and

(7) reorganize the efforts of public and private entities and individuals in seeking the development of drugs for rare diseases or conditions and in developing such drugs.

(d) The Board shall consult with interested persons respecting the activities of the Board under this section and as part of such consultation shall provide the opportunity for the submission of oral views.

(e) The Board shall submit to the Committee on Labor and Human Resources of the Senate and the Committee on Energy and Commerce of the House of Representatives an annual report---

(1) identifying the drugs which have been designated under section 526 of the Federal Food, Drug, and Cosmetic Act for a rare disease or condition,

(2) describing the activities of the Board, and

(3) containing the results of the evaluations carried out by the Board.

The Director of the National Institutes of Health and the Administrator of the Alcohol, Drug Abuse, and Mental Health Administration shall submit to the Board for inclusion in the annual report a report on the rare disease and condition research activities of the Institutes of the National Institutes of Health and the Alcohol, Drug Abuse, and Mental Health Administration; the Secretary of the Treasury shall submit to the Board for inclusion in the annual report a report on the use of the credit against tax provided by section 44H of the Internal Revenue Code of 1954; and the Secretary of Health and Human Services shall submit to the Board for inclusion in the annual report a report on the program of assistance under section 5 of the Orphan Drug Act for the development of drugs for rare diseases and conditions. Each annual report shall be submitted by June 1 of each year for the preceding calendar year.