101ST CONGRESS
*2d Session*
HOUSE OF REPRESENTATIVES
REPORT
101-635

# ORPHAN DRUG AMENDMENTS OF 1990

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JULY 27, 1990.—Committed to the Committee of the Whole House on the State of the Union and ordered to be printed

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

# REPORT

[To accompany H.R. 4638]

[Including cost estimate of the Congressional Budget Office]

The Committee on Energy and Commerce, to whom was referred the bill (H.R. 4638) to revise the orphan drug provisions of the Federal Food, Drug, and Cosmetic Act and the Orphan Drug Act, and for other purposes, having considered the same, report favorably thereon with an amendment and recommend that the bill as amended do pass.

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The amendment is as follows:

39-006

Strike out all after the enacting clause and insert in lieu thereof the following:

SECTION 1. SHORT TITLE; REFERENCE.

(a) SHORT TITLE.—This Act may be cited as the "Orphan Drug Amendments of 1990".

(b) REFERENCE.—Whenever in this Act (other than sections 4 and 5) an amendment or repeal is expressed in terms of an amendment to, or repeal of, a section or other provision, the reference shall be considered to be made to a section or other provision of the Federal Food, Drug, and Costmetic Act.

SEC. 2. DESIGNATIONS.

(a) IN GENERAL.—Section 526(a)(2) (21 U.S.C. 360bb(a)(2)) is amended by inserting before the period at the end a comma and the following: "and on the basis of projections as to the number of persons who will be affected by the disease or condition 3 years from the date the request for designation of the drug is made under paragraph (1)".

(b) EXCLUSIVITY.—Section 527(b) (21 U.S.C. 360cc(b)) is amended by striking out "or" at the end of paragraph (1), by striking out the period at the end of paragraph (2) and inserting in lieu thereof a semicolon, and by adding at the end the following:

"(3) if a drug has been designated under section 526 for a rare disease or condition described in section 526(a)(2)(A) and if after such designation such disease or condition does not meet such description; or".

SEC. 3. SIMULTANEOUS DEVELOPMENT.

(a) IN GENERAL.—Section 527(b) (21 U.S.C. 360cc(b)), as amended by section 2(b), is amended by inserting "(1)" after "(b)", by redesignating paragraphs (1), (2), and (3) as subparagraphs (A), (B), and (C), respectively, by striking out "for a person who is not" and by inserting in lieu there of "for an applicant who is not", and by adding at the end the following:

"(D) the Secretary finds, after providing the holder, such applicant, and other interested persons an opportunity to present their views, that the drugs of the holder and such applicant were developed simultaneously.

The Secretary shall make a decision on a request for a finding under subparagraph (D) not later than 60 days after the filing of the request.

"(2) For purposes of paragraph (1)(D), drugs of a holder and an applicant shall be considered to be developed simultaneously only if—

"(A) the applicant requested that its drug be designated under section 526 no later than 6 months after publication of the designation under section 526(c) of the holder's drug,

"(B) the applicant initiated the human clinical trials that the applicant relied on in its application for such approval, certification, or license not more than 12 months after the date the holder initiated the human clinical trials that the holder relied on in its application for such approval, certification, or license, and

"(C) the applicant submitted such application, including the reports of the clinical and animal studies necessary for approval, certification, or licensing, not more than 12 months after the holder submitted its application, including such reports, for such action.

"(3)(A) Paragraph (1)(D) does not apply to a drug—

"(i) approved under section 505, certified under section 507, or licensed under section 351 of the Public Health Service Act before August 15, 1990,

"(ii) for which an application under section 505 or 507 or such section 351 was submitted before August 15, 1990, or

"(iii) for which an exemption under section 505(i) or 507(d) was in effect before August 15, 1990, for which human clinical trials were actively being conducted before such date, and for which an application for designation under section 526 was submitted on or before July 16, 1990.

"(B) A drug designated under section 526 before the date of the enactment of the Orphan Drug Amendments of 1990 shall be considered to have been developed simultaneously under paragraph (1)(D) if it meets the requirements of subparagraphs (B) and (C) of paragraph (2).".

(b) PUBLICATION.—Section 526(c) (21 U.S.C. 360bb(c)) is amended—

"(1) by inserting "for a rare disease or condition" after "(a)", and

"(2) by striking out "shall be made available to the public" and inserting in lieu thereof "shall be promptly published in the Federal Register and otherwise

made available to the public in a manner designed to notify persons who have such disease or condition".

(c) STUDY.—

(1) The Secretary of Health and Human Services shall, subject to paragraph (2), enter into a contract with a public or nonprofit private entity to conduct a study of the effect on the development of drugs for rare diseases or conditions (as defined in section 526(a) of the Federal Food, Drug, and Cosmetic Act) of the amendments made by subsections (a) and (b).

(2) Upon the expiration of 2 years from the date of the enactment of this Act, the Secretary shall request the Institute of Medicine of the National Academy of Sciences to enter into the contract under paragraph (1) for conducting the study described in such paragraph. If such Institute declines to conduct the study, the Secretary shall conduct the study prescribed by paragraph (1) through another public or nonprofit entity.

(3) The Secretary shall ensure that the study required in paragraph (1) is completed after sufficient data necessary for the study are available.

SEC. 4. OFFICE FOR ORPHAN DISEASES AND CONDITIONS.

Section 227 of the Public Health Service Act (42 U.S.C. 236)—

(1) by amending subsection (a) to read as follows:

"(a) There is established under the Assistant Secretary for Health an Office for Orphan Diseases and Conditions.",

(2) by striking out "Board" each place it appears and inserting in lieu thereof "Office",

(3) by striking out "drugs and devices" in subsection (b) and inserting in lieu thereof "drugs, devices, and medical foods",

(4) by inserting "of chapter V" after "subchapter B" in subsection (c)(1)(A), and

(5) by redesignating subsection (e) as subsection (f) and by inserting after subsection (d) the following:

"(e) There is established an advisory committee to advise the Office in carrying out its functions under this section. The advisory committee shall be appointed by the Secretary, in consultation with the Office and the Commissioner of the Food and Drug Administration, from persons knowledgable about rare diseases and conditions, including 5 representatives of organizations of persons with rare diseases or conditions, 3 research scientists, and 3 representatives of health-related companies. The Secretary shall also appoint as liaisons to the committee individuals from the Food and Drug Administration, the National Institutes of Health, and other appropriate Federal agencies.".

SEC. 5. AUTHORIZATION FOR ORPHAN DRUG ACT.

Section 5(c) of the Orphan Drug Act (21 U.S.C. 360ee(c) is amended by inserting before the period at the end a comma and the following: "$20,000,000 for fiscal year 1991, $25,000,000 for fiscal year 1992, and $30,000,000 for fiscal year 1993".

## PURPOSE AND SUMMARY

The purpose of this legislation is to stengthen the Orphan Drug Act and to reauthorize the orphan drug grant program. Enacted in 1983, the Act creates incentives for research on and development of drugs for the treatment of rare diseases. The incentives are grants, tax credits and market exclusivity. By any measure, the Act has been a tremendous success. Pursuant to its requirements, the Food and Drug Administration ("FDA") has granted 375 orphan designations and it has approved 41 orphan drugs. However, hearings held by the Subcommittee on Health and the Environment identified a number of problems which are addressed by H.R. 4638.

First, under the Act, a rare disease is defined as a disease that affects fewer that 200,000 people. This standard may be met at the time the drug is designated, which can be many years before the drug is actually marketed. In the case of a disease such as Acquired Immune Deficiency Syndrome ("AIDS"), which has a rapidly growing patient population, a drug might qualify for orphan status at the time of designation, even though growth in patient

population is predictable and, at the time it is first sold, far more than 200,000 people have the disease. Section 2 of the bill would address this problem by requiring that the FDA project three years into the future when deciding whether a disease or condition qualifies as affecting fewer than 200,000 people. It would also mandate that a drug would lose its orphan drug status if the population ever goes above 200,000.

Second, in at least three instances, drugs have received the seven years of exclusivity even though in each case one or more other companies had been developing the product and were racing to get their product on the market. Under section 3 of the bill, two or more companies can both get on the market if they developed the drug simultaneously, which is defined to mean that they initiated clinical testing and filed their license applications within one year of each other.

In addition, this legislation would create an Office of Orphan Diseases and Conditions within the Office of the Assistant Secretary of Health, and it would authorize the orphan drug grant program for 1990, 1991, and 1992.

## Background and Need for the Legislation

The Orphan Drug Act of 1983 creates incentives for the development of drugs that are designed to treat rare diseases that occur so infrequently that additional incentives are necessary to stimulate the investment needed to bring the drugs to market. Drugs that qualify as orphan drugs are eligible for grants, tax credits and seven years of market exclusivity. Under current law, these benefits are available to drugs for any disease or condition that affects fewer than 200,000 people.

The authorization for the grant program expires at the end of 1990, and the tax credits will terminate at that time as well. (A separate bill, H.R. 4639, would extend the tax credit program.)

In at least three instances, drugs of strong commercial value have qualified for orphan status and therefore have received seven years of market exclusivity. Those drugs are:

*EPO,* approved for treatment of anemia associated with chronic renal failure, including patients on kidney dialysis. The drug costs about $8,000 per year. In 1990, EPO will have $200 million in annual sales, most of which will be purchased by the federal government through the medicare program.

*Aerosol Pentamidine,* approved for treatment and prevention of a type of pneumonia that is the most common complication of AIDS. Aerosol Pentamidine costs about $1,000 per year and has substantial annual sales.

*Human Growth Hormone,* approved for treatment of children who have growth failure due to the absence of adequate human growth hormone. HGH costs between $10,000 and $30,000 per year for each patient and has annual sales of between $125 and $150 million.

In each of these cases, two or more companies conducted clinical trials and submitted a new drug application, but only the first com-

pany to receive an approval from the FDA was permitted to market the drug.

The legislation is designed to retain incentives to develop drugs for rare diseases, but to provide for limited competition where a drug is so profitable that two or more companies are in a true race to market it. It would also require the FDA to look into the future when deciding whether more than 200,000 people have a particular disease or condition.

## Hearings

On February 7, 1990, the Committee's Subcommittee on Health and the Environment held a one-day hearing on the reauthorization of the grant program and on how well the Orphan Drug Act has been working in general. Testimony was received from 12 witnesses, with additional material submitted by 11 individuals and organizations. The witnesses included the Acting Commissioner of the Food and Drug Administration, representatives of national organizations representing individuals with diseases that may be treated by drugs subject to the Act, and representatives of companies that would be affected by proposed changes in the law.

## Committee Consideration

On June 21, 1990, the Subcommittee on Health and the Environment met in open session and ordered reported H.R. 4638, as amended, by a vote of 6–4, a quorum being present. On July 17, 1990, the Committee met in open session and ordered reported H.R. 4638 with one amendment, by voice vote, a quorum being present.

## Committee Oversight Findings

Pursuant to rule XI of the Rules of the House of Representatives, the Subcommittee on Health and the Environment held oversight hearings and made findings that are reflected in the legislative report.

## Committee on Government Operations

No oversight findings have been submitted to the Committee by the Committee on Government Operations

## Committee Cost Estimate

In compliance with clause 7(a) of rule XIII of the Rules of the House of Representatives, the Committee believes that the cost incurred in carrying out H.R. 4638 would be $20 million for 1991, $25 million for 1992 and $30 million for 1993.

## Congressional Budget Office Estimate

U.S. Congress,
Congressional Budget Office,
*Washington, DC, July 26, 1990.*

Hon. John D. Dingell,
*Chairman, Committee on Energy and Commerce,*
*House of Representatives, Washington, DC.*

Dear Mr. Chairman: The Congressional Budget Office has prepared the attached cost estimate for H.R. 4638, the Orphan Drug Amendments of 1990, as ordered reported by the House Committee on Energy and Commerce on July 17, 1990.

If you wish further details on this estimate, we will be pleased to provide them.

Sincerely,

Robert D. Reischauer.

## Congressional Budget Office Cost Estimate

1. Bill number: H.R. 4638.
2. Bill title: Orphan Drug Amendments of 1990.
3. Bill status: As ordered reported by the House Committee on Energy and Commerce on July 17, 1990.
4. Bill purpose: To revise the orphan drug provisions of the Federal Food, Drug, and Cosmetic Act and the Orphan Drug Act, and for other purposes.
5. Estimated cost to the Federal Government:

[By fiscal years, in millions of dollars]

| | 1991 | 1992 | 1993 | 1994 | 1995 |
|---|---|---|---|---|---|
| Estimated authorization levels: | | | | | |
| Orphan drug grants | 20 | 25 | 30 | ... | ... |
| Study | ... | ... | (1) | ... | ... |
| Total estimated authorization levels | 20 | 25 | 30 | ... | ... |
| Total estimated outlays | 17 | 23 | 29 | 5 | 2 |

[1] Less than $500,000.

The costs of this bill fall within budget function 550.

Basis of estimate: H.R. 4638 would reauthorize federal funds to assist in defraying the costs of developing drugs, medical devices, and medical foods for rare diseases or conditions. The bill specifies authorization levels for this program.

In addition, the bill would require the Secretary of Health and Human Services (HHS) to study, two years after enactment, the effect of issuing licenses to more than one manufacturer for the production of a rare disease drug if the Secretary of HHS decides that more than one manufacturer simultaneously developed the drug. CBO estimates that the study would cost the federal government less than $500,000 in fiscal year 1993.

This estimate assumes that all authorizations are fully appropriated at the beginning of each fiscal year. Outlays are estimated using spendout rates computed by CBO on the basis of recent program data.

6. Estimated cost to State and local government: None.
7. Estimate comparison: None.
8. Previous CBO estimate: None.
9. Estimated prepared by: Karen Graham.
10. Estimate approved by: C.G. Nuckols for James L. Blum, Assistant Director for Budget Analysis.

## Inflationary Impact Statement

Pursuant to clause 2(l)(4) of rule XI of the Rules of the House of Representatives, the Committee makes the following statement with regard to the inflationary impact of the reported bill:

> H.R. 4638 is likely to have a favorable impact on inflation. It should result in increased competition for drugs that qualify under the standards in section 2 or section 3.

## Section-by-Section Analysis

### SECTION 1: SHORT TITLE

The short title of the bill is the Orphan Drug Amendments of 1990.

### SECTION 2: DESIGNATIONS

Under current law, the applicant for orphan drug status must show that, at the time of designation, "the disease or condition" for which the drug is marketed "affects less than 200,000 persons in the United States." Section 526(a)(2) of the Federal Food, Drug and Cosmetic Act ("FDC Act" or "the Act"), 21 U.S.C. 360bb(a)(2). In the case of a disease with a growing population (such as AIDS), this means that a drug can qualify for orphan drug status even though it is expected that in the near future more than 200,000 people will be affected.

Section 2 requires that the determination as to whether 200,000 people are affected by a disease or condition be made on the basis of projections as to the number of people who will be affected three years from the date that the designation is requested. Thus, the legislation would require the applicant for an orphan drug designation to carry the burden of proving that fewer than 200,000 persons have the disease or condition both at the time the request for a designation is made and during the three subsequent years.

Section 527 of the FDC Act also requires the Secretary to permit additional drugs on the market if certain, specified conditions are met. The legislation would amend section 527 to require the Secretary to lift the prohibition on marketing imposed by the Orphan Drug Act's exclusivity provision if at any time after designation an applicant demonstrates that the patient population exceeds 200,000.

However, this legislation does not alter the statutory definition of rare disease or condition (which is a disease that "affects less than 200,000 persons in the United States") nor does it alter the Secretary's authority to interpret that provision.

SECTION 3: SIMULTANEOUS DEVELOPMENT

Current law provides that an unlimited number of companies may obtain an orphan drug designation, which markes them eligible for the grant program (to fund research on orphan drugs) and for seven years of market exclusivity. An unlimited number of companies may also apply for approval of an orphan drug, but only one (the first to obtain approval) is given the seven years of market exclusivity. The remaining sponsors must wait until the end of the seven-year period to market their products.

Section 3 provides that if one or more companies demonstrates that it researched and developed its drug simultaneously with the holder of the exclusive right to market a drug under the Act, that company may also obtain permission to market the drug within the seven-year period. In order to qualify under this provision, an applicant must demonstrate that:

(1) the applicant requested that its drug be designated within 6 months of the time that the holder received its designation;

(2) the applicant initiated the human clinical trials that it relied on in its application no more than 12 months after the date the holder initiated its human clinical trials; and

(3) the applicant submitted its application, including reports of clinical and animal studies necessary for approval, less than one year after the holder submitted its application, including the same reports.

The legislation would not allow a company to qualify under the third part of the test by submitting a sham application. In order to qualify, the application must contain the data and reports of animal and human studies that are necessary for approval, certification or a license. However, the submission of supplemental information such as reanalysis of completed studies, safety updates, or even an additional clinical study that was not essential for approval, would not disqualify an applicant from the provision.

Section 3 would not apply to any drug that is approved prior to August 15, 1990, for which an application for approval is submitted prior to that date, or which is both in human clinical trials prior to August 15, 1990 and had a request for designation submitted by July 16, 1990.

As used in this section, the term "human clinical trials" includes studies of a drug in humans used to establish the safety and effectiveness of the drug for the indication that is designated under the Orphan Drug Act. These trials would be conducted pursuant to an investigational new drug application in the United States or similar application in a foreign country. The term includes studies directed solely at establishing a safe dose of the drug, so long as the study was performed in connection with the indication for the orphan disease or condition. It would not include a compassionate use of the drug or the use of a drug outside the label indications unless that use was associated with an organized clinical study of the drug.

Section 3 also requires that the Secretary initiate a study of the impact of the simultaneous development provision on the development of drugs for rare diseases. The Secretary would have to initiate the study within two years, giving the Institute of Medicine of

the National Academy of Sciences the option of undertaking the study. The study would be completed as soon as sufficient information for the study is available.

SECTION 4: OFFICE FOR ORPHAN AND RARE DISEASES AND CONDITIONS

This section would eliminate the Orphan Products Board and substitute an Office for Orphan Diseases and Conditions within the Office of the Assistant Secretary of Health. The Office would coordinate the activities within the federal government concerning the development of drugs, devices and medical foods for persons with orphan diseases and conditions. It would also promote research on drugs for the treatment of rare diseases, sponsor educational activities, serve as a link between government and private activities related to orphan drugs, and provide information to Congress as requested. The Office would be assisted by an advisory committee comprised of representatives of organizations of persons with rare diseases and conditions, research scientists and representatives of health-related companies.

SECTION 5: AUTHORIZATION FOR ORPHAN DRUG ACT

Section 5 provides for an authorization for the grant program for 1991, 1992 and 1993. The program is currently authorized at $14 million. The bill provides for an authorization of $20 million for FY 1991, $25 million for FY 1992, and $30 million for FY 1993.

## Agency Views

Secretary of Health and Human Services,
*Washington, DC, June 20, 1990.*

Hon. Henry A. Waxman,
*Chairman, Subcommittee on Health and the Environment, Committee on Energy and Commerce, House of Representatives, Washington, DC.*

Dear Mr. Chairman: I take this opportunity to inform you of the views of the Administration on H.R. 4638, a bill entitled the "Orphan Drug Amendments of 1990" which is pending before your Subcommittee.

Earlier this year, James Benson, Acting Commissioner of Food and Drugs, in testimony before your Subcommittee, indicated that the Food and Drug Administration (FDA) is generally very pleased with the current Orphan Drug Program. Thus far, 49 new treatments for rare diseases have been approved under this program, and a total of 370 orphan product designations have been granted. These therapies have been and will continue to be instrumental in helping to save lives and alleviate the agony of those unfortunate enough to suffer from such rare diseases as Wilson's disease, AIDS-related illnesses, rare forms of cancer, and other diseases.

The incentives created under the Orphan Drug Act (the Act), including research grants, tax credits for research and development, and exclusive marketing rights, make it financially feasible for companies to make the large investment in capital necessary to develop new treatments for rare diseases. These incentives may be

particularly important for companies developing new biotechnology products.

Another critical issue, in addition to the concerns of this Department that the effectiveness of the Act in spurring development of orphan drugs is not compromised, is the effect of the Act on the biotechnology marketplace. The Vice President, in his role as Chairman of the Administration's Council on Competitiveness, directed the Council's Working Group on Biotechnology to review the Act and your proposed amendments with respect to their impact on the competitiveness of our Nation's biotechnology industry.

Despite concerns about anomalies in the operation of the Act, we have concluded that the Act should not be altered in any fundamental manner. We welcome any suggestions for improving the Act to meet fiscal and economic concerns without jeopardizing the integrity and effectiveness of the Act. We do not believe that current legislative proposals will prove successful at improving the Act's administration or effectiveness.

We believe that FDA has done an excellent job in implementing the Act, and should be encouraged to continue to refine its efforts to limit the special incentives of this law to the development of true orphan treatments.

In conclusion, we believe that the Orphan Drug Act has been a success in stimulating the development of drugs for limited patient populations, as was Congress' intent. We do recognize that questions have been raised about the appropriateness of a few specific drugs receiving the benefits under the Act. However, we have not identified a solution to these problems that does not jeopardize the stimulus provided by the Act for research on and development of orphan drugs. For these reasons, we do not support the provisions of H.R. 4638.

The Office of Management and Budget has advised that there is no objection to the presentation of these views from the standpoint of the Administration's programs.

Sincerely,

LOUIS W. SULLIVAN, M.D.,
*Secretary.*

## CHANGES IN EXISTING LAW MADE BY THE BILL, AS REPORTED

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

### FEDERAL FOOD, DRUG, AND COSMETIC ACT

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### CHAPTER V—DRUGS AND DEVICES

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**Subchapter B—Drugs for Rare Diseases or Conditions**

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DESIGNATION OF DRUGS FOR RARE DISEASES OR CONDITIONS

SEC. 526. (a)(1) * * *

(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 the subsection is made, *and on the basis of projections as to the number of persons who will be affected by the disease or condition 3 years from the date the request for designation of the drug is made under paragraph (1).*

* * * * * * *

(c) Notice respecting the designation of a drug under subsection (a) *for a rare disease or condition* [shall be made available to the public] *shall be promptly published in the Federal Register and otherwise made available to the public in a manner designed to notify persons who have such disease or condition.*

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PROTECTION FOR DRUGS FOR RARE DISEASE OR CONDITIONS

SEC. 527. (a) * * *

(b) *(1)* If an application filed pursuant to section 505 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, 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] *for an applicant who is not* the holder of such approved application, of such certification, or of such license if—

[(1)] *(A)* 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)] *(B)* 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[.]*;*

*(C) if a drug has been designated under section 526 for a rare disease or condition described in section 526(a)(2)(A) and if after*

*such designation such disease or condition does not meet such description; or*

*(D) the Secretary finds, after providing the holder, such applicant, and other interested persons an opportunity to present their views, that the drugs of the holder and such applicant were developed simultaneously.*

*The Secretary shall make a decision on a request for a finding under subparagraph (D) not later than 60 days after the filing of the request.*

*(2) For purposes of paragraph (1)(D), drugs of a holder and an applicant shall be considered to be developed simultaneously only if—*

*(A) the applicant requested that its drug be designated under section 526 no later than 6 months after publication of the designation under section 526(c) of the holder's drug,*

*(B) the applicant initiated the human clinical trials that the applicant relied on in its application for such approval, certification, or license not more than 12 months after the date the holder initiated the human clinical trials that the holder relied on in its application for such approval, certification, or license, and*

*(C) the applicant submitted such application, including the reports of the clinical and animal studies necessary for approval, certification, or licensing, not more than 12 months after the holder submitted its application, including such reports, for such action.*

*(3)(A) Paragraph (1)(D) does not apply to a drug—*

*(i) approved under section 505, certified under section 507, or licensed under section 351 of the Public Health Service Act before August 15, 1990,*

*(ii) for which an application under section 505 or 507 of such section 351 was submitted before August 15, 1990, or*

*(iii) for which an exemption under section 505(i) or 507(d) was in effect before August 15, 1990, for which human clinical trials were actively being conducted before such date, and for which an application for designation under section 526 was submitted on or before July 16, 1990.*

*(B) A drug designated under section 526 before the date of the enactment of the Orphan Drug Amendments of 1990 shall be considered to have been developed simultaneously under paragraph (1)(D) if it meets the requirements of subparagraphs (B) and (C) of paragraph (2).*

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SECTION 227 OF THE PUBLIC HEALTH SERVICE ACT

ORPHAN PRODUCTS BOARD

SEC. 227. [(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 representa-

tives, 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.] *(a) There is established under the Assistant Secretary for Health an Office for Orphan Diseases and Conditions.*

(b) The function of the [Board] *Office* shall be to promote the development of [drugs and devices] *drugs, devices, and medical foods* 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, such diseases or conditions.

(c) In the case of drugs for rare diseases or conditions the [Board] *Office* shall—

(1) evaluate—

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

(B) the implementation of such subchapter;

* * * * * * *

(d) The [Board] *Office* 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) There is established an advisory committee to advise the Office in carrying out its functions under this section. The advisory committee shall be appointed by the Secretary, in consultation with the Office and the Commissioner of the Food and Drug Administration, from persons knowledgable about rare diseases and conditions, including 5 representatives of organizations of persons with rare diseases or conditions, 3 research scientists, and 3 representatives of health-related companies. The Secretary shall also appoint as liaisons to the committee individuals from the Food and Drug Administration, the National Institutes of Health, and other appropriate Federal agencies.*

[(e)] *(f)* The [Board] *Office* 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] *Office.*

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] *Office* 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] *Office* 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] *Office* 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 calender year.

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## Section 5 of the Orphan Drug Act

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

Sec. 5. (a) * * *

* * * * * * *

(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, *$20,000,000 for fiscal year 1991, $25,000,000 for fiscal year 1992, and $30,000,000 for fiscal year 1993.*

○