

103<sup>D</sup> CONGRESS  
2<sup>D</sup> SESSION

# H. R. 4160

To amend the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act, and the Orphan Drug Act to revise the provisions of such Acts relating to orphan drugs.

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## IN THE HOUSE OF REPRESENTATIVES

MARCH 24, 1994

Mr. WAXMAN (for himself and Mr. STUDDS) introduced the following bill;  
which was referred to the Committee on Energy and Commerce

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## A BILL

To amend the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act, and the Orphan Drug Act to revise the provisions of such Acts relating to orphan drugs.

1 *Be it enacted by the Senate and House of Representa-*  
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE AND REFERENCE.**

4 (a) SHORT TITLE.—This Act may be cited as the  
5 “Orphan Drug Act Amendments of 1994 ”.

6 (b) REFERENCE.—Whenever in this Act (other than  
7 sections 5 and 6) an amendment or repeal is expressed  
8 in terms of an amendment to, or repeal of, a section or

1 other provision, the reference shall be considered to be  
2 made to a section or other provision of the Federal Food,  
3 Drug, and Cosmetic Act.

4 **SEC. 2. PERIOD OF EXCLUSIVITY.**

5 (a) INITIAL PERIOD.—Subsection (a) of section 527  
6 (21 U.S.C. 360cc) is amended—

7 (1) by inserting “(1)” after “(a)”,

8 (2) by redesignating paragraphs (1), (2), and

9 (3) as subparagraphs (A), (B), and (C), respectively,

10 (3) by striking “seven years” and inserting “4  
11 years”, and

12 (4) by striking “505(c)(2)” and inserting  
13 “505(c)(1)(B)”.

14 (b) ADDITIONAL PERIOD.—Subsection (a) of such  
15 section 527 (21 U.S.C. 360cc) (as amended by subsection  
16 (a)) is amended by adding at the end the following:

17 “(2) The holder of the approved application, certifi-  
18 cation, or license of a drug to which the 4-year period of  
19 exclusivity applies under paragraph (1) may, after the ex-  
20 piration of 3½ years of such period but not later than  
21 90 days before the expiration of such period, apply to the  
22 Secretary for a 3-year extension of such period. Such an  
23 application shall contain such information as the Secretary  
24 determines is necessary to evaluate such application.

1 “(3) The Secretary shall approve an application sub-  
2 mitted under paragraph (2) if the applicant—

3 “(A) demonstrates that the drug has a limited  
4 commercial potential as determined under regula-  
5 tions of the Secretary, taking into account sales in-  
6 formation respecting such drug and any other factor  
7 identified by the Secretary in such regulations which  
8 is relevant to the commercial potential of such drug,  
9 and

10 “(B) makes such demonstration on the basis of  
11 the regulations of the Secretary referred to in sub-  
12 paragraph (A) which were in effect—

13 “(i) on the date—

14 “(I) such drug received its designation  
15 under section 526(a), or

16 “(II) such applicant applied for an ex-  
17 emption for such drug under section 505(i)  
18 or 507(d),

19 whichever first occurs, or

20 “(ii) if the date under clause (i) occurred  
21 before the date such regulations were in effect,  
22 or the date such regulations were in effect.”.

23 (c) CONFORMING AMENDMENT.—Section 527(b) (21  
24 U.S.C. 360cc(b)) is amended—

1 (1) by striking “during the seven-year period  
2 beginning on the date of the application approval”  
3 and inserting “during the applicable period of exclu-  
4 sivity under subsection (a)”, and

5 (2) by striking “such seven year period” and in-  
6 sserting “the applicable period of exclusivity under  
7 subsection (a)”.

8 (d) EFFECTIVE DATE.—The amendments made by  
9 subsections (a) and (b) shall not apply to a drug—

10 (1) for which an application under section 505  
11 or 507 of the Federal Food, Drug, and Cosmetic Act  
12 or section 351 of the Public Health Service Act was  
13 submitted before March 1, 1994, or

14 (2) for which an exemption under section 505(i)  
15 or 507(d) of the Federal Food, Drug, and Cosmetic  
16 Act was in effect before March 1, 1994, for which  
17 human clinical trials were actively being conducted  
18 before such date, and for which an application for  
19 designation under section 526 of such Act was sub-  
20 mitted before the date of enactment of the Orphan  
21 Drug Act Amendments of 1994.

22 The 7 year period of exclusivity provided by section 527(a)  
23 of the Federal Food, Drug, and Cosmetic Act before the  
24 date of the enactment of this Act shall, after such date,  
25 apply to a drug described in paragraph (1) or (2).

1 (e) REGULATIONS.—The Secretary shall issue final  
2 regulations to implement paragraphs (2) and (3) of sec-  
3 tion 527(a) of the Federal Food, Drug, and Cosmetic Act  
4 (21 U.S.C. 360cc) (as amended by subsection (b)) not  
5 later than 6 months after the date of the enactment of  
6 this Act.

7 **SEC. 3. DESIGNATIONS.**

8 (a) IN GENERAL.—Section 526(a)(2) (21 U.S.C.  
9 360bb(a)(2)) is amended to read as follows:

10 “(2) For purposes of paragraph (1), the term ‘rare  
11 disease or condition’ means any disease or condition  
12 which—

13 “(A) affects fewer than 200,000 persons in the  
14 United States determined on the basis of—

15 “(i) the facts and circumstances as of the  
16 date the request for designation of the drug  
17 under this subsection is made, and

18 “(ii) projections as to the number of per-  
19 sons who will be affected by the disease or con-  
20 dition on a date which is 3 years from date  
21 such request was made, or

22 “(B) affects more than 200,000 persons in the  
23 United States and for which there is no reasonable  
24 expectation that the cost of developing and making  
25 available in the United States a drug for such dis-

1 ease or condition will be recovered from sales in the  
2 United States of such drug.”.

3 (b) EXCLUSIVITY.—Section 527(b) (21 U.S.C.  
4 360cc(b)) is amended—

5 (1) in paragraph (1), by striking “The” and in-  
6 serting “the” and by striking “or” at the end of  
7 such paragraph,

8 (2) by striking the period at the end of para-  
9 graph (2) and inserting “; or”, and

10 (3) by adding at the end the following:

11 “(3) a drug has been designated under section  
12 526 for a rare disease or condition described in sec-  
13 tion 526(a)(2)(A) and if after such designation it is  
14 determined that—

15 “(A) such disease or condition affects more  
16 than 200,000 persons in the United States, and

17 “(B) such drug does not meet the require-  
18 ment of section 526(a)(2)(B).”.

19 **SEC. 4. SIMULTANEOUS DEVELOPMENT.**

20 (a) IN GENERAL.—Section 527(b) (21 U.S.C.  
21 360cc(b)), as amended by section 3(b), is amended by in-  
22 serting “(1)” after “(b)”, by redesignating paragraphs  
23 (1), (2), and (3) as subparagraphs (A), (B), and (C), re-  
24 spectively, by striking “for a person who is not” and by

1 inserting “for an applicant who is not”, and by adding  
2 at the end the following:

3 “(D) the Secretary finds, after providing the  
4 holder, such applicant, and any other interested per-  
5 son an opportunity to present their views, that the  
6 drugs of the holder and such applicant were devel-  
7 oped simultaneously.

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

11 “(2) For purposes of paragraph (1)(D), drugs of a hold-  
12 er and an applicant shall be considered to be developed  
13 simultaneously only if—

14 “(A) the applicant requested that its drug be des-  
15 igned under section 526 no later than 6 months  
16 after publication of the designation under section  
17 526(c) of the holder’s drug,

18 “(B) the applicant initiated the human clinical  
19 trials that the applicant relied on in its application  
20 for such approval, certification, or license not more  
21 than 12 months after the date the holder initiated  
22 the human clinical trials that the holder relied on in  
23 its application for such approval, certification, or  
24 license, and

1           “(C) the applicant submitted such application, in-  
2           cluding the reports of the clinical and animal studies  
3           necessary for approval, certification, or licensing, not  
4           more than 12 months after the holder submitted its  
5           application, including such reports, for such action.

6           “(3) Paragraph (1)(D) does not apply to a drug—

7                   “(A) for which an application under section 505  
8                   or 507 or section 351 of the Public Health Service  
9                   Act was submitted before March 1, 1994, or

10                   “(B) for which an exemption under section  
11                   505(i) or 507(d) was in effect before March 1, 1994,  
12                   for which human clinical trials were actively being  
13                   conducted before such date, and for which an appli-  
14                   cation for designation under section 526 was submit-  
15                   ted before the date of enactment of the Orphan  
16                   Drug Act Amendments of 1994.”.

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

19                   (1) by inserting “for a rare disease or condition”  
20                   after “(a)”, and

21                   (2) by striking out “shall be made available to the  
22                   public” and inserting in lieu thereof “shall be  
23                   promptly published in the Federal Register and oth-  
24                   erwise made available to the public in a manner de-

1 signed to notify persons who have such disease or  
2 condition”.

3 **SEC. 5. OFFICE FOR ORPHAN DISEASES AND CONDITIONS.**

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

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

8 “(a) There is established in the Department of  
9 Health and Human Services an Office for Orphan Dis-  
10 eases and Conditions. Such Office shall be established at  
11 a level within the Department with sufficient authority to  
12 assure full implementation of the functions and respon-  
13 sibilities established by this section.”,

14 (2) by striking “Board” each place the term ap-  
15 pears and inserting “Office”,

16 (3) by striking “drugs and devices” in sub-  
17 section (b) and inserting “drugs, devices, and medi-  
18 cal foods”,

19 (4) by inserting “of chapter V” after “sub-  
20 chapter B” in subsection (c)(1)(A),

21 (5) by adding at the end the following new sub-  
22 section:

23 “(f)(1) There is established in the Office an advisory  
24 committee to advise the Office in carrying out the func-  
25 tions of the Office under this section.

1       “(2) The advisory committee shall be comprised of  
2 11 members appointed by the Secretary, in consultation  
3 with the Office and the Commissioner of the Food and  
4 Drug Administration, from persons knowledgeable about  
5 rare diseases and conditions, including—

6           “(A) 5 representatives of organizations of per-  
7 sons with rare diseases or conditions;

8           “(B) 3 research scientists; and

9           “(C) 3 representatives of health-related compa-  
10 nies.

11       “(3) The Secretary shall also appoint, as liaisons to  
12 the advisory committee, individuals from the Food and  
13 Drug Administration, the National Institutes of Health,  
14 and other appropriate Federal agencies.

15       “(4) Vacancies occurring in the membership of the  
16 advisory committee shall be filled in the same manner as  
17 the original appointment for the position being vacated.  
18 Vacancies shall not affect the power of the remaining  
19 members to execute the duties of the advisory committee.

20       “(5) Members of the advisory committee, and liaisons  
21 to the advisory committee, shall not be compensated, but  
22 shall receive travel expenses, including per diem in lieu  
23 of subsistence, at rates authorized for employees of agen-  
24 cies under subchapter 1 of chapter 57 of title 5, United  
25 States Code, for each day the member or liaison is en-

1 gaged in the performance of duties away from the home  
2 or regular place of business of the member or liaison.

3 “(6) Notwithstanding section 1342 of title 31, United  
4 States Code, the advisory committee may accept the vol-  
5 untary services provided by a member of the advisory com-  
6 mittee or a liaison to the advisory committee.”, and

7 (6) by amending the section heading to read as  
8 follows: “OFFICE FOR ORPHAN DISEASES AND CON-  
9 DITIONS”.

10 **SEC. 6. AUTHORIZATION FOR ORPHAN DRUG ACT.**

11 Section 5(c) of the Orphan Drug Act (21 U.S.C.  
12 360ee(c)) is amended by striking “\$10,000,000” and all  
13 that follows and inserting “\$20,000,000 for fiscal year  
14 1995, \$25,000,000 for fiscal year 1996, and \$30,000,000  
15 for fiscal year 1997.”.

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