

**Calendar No. 609**

103D CONGRESS  
2D SESSION

**S. 1981**

**[Report No. 103-366]**

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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, and for other purposes.

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SEPTEMBER 14 (legislative day, SEPTEMBER 12), 1994  
Reported without amendment

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**IN THE SENATE OF THE UNITED STATES**

MARCH 24 (legislative day, FEBRUARY 22), 1994

Mrs. KASSEBAUM (for herself, Mr. METZENBAUM, Mr. KENNEDY, and Mr. SIMON) introduced the following bill; which was read twice and referred to the Committee on Labor and Human Resources

SEPTEMBER 14 (legislative day, SEPTEMBER 12), 1994

Reported by Mr. KENNEDY, without amendment

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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, and for other purposes.

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

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

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

4 (b) REFERENCE.—Whenever in this Act (other than  
5 sections 5 and 6) an amendment or repeal is expressed  
6 in terms of an amendment to, or repeal of, a section or  
7 other provision, the reference shall be considered to be  
8 made to a section or other provision of the Federal Food,  
9 Drug, and Cosmetic Act (21 U.S.C. 201 et seq.).

10 **SEC. 2. PERIOD OF EXCLUSIVITY.**

11 (a) INITIAL PERIOD.—Subsection (a) of section 527  
12 (21 U.S.C. 360cc) is amended—

13 (1) by inserting “(1)” after “(a)”;

14 (2) by redesignating paragraphs (1), (2), and  
15 (3) as subparagraphs (A), (B), and (C), respectively;

16 (3) by striking “seven years” and inserting “4  
17 years”; and

18 (4) by striking “505(c)(2)” and inserting  
19 “505(c)(1)(B)”.

20 (b) ADDITIONAL PERIOD.—Subsection (a) of section  
21 527 (21 U.S.C. 360cc) (as amended by subsection (a)) is  
22 amended by adding at the end the following new para-  
23 graphs:

24 “(2) The holder of the approved application, certifi-  
25 cation, or license of a drug to which the 4-year period of  
26 exclusivity applies under paragraph (1) may, after the ex-

1 piration of 3½ years of such period but not later than  
2 90 days before the expiration of such period, apply to the  
3 Secretary for a 3-year extension of such period. Such an  
4 application shall contain such information as the Secretary  
5 determines is necessary to evaluate such application.

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

8 “(A) demonstrates that the drug has a limited  
9 commercial potential as determined under regula-  
10 tions of the Secretary, taking into account sales in-  
11 formation respecting such drug and any other factor  
12 identified by the Secretary in such regulations that  
13 is relevant to the commercial potential of such drug,  
14 and

15 “(B) makes such demonstration on the basis of  
16 the regulations of the Secretary referred to in sub-  
17 paragraph (A) that were in effect—

18 “(i) on the date—

19 “(I) such drug received its designation  
20 under section 526(a), or

21 “(II) such applicant applied for an ex-  
22 emption for such drug under section 505(i)  
23 or 507(d),

24 whichever first occurs, or

1           “(ii) if the date under clause (i) occurred  
2           before the date such regulations were in effect,  
3           on the date such regulations were in effect.”.

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

6           (1) by striking “during the seven-year period  
7           beginning on the date of the application approval”  
8           and inserting “during the applicable period of exclu-  
9           sivity under subsection (a)”;

10          (2) by striking “such seven year period” and in-  
11          serting “the applicable period of exclusivity under  
12          subsection (a)”.

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

15          (1) for which an application under section 505  
16          or 507 of the Federal Food, Drug, and Cosmetic Act  
17          or section 351 of the Public Health Service Act was  
18          submitted before March 1, 1994; or

19          (2) for which an exemption under section 505(i)  
20          or 507(d) of the Federal Food, Drug, and Cosmetic  
21          Act was in effect before March 1, 1994, for which  
22          human clinical trials were actively being conducted  
23          before such date, and for which an application for  
24          designation under section 526 of such Act was sub-

1       mitted before the date of enactment of the Orphan  
2       Drug Act Amendments of 1994.

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

7       (e) REGULATIONS.—The Secretary shall issue final  
8       regulations to implement paragraphs (2) and (3) of sec-  
9       tion 527(a) of the Federal Food, Drug, and Cosmetic Act  
10      (21 U.S.C. 360cc) (as amended by subsection (b)) not  
11      later than 6 months after the date of the enactment of  
12      this Act.

13      **SEC. 3. DESIGNATIONS.**

14      (a) IN GENERAL.—Section 526(a)(2) (21 U.S.C.  
15      360bb(a)(2)) is amended to read as follows:

16      “(2) For purposes of paragraph (1), the term ‘rare  
17      disease or condition’ means any disease or condition  
18      that—

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

21                      “(i) the facts and circumstances as of the  
22                      date the request for designation of the drug  
23                      under this subsection is made, and

24                      “(ii) projections as to the number of per-  
25                      sons who will be affected by the disease or con-

1           dition on a date which is 3 years from date  
2           such request was made, or

3           “(B) affects more than 200,000 persons in the  
4           United States and for which there is no reasonable  
5           expectation that the cost of developing and making  
6           available in the United States a drug for such dis-  
7           ease or condition will be recovered from sales in the  
8           United States of such drug.”.

9           (b) EXCLUSIVITY.—Section 527(b) (21 U.S.C.  
10 360cc(b)) is amended—

11           (1) in paragraph (1), by striking “or” at the  
12           end of such paragraph;

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

15           (3) by adding at the end the following new  
16           paragraph:

17           “(3) a drug has been designated under section  
18           526 for a rare disease or condition described in sec-  
19           tion 526(a)(2)(A) and if after such designation it is  
20           determined that—

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

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

1 **SEC. 4. SIMULTANEOUS DEVELOPMENT.**

2 (a) IN GENERAL.—Section 527(b) (21 U.S.C.  
3 360cc(b)), as amended by section 3(b), is amended by—

4 (1) inserting “(1)” after “(b)”;

5 (2) by redesignating paragraphs (1), (2), and  
6 (3) as subparagraphs (A), (B), and (C), respectively;

7 (3) by striking “for a person who is not” and  
8 inserting “for an applicant who is not”; and

9 (4) by adding at the end the following new sub-  
10 paragraphs:

11 “(D) the Secretary finds, after providing the  
12 holder, such applicant, and any other interested per-  
13 son an opportunity to present their views, that the  
14 drugs of the holder and such applicant were devel-  
15 oped simultaneously.

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

19 “(2) For purposes of paragraph (1)(D), drugs of a  
20 holder and an applicant shall be considered to be devel-  
21 oped simultaneously only if—

22 “(A) the applicant requested that its drug be  
23 designated under section 526 not later than 6  
24 months after publication of the designation under  
25 section 526(c) of the holder’s drug;

1           “(B) the applicant initiated the human clinical  
2 trials that the applicant relied on in its application  
3 for such approval, certification, or license not more  
4 than 12 months after the date the holder initiated  
5 the human clinical trials that the holder relied on in  
6 its application for such approval, certification, or li-  
7 cense; and

8           “(C) the applicant submitted such application,  
9 including the reports of the clinical and animal stud-  
10 ies necessary for approval, certification, or licensing,  
11 not more than 12 months after the holder submitted  
12 its application, including such reports, for such ac-  
13 tion.

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

15           “(A) for which an application under section 505  
16 or 507 or section 351 of the Public Health Service  
17 Act was submitted before March 1, 1994; or

18           “(B) for which an exemption under section  
19 505(i) or 507(d) was in effect before March 1, 1994,  
20 for which human clinical trials were actively being  
21 conducted before such date, and for which an appli-  
22 cation for designation under section 526 was submit-  
23 ted before the date of enactment of the Orphan  
24 Drug Act Amendments of 1994.”.

1 (b) PUBLICATION.—Section 526(c) (21 U.S.C.  
2 360bb(c)) is amended—

3 (1) by inserting “for a rare disease or condi-  
4 tion” after “(a)”; and

5 (2) by striking “shall be made available to the  
6 public” and inserting “shall be promptly published  
7 in the Federal Register and otherwise made avail-  
8 able to the public in a manner designed to notify  
9 persons who have such disease or condition”.

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

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

13 (1) in subsection (a), to read as follows:

14 “(a) There is established in the Department of  
15 Health and Human Services an Office for Orphan Dis-  
16 eases and Conditions. Such Office shall be established at  
17 a level within the Department with sufficient authority to  
18 assure full implementation of the functions and respon-  
19 sibilities established by this section.”;

20 (2) by striking “Board” each place the term ap-  
21 pears and inserting “Office”;

22 (3) in subsection (b), by striking “drugs and  
23 devices” and inserting “drugs, devices, and medical  
24 foods”;

1 (4) in subsection (c)(1)(A), by inserting “of  
2 chapter V” after “subchapter B”;

3 (5) by adding at the end the following new sub-  
4 section:

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

8 “(2) The advisory committee shall be comprised of  
9 11 members appointed by the Secretary, in consultation  
10 with the Office and the Commissioner of the Food and  
11 Drug Administration, from persons knowledgeable about  
12 rare diseases and conditions, including—

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

15 “(B) 3 research scientists; and

16 “(C) 3 representatives of health-related compa-  
17 nies.

18 “(3) The Secretary shall also appoint, as liaisons to  
19 the advisory committee, individuals from the Food and  
20 Drug Administration, the National Institutes of Health,  
21 and other appropriate Federal agencies.

22 “(4) Vacancies occurring in the membership of the  
23 advisory committee shall be filled in the same manner as  
24 the original appointment for the position being vacated.

1 Vacancies shall not affect the power of the remaining  
2 members to execute the duties of the advisory committee.

3 “(5) Members of the advisory committee, and liaisons  
4 to the advisory committee, shall not be compensated, but  
5 shall receive travel expenses, including per diem in lieu  
6 of subsistence, at rates authorized for employees of agen-  
7 cies under subchapter 1 of chapter 57 of title 5, United  
8 States Code, for each day the member or liaison is en-  
9 gaged in the performance of duties away from the home  
10 or regular place of business of the member or liaison.

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

15 (6) by amending the section heading to read as  
16 follows:

17 “OFFICE FOR ORPHAN DISEASES AND CONDITIONS”.

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

19 Section 5(c) of the Orphan Drug Act (21 U.S.C.  
20 360ee(c)) is amended by striking “\$10,000,000” and all  
21 that follows and inserting “\$20,000,000 for fiscal year  
22 1995, \$25,000,000 for fiscal year 1996, and \$30,000,000  
23 for fiscal year 1997.”.