

Calendar No. 298

107TH CONGRESS
1ST SESSION

S. 1379

[Report No. 107-129]

To amend the Public Health Service Act to establish an Office of Rare Diseases at the National Institutes of Health, and for other purposes.

IN THE SENATE OF THE UNITED STATES

AUGUST 3, 2001

Mr. KENNEDY (for himself, Mr. HATCH, Mr. HOLLINGS, Mr. BINGAMAN, Mr. DURBIN, Mr. JEFFORDS, Mrs. CLINTON, Mr. SMITH of Oregon, Ms. COLLINS, and Mr. KERRY) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

DECEMBER 18, 2001

Reported by Mr. KENNEDY, with an amendment

[Strike out all after the enacting clause and insert the part printed in italic]

A BILL

To amend the Public Health Service Act to establish an Office of Rare Diseases at the National Institutes of Health, 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.**

2 This Act may be cited as the "Rare Diseases Act of
3 2001".

4 **SEC. 2. FINDINGS AND PURPOSES.**

5 (a) FINDINGS.—Congress makes the following find-
6 ings:

7 (1) Rare diseases and disorders are those which
8 affect small patient populations, typically popu-
9 lations smaller than 200,000 individuals in the
10 United States. Such diseases and conditions include
11 Huntington's disease, amyotrophic lateral sclerosis
12 (Lou Gehrig's disease), Tourette syndrome, Crohn's
13 disease, cystic fibrosis, cystinosis, and Duchenne
14 muscular dystrophy.

15 (2) For many years, the 25,000,000 Americans
16 suffering from the over 6,000 rare diseases and dis-
17 orders were denied access to effective medicines be-
18 cause prescription drug manufacturers could rarely
19 make a profit from marketing drugs for such small
20 groups of patients. The prescription drug industry
21 did not adequately fund research into such treat-
22 ments. Despite the urgent health need for these
23 medicines, they came to be known as "orphan
24 drugs" because no companies would commercialize
25 them.

23 (6) Despite the tremendous success of the Or-
24 phan Drug Act, rare diseases and disorders deserve
25 greater emphasis in the national biomedical research

1 enterprise. The Office of Rare Diseases at the Na-
2 tional Institutes of Health was created in 1993, but
3 lacks a statutory authorization.

4 (7) The National Institutes of Health has re-
5 ceived a substantial increase in research funding
6 from Congress for the purpose of expanding the na-
7 tional investment of the United States in behavioral
8 and biomedical research.

9 (8) Notwithstanding such increases, funding for
10 rare diseases and disorders at the National Insti-
11 tutes of Health has not increased appreciably.

12 (9) To redress this oversight, the Department
13 of Health and Human Services has proposed the es-
14 tablishment of a network of regional centers of excel-
15 lence for research on rare diseases.

16 (10) The Food and Drug Administration sup-
17 ports small clinical trials through Orphan Products
18 Research Grants. Such grants embody successful
19 partnerships of government and industry, and have
20 led to the development of at least 23 drugs and four
21 medical devices for rare diseases and disorders. Yet
22 the appropriations in Fiscal Year 2001 for such
23 grants were less than in Fiscal Year 1995.

24 (b) PURPOSES.—The purposes of this Act are to—

1 (1) amend the Public Health Service Act to es-
2 tablish an Office of Rare Diseases at the National
3 Institutes of Health; and

7 **TITLE I—NATIONAL INSTITUTES**
8 **OF HEALTH**

9 SEC. 101. NIH OFFICE OF RARE DISEASES.

10 Title IV of the Public Health Service Act (42 U.S.C.
11 281 et seq.) is amended by inserting after section 404D
12 the following:

13 "OFFICE OF RARE DISEASES

14 “SEC. 404E. (a) ESTABLISHMENT.—There is estab-
15 lished within the Office of the Director of NIH an office
16 to be known as the Office of Rare Diseases (in this section
17 referred to as the ‘Office’), which shall be headed by a
18 Director (in this section referred to as the ‘Director’), ap-
19 pointed by the Director of NIH.

20 ~~“(b) DUTIES.”~~

21 “(1) IN GENERAL.—The Director of the Office
22 shall carry out the following:

23 “(A) The Director shall recommend an
24 agenda for conducting and supporting research
25 on rare diseases through the national research
26 institutes and centers. The agenda shall provide

1 for a broad range of research and education ac-
2 tivities, including scientific workshops and
3 symposia to identify research opportunities for
4 rare diseases.

5 “(B) The Director shall, with respect to
6 rare diseases, promote coordination and co-
7 operation among the national research insti-
8 tutes and centers and entities whose research is
9 supported by such institutes.

10 “(C) The Director shall enter into coopera-
11 tive agreements with and make grants for re-
12 gional centers of excellence on rare diseases in
13 accordance with section 404F.

14 “(D) The Director shall promote the suffi-
15 cient allocation of the resources of the National
16 Institutes of Health for conducting and sup-
17 porting research on rare diseases.

18 “(E) The Director shall promote and en-
19 courage the establishment of a centralized
20 clearinghouse for rare and genetic disease infor-
21 mation that will provide understandable infor-
22 mation about these diseases to the public, med-
23 ical professionals, patients and families.

24 “(F) The Director shall biennially prepare
25 a report that describes the research and edu-

1 eation activities on rare diseases being con-
2 ducted or supported through the national re-
3 search institutes and eenters, and that identi-
4 fies particular projects or types of projects that
5 should in the future be conducted or supported
6 by the national research institutes and eenters
7 or other entities in the field of research on rare
8 diseases.

9 “(G) The Director shall prepare the NIH
10 Director’s annual report to Congress on rare
11 disease research conducted by or supported
12 through the national research institutes and
13 eenters.

14 “(2) PRINCIPAL ADVISOR REGARDING ORPHAN
15 DISEASES.—With respect to rare diseases, the Direc-
16 tor shall serve as the principal advisor to the Direc-
17 tor of NIH and shall provide advice to other relevant
18 agencies. The Director shall provide liaison with na-
19 tional and international patient, health and scientific
20 organizations concerned with rare diseases.

21 “(e) DEFINITION.—For purposes of this section, the
22 term ‘rare disease’ means any disease or condition that
23 affects less than 200,000 persons in the United States.

24 “(d) AUTHORIZATION OF APPROPRIATIONS.—For the
25 purpose of carrying out this section, there are authorized

1 to be appropriated \$4,000,000 for fiscal year 2002, and
2 such sums as may be necessary for each subsequent fiscal
3 year.”.

4 **SEC. 102. RARE DISEASE REGIONAL CENTERS OF EXCEL-
5 LENCE.**

6 Title IV of the Public Health Service Act (42 U.S.C.
7 281 et seq.), as amended by section 101, is further amend-
8 ed by inserting after section 404E the following:

9 “**RARE DISEASE REGIONAL CENTERS OF EXCELLENCE**

10 “**SEC. 404F. (a) COOPERATIVE AGREEMENTS AND
11 GRANTS.**

12 “**(1) IN GENERAL.**—The Director of the Office
13 of Rare Diseases (in this section referred to as the
14 ‘Director’) shall enter into cooperative agreements
15 with and make grants to public or private nonprofit
16 entities to pay all or part of the cost of planning, es-
17 tablishing, or strengthening, and providing basic op-
18 erating support for regional centers of excellence for
19 clinical research into, training in, and demonstration
20 of diagnostic, prevention, control, and treatment
21 methods for rare diseases.

22 “**(2) POLICIES.**—A cooperative agreement or
23 grant under paragraph (1) shall be entered into in
24 accordance with policies established by the Director
25 of NIH.

1 “(b) COORDINATION WITH OTHER INSTITUTES.—

2 The Director shall coordinate the activities under this sec-
3 tion with similar activities conducted by other national re-
4 search institutes, centers and agencies of the National In-
5 stitutes of Health and by the Food and Drug Administra-
6 tion to the extent that such institutes, centers and agen-
7 cies have responsibilities that are related to rare diseases.

8 “(c) USES FOR FEDERAL PAYMENTS UNDER COOP-
9 ERATIVE AGREEMENTS OR GRANTS.—Federal payments
10 made under a cooperative agreement or grant under sub-
11 section (a) may be used for—

12 “(1) staffing, administrative, and other basic
13 operating costs, including such patient care costs as
14 are required for research;

15 “(2) clinical training, including training for al-
16 lied health professionals, continuing education for
17 health professionals and allied health professions
18 personnel, and information programs for the public
19 with respect to rare diseases; and

20 “(3) clinical research and demonstration pro-
21 grams.

22 “(d) PERIOD OF SUPPORT; ADDITIONAL PERIODS.—

23 Support of a center under subsection (a) may be for a
24 period of not to exceed 5 years. Such period may be ex-
25 tended by the Director for additional periods of not more

1 than 5 years if the operations of such center have been
2 reviewed by an appropriate technical and scientific peer
3 review group established by the Director and if such group
4 has recommended to the Director that such period should
5 be extended.

6 “(e) AUTHORIZATION OF APPROPRIATIONS.—For the
7 purpose of carrying out this section, there are authorized
8 to be appropriated \$20,000,000 for fiscal year 2002, and
9 such sums as may be necessary for each subsequent fiscal
10 year.”.

11 **TITLE II—FOOD AND DRUG**
12 **ADMINISTRATION**

13 SEC. 201. GRANTS AND CONTRACTS FOR THE DEVELOP-
14 MENT OF ORPHAN DRUGS.

15 Subseetion (e) of seetion 5 of the Orphan Drug Act
16 (21 U.S.C. 360ee(e)) is amended to read as follows:

17 "(e) For grants and contracts under subsection (a)
18 there are authorized to be appropriated \$25,000,000 for
19 fiscal year 2002, and such sums as may be necessary for
20 each subsequent fiscal year.".

21 SEC. 202. TECHNICAL AMENDMENT.

22 Section 527(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ee(a)) is amended in the matter
23 following paragraph (2)—

25 (1) by striking "of such certification,"; and

1 (2) by striking “, the issuance of the certifi-
2 cation.”.

3 SECTION 1. SHORT TITLE.

4 *This Act may be cited as the “Rare Diseases Act of*
5 *2001”.*

6 SEC. 2. FINDINGS AND PURPOSES.

7 (a) FINDINGS.—Congress makes the following findings:

1 (3) During the 1970s, an organization called the
2 National Organization for Rare Disorders (NORD)
3 was founded to provide services and to lobby on behalf
4 of patients with rare diseases and disorders. NORD
5 was instrumental in pressing Congress for legislation
6 to encourage the development of orphan drugs.

7 (4) The Orphan Drug Act created financial in-
8 centives for the research and production of such or-
9 phan drugs. New federal programs at the National
10 Institutes of Health and the Food and Drug Adminis-
11 tration encouraged clinical research and commercial
12 product development for products that target rare dis-
13 eases. An Orphan Products Board was established to
14 promote the development of drugs and devices for rare
15 diseases or disorders.

16 (5) Before 1983, some 38 orphan drugs had been
17 developed. Since the enactment of the Orphan Drug
18 Act, more than 220 new orphan drugs have been ap-
19 proved and marketed in the United States and more
20 than 800 additional drugs are in the research pipe-
21 line.

22 (6) Despite the tremendous success of the Orphan
23 Drug Act, rare diseases and disorders deserve greater
24 emphasis in the national biomedical research enter-
25 prise. The Office of Rare Diseases at the National In-

1 *stitutes of Health was created in 1993, but lacks a*
2 *statutory authorization.*

3 *(7) The National Institutes of Health has re-*
4 *ceived a substantial increase in research funding from*
5 *Congress for the purpose of expanding the national*
6 *investment of the United States in behavioral and*
7 *biomedical research.*

8 *(8) Notwithstanding such increases, funding for*
9 *rare diseases and disorders at the National Institutes*
10 *of Health has not increased appreciably.*

11 *(9) To redress this oversight, the Department of*
12 *Health and Human Services has proposed the estab-*
13 *lishment of a network of regional centers of excellence*
14 *for research on rare diseases.*

15 *(10) The Food and Drug Administration sup-*
16 *ports small clinical trials through Orphan Products*
17 *Research Grants. Such grants embody successful part-*
18 *nerships of government and industry, and have led to*
19 *the development of at least 23 drugs and four medical*
20 *devices for rare diseases and disorders. Yet the appro-*
21 *priations in Fiscal Year 2001 for such grants were*
22 *less than in Fiscal Year 1995.*

23 *(b) PURPOSES.—The purposes of this Act are to—*

1 (1) amend the Public Health Service Act to es-
2 tablish an Office of Rare Diseases at the National In-
3 stitutes of Health; and

4 (2) increase the national investment in the devel-
5 opment of diagnostics and treatments for patients
6 with rare diseases and disorders.

7 **TITLE I—NATIONAL INSTITUTES
8 OF HEALTH**

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16 to be known as the Office of Rare Diseases (in this section
17 referred to as the ‘Office’), which shall be headed by a Direc-
18 tor (in this section referred to as the ‘Director’), appointed
19 by the Director of NIH.

20 “(b) DUTIES.—

21 “(1) IN GENERAL.—The Director of the Office
22 shall carry out the following:

23 “(A) The Director shall recommend an
24 agenda for conducting and supporting research
25 on rare diseases through the national research
26 institutes and centers. The agenda shall provide

1 *for a broad range of research and education ac-
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3 *symposia to identify research opportunities for
4 *rare diseases.****

5 *“(B) The Director shall, with respect to rare
6 *diseases, promote coordination and cooperation
7 *among the national research institutes and cen-
8 *ters and entities whose research is supported by
9 *such institutes.*****

10 *“(C) The Director, in collaboration with the
11 *directors of the other relevant institutes and cen-
12 *ters of the National Institutes of Health, shall
13 *enter into cooperative agreements with and make
14 *grants for regional centers of excellence on rare
15 *diseases in accordance with section 404F.******

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17 *cient allocation of the resources of the National
18 *Institutes of Health for conducting and sup-
19 *porting research on rare diseases.****

20 *“(E) The Director shall promote and en-
21 *courage the establishment of a centralized clear-
22 *inghouse for rare and genetic disease informa-
23 *tion that will provide understandable informa-
24 *tion about these diseases to the public, medical
25 *professionals, patients and families.******

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11 *Director’s annual report to Congress on rare dis-*
12 *ease research conducted by or supported through*
13 *the national research institutes and centers.*

14 “(2) **PRINCIPAL ADVISOR REGARDING ORPHAN**
15 **DISEASES.**—*With respect to rare diseases, the Director*
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17 *NIH and shall provide advice to other relevant agen-*
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20 *nizations concerned with rare diseases.*

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5 group established by the Director and if such group has rec-
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