

Calendar No. 714

103D CONGRESS
2D SESSION

S. 1203

A BILL

To establish a Center for Rare Disease Research in the National Institutes of Health, and for other purposes.

OCTOBER 5 (legislative day, SEPTEMBER 12), 1994
Reported with an amendment

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[Report No. 103-399]

To establish a Center for Rare Disease Research in the National Institutes of Health, and for other purposes.

IN THE SENATE OF THE UNITED STATES

JULY 1 (legislative day, JUNE 30), 1993

Mr. HATFIELD (for himself and Mr. DECONCINI) introduced the following bill; which was read twice and referred to the Committee on Labor and Human Resources

OCTOBER 5 (legislative day, SEPTEMBER 12), 1994

Reported by Mr. KENNEDY, with an amendment

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

A BILL

To establish a Center for Rare Disease Research in 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,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Center for Rare
5 Disease Research Act of 1993”.

1 **SEC. 2. ESTABLISHMENT OF CENTER FOR RARE DISEASE**
 2 **RESEARCH.**

3 Part E of title IV of the Public Health Service Act
 4 (42 U.S.C. 287 et seq.) is amended by adding at the end
 5 thereof the following new subpart:

6 **“Subpart 4—Center for Rare Disease Research**

7 **“SEC. 486A. ESTABLISHMENT.**

8 “(a) IN GENERAL.—There shall be established in the
 9 Office of the Director of the National Institutes of Health
 10 a Center for Rare Disease Research (hereafter referred
 11 to in this section as the ‘Center’). The Director of the Na-
 12 tional Institutes of Health shall appoint an individual with
 13 expertise in rare diseases to serve as the Director of
 14 the Center (hereafter referred to in this section as the
 15 ‘Director’).

16 “(b) PURPOSE.—The general purpose of the Center
 17 is to promote and coordinate the conduct of research on
 18 rare diseases and to establish and manage a rare disease
 19 research clinical database. In carrying out the purpose of
 20 the Center, the Director shall—

21 “(1) conduct and support research and research
 22 training;

23 “(2) award grants and contracts;

24 “(3) identify projects of research on rare dis-
 25 eases that should be conducted or supported by the
 26 National Institutes of Health;

1 “(4) disseminate information among the insti-
2 tutes and the public on rare diseases;

3 “(5) develop and maintain a central database
4 on current clinical research projects for rare dis-
5 eases;

6 “(6) encourage the participation of a diversity
7 of individuals in the conduct of rare disease re-
8 search; and

9 “(7) coordinate the conduct of rare disease re-
10 search among all institutes and other Federal agen-
11 cies.

12 “(c) STRATEGIC RESEARCH PLAN.—The Director
13 shall develop a comprehensive plan for the conduct and
14 support of research on rare diseases. The plan shall—

15 “(1) identify current research activities con-
16 ducted or supported by the Federal Government and
17 private entities, opportunities and needs for addi-
18 tional research and priorities for such research;

19 “(2) make recommendations for the improved
20 coordination of research conducted by the Federal
21 Government among its agencies and private entities;

22 “(3) give emphasis to areas with respect to
23 which little research has been conducted;

24 “(4) examine the extent of research on gene
25 therapy and genetic transfers and develop a plan to

1 enhance the extent of research on gene therapy, par-
2 ticularly for rare diseases;

3 ~~“(5) determine the need for registries of re-~~
4 ~~search subjects and epidemiological studies of rare~~
5 ~~disease populations;~~

6 ~~“(6) identify the obstacles to the development~~
7 ~~of treatments for rare diseases; and~~

8 ~~“(7) examine training and education require-~~
9 ~~ments for physicians treating rare diseases.~~

10 ~~“(d) COORDINATING COMMITTEE.—~~

11 ~~“(1) ESTABLISHMENT.—The Secretary shall es-~~
12 ~~tablish a Coordinating Committee on Rare Disease~~
13 ~~Research (hereafter referred to as the ‘Coordinating~~
14 ~~Committee’) to be composed of—~~

15 ~~“(A) the Directors of the agencies and in-~~
16 ~~stitutes of the National Institutes of Health;~~

17 ~~“(B) the Director of the Center who shall~~
18 ~~serve as the chairperson of the Coordinating~~
19 ~~Committee;~~

20 ~~“(C) the Administrator of the Veterans~~
21 ~~Administration;~~

22 ~~“(D) the Secretary of Defense;~~

23 ~~“(E) the Administrator of the Food and~~
24 ~~Drug Administration;~~

1 ~~“(F) the Administrator of the Health Care~~
2 ~~Financing Administration; and~~

3 ~~“(G) the Director of the Center for Dis-~~
4 ~~ease Control and Prevention.~~

5 ~~“(2) DUTIES.—The Coordinating Committee~~
6 ~~shall—~~

7 ~~“(A) identify needs for research on rare~~
8 ~~diseases;~~

9 ~~“(B) estimate the funds needed during~~
10 ~~each fiscal year to adequately fund research on~~
11 ~~rare diseases;~~

12 ~~“(C) encourage the agencies and Institutes~~
13 ~~of the National Institutes of Health to support~~
14 ~~and conduct research on rare diseases;~~

15 ~~“(D) coordinate research on rare diseases~~
16 ~~in the Federal Government in an effort to avoid~~
17 ~~duplication and enhance research in areas tradi-~~
18 ~~tionally not funded;~~

19 ~~“(E) ensure that a diversity of individuals~~
20 ~~are able to participate as research subjects in~~
21 ~~projects conducting research on rare diseases;~~
22 ~~and~~

23 ~~“(F) biennially prepare and submit to the~~
24 ~~Secretary and the Congress a report concerning~~
25 ~~the activities of the Coordinating Committee.~~

1 “(e) NATIONAL ADVISORY BOARD ON RARE DISEASE
2 RESEARCH.—

3 “(1) ESTABLISHMENT.—The Director shall es-
4 tablish an advisory board to be known as the Advi-
5 sory Board on Rare Disease Research (hereafter re-
6 ferred to in this section as the ‘Board’).

7 “(2) DUTIES.—The Board shall review and as-
8 sess Federal research needs, priorities, activities,
9 funding and findings regarding rare diseases and
10 shall advise the Director on the development and im-
11 plementation of the research plan required under
12 subsection (c).

13 “(3) COMPOSITION.—The Board shall be com-
14 posed of 15 individuals to be appointed by the Direc-
15 tor of the National Institutes of Health, of which
16 eight individuals shall be representatives of health
17 and scientific disciplines with respect to rare dis-
18 eases and seven individuals shall be representing the
19 interest of individuals with rare diseases. Such indi-
20 viduals shall not be officers or employees of the
21 Federal Government.

22 “(4) EX OFFICIO MEMBERS.—The members of
23 the Coordinating Committee shall serve as ex officio
24 members of the Board.

1 ~~“(5) CHAIRPERSON.—~~The members of the
2 Board shall annually select an individual to serve as
3 the chairperson of the Board.

4 ~~“(f) NATIONAL RARE DISEASE CLINICAL AND IN-~~
5 ~~FORMATIONAL DATABASE.—~~

6 ~~“(1) ESTABLISHMENT.—~~The Director shall es-
7 tablish a database that—

8 ~~“(A) identifies the extent, location, and~~
9 ~~sponsor of current research conducted on rare~~
10 ~~diseases;~~

11 ~~“(B) connects researchers of rare diseases~~
12 ~~with patients needed as subjects for clinical~~
13 ~~trials;~~

14 ~~“(C) provides physicians and individuals~~
15 ~~with information concerning the location and~~
16 ~~sponsors of clinical trials on rare diseases; and~~

17 ~~“(D) connects patients with support~~
18 ~~groups in rare diseases.~~

19 ~~“(2) AVAILABILITY.—~~The Director shall ensure
20 that information in the database is available to the
21 general public.

22 ~~“(g) AUTHORIZATION OF APPROPRIATIONS.—~~There
23 are authorized to be appropriated to carry out this section,
24 \$10,000,000 for each of the fiscal years 1994 through
25 1996.

1 “(h) **DEFINITION.**—As used in this section, the term
2 ‘rare disease’ means any disease or condition that affects
3 fewer than 200,000 individuals in the United States.”.

4 **SECTION 1. SHORT TITLE.**

5 *This Act may be cited as the “Office for Rare Disease
6 Research Act of 1994”.*

7 **SEC. 2. ESTABLISHMENT OF OFFICE FOR RARE DISEASE
8 RESEARCH.**

9 *Part A of title IV of the Public Health Service Act
10 (42 U.S.C. 281 et seq.) is amended by adding at the end
11 thereof the following new section:*

12 **“SEC. 404F. OFFICE FOR RARE DISEASE RESEARCH.**

13 “(a) **ESTABLISHMENT.**—*There is established within
14 the Office of the Director of the National Institutes of
15 Health an office to be known as the Office for Rare Disease
16 Research (in this section referred to as the ‘Office’). The
17 Office shall be headed by a director, who shall be appointed
18 by the Director of the National Institutes of Health.*

19 “(b) **PURPOSE.**—*The purpose of the Office is to pro-
20 mote and coordinate the conduct of research on rare diseases
21 through a strategic research plan and to establish and man-
22 age a rare disease research clinical database.*

23 “(c) **ADVISORY COUNCIL.**—*The Secretary shall estab-
24 lish an advisory council for the purpose of providing advice
25 to the director of the Office concerning carrying out the*

1 *strategic research plan and other duties under this section.*
2 *Section 222 shall apply to such council to the same extent*
3 *and in the same manner as such section applies to commit-*
4 *tees or councils established under such section.*

5 “(d) *DUTIES.—In carrying out subsection (b), the di-*
6 *rector of the Office shall—*

7 “(1) *develop a comprehensive plan for the con-*
8 *duct and support of research on rare diseases;*

9 “(2) *coordinate and disseminate information*
10 *among the institutes and the public on rare diseases;*

11 “(3) *support research training and encourage the*
12 *participation of a diversity of individuals in the con-*
13 *duct of rare disease research;*

14 “(4) *identify projects or research on rare diseases*
15 *that should be conducted or supported by the National*
16 *Institutes of Health;*

17 “(5) *develop and maintain a central database on*
18 *current government sponsored clinical research*
19 *projects for rare diseases;*

20 “(6) *determine the need for registries of research*
21 *subjects and epidemiological studies of rare disease*
22 *populations; and*

23 “(7) *prepare biennial reports on the activities*
24 *carried out or to be carried out by the Office and sub-*
25 *mit such reports to the Secretary and the Congress.”.*