

103<sup>D</sup> CONGRESS  
1<sup>ST</sup> SESSION

# H. R. 3577

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

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## IN THE HOUSE OF REPRESENTATIVES

NOVEMBER 19, 1993

Mr. WASHINGTON (for himself, Mr. FRANKS of Connecticut, and Mr. TOWNS) introduced the following bill; which was referred to the Committee on Energy and Commerce

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## 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 Dis-  
5 ease Research Act of 1993”.

6 **SEC. 2. ESTABLISHMENT OF CENTER FOR RARE DISEASE**  
7 **RESEARCH.**

8 Part E of title IV of the Public Health Service Act  
9 (42 U.S.C. 287 et seq.) is amended by adding at the end  
10 thereof the following new subpart:

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

2       **“SEC. 485C. ESTABLISHMENT.**

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

11          “(b) PURPOSE.—The general purpose of the Center  
12 is to promote and coordinate the conduct of research on  
13 rare diseases and to establish and manage a rare disease  
14 research clinical database. In carrying out the purpose of  
15 the Center, the Director shall—

16           “(1) conduct and support research and research  
17 training;

18           “(2) award grants and contracts;

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

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

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

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

4           “(7) coordinate the conduct of rare disease re-  
5 search among all institutes and other Federal agen-  
6 cies.

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

10           “(1) identify current research activities con-  
11 ducted or supported by the Federal Government and  
12 private entities, opportunities and needs for addi-  
13 tional research and priorities for such research;

14           “(2) make recommendations for the improved  
15 coordination of research conducted by the Federal  
16 Government among its agencies and private entities;

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

19           “(4) examine the extent of research on gene  
20 therapy and genetic transfers and develop a plan to  
21 enhance the extent of research on gene therapy, par-  
22 ticularly for rare diseases;

23           “(5) determine the need for registries of re-  
24 search subjects and epidemiological studies of rare  
25 disease populations;

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

3           “(7) examine training and education require-  
4 ments for physicians treating rare diseases.

5           “(d) COORDINATING COMMITTEE.—

6           “(1) ESTABLISHMENT.—The Secretary shall es-  
7 tablish a Coordinating Committee on Rare Disease  
8 Research (hereafter referred to as the ‘Coordinating  
9 Committee’) to be composed of—

10           “(A) the Directors of the agencies and in-  
11 stitutes of the National Institutes of Health;

12           “(B) the Director of the Center who shall  
13 serve as the chairperson of the Coordinating  
14 Committee;

15           “(C) the Administrator of the Veterans  
16 Administration;

17           “(D) the Secretary of Defense;

18           “(E) the Administrator of the Food and  
19 Drug Administration;

20           “(F) the Administrator of the Health Care  
21 Financing Administration; and

22           “(G) the Director of the Center for Dis-  
23 ease Control and Prevention.

24           “(2) DUTIES.—The Coordinating Committee  
25 shall—

1           “(A) identify needs for research on rare  
2 diseases;

3           “(B) estimate the funds needed during  
4 each fiscal year to adequately fund research on  
5 rare diseases;

6           “(C) encourage the agencies and Institutes  
7 of the National Institutes of Health to support  
8 and conduct research on rare diseases;

9           “(D) coordinate research on rare diseases  
10 in the Federal Government in an effort to avoid  
11 duplication and enhance research in areas tradi-  
12 tionally not funded;

13           “(E) ensure that a diversity of individuals  
14 are able to participate as research subjects in  
15 projects conducting research on rare diseases;  
16 and

17           “(F) biennially prepare and submit to the  
18 Secretary and the Congress a report concerning  
19 the activities of the Coordinating Committee.

20           “(e) NATIONAL ADVISORY BOARD ON RARE DISEASE  
21 RESEARCH.—

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

1           “(2) DUTIES.—The Board shall review and as-  
2           sess Federal research needs, priorities, activities,  
3           funding and findings regarding rare diseases and  
4           shall advise the Director on the development and im-  
5           plementation of the research plan required under  
6           subsection (c).

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

16          “(4) EX OFFICIO MEMBERS.—The members of  
17          the Coordinating Committee shall serve as ex officio  
18          members of the Board.

19          “(5) CHAIRPERSON.—The members of the  
20          Board shall annually select an individual to serve as  
21          the chairperson of the Board.

22          “(f) NATIONAL RARE DISEASE CLINICAL AND IN-  
23          FORMATIONAL DATABASE.—

24          “(1) ESTABLISHMENT.—The Director shall es-  
25          tablish a database that—

1           “(A) identifies the extent, location, and  
2           sponsor of current research conducted on rare  
3           diseases;

4           “(B) connects researchers of rare diseases  
5           with patients needed as subjects for clinical  
6           trials;

7           “(C) provides physicians and individuals  
8           with information concerning the location and  
9           sponsors of clinical trials on rare diseases; and

10          “(D) connects patients with support  
11          groups in rare diseases.

12          “(2) AVAILABILITY.—The Director shall ensure  
13          that information in the database is available to the  
14          general public.

15          “(g) AUTHORIZATION OF APPROPRIATIONS.—There  
16          are authorized to be appropriated to carry out this section,  
17          \$10,000,000 for each of the fiscal years 1994 through  
18          1996.

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

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