

107TH CONGRESS
2D SESSION

H. R. 4013

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 HOUSE OF REPRESENTATIVES

MARCH 20, 2002

Mr. SHIMKUS (for himself, Mr. WAXMAN, Mr. FOLEY, Mr. BROWN of Ohio, Mrs. ROUKEMA, Mr. RUSH, Mr. KING, Mr. GREENWOOD, and Mr. DINGELL) introduced the following bill; which was referred to the Committee on Energy and Commerce

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 2002”.

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.

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

8 (4) The Orphan Drug Act created financial in-
9 centives for the research and production of such or-
10 phan drugs. New Federal programs at the National
11 Institutes of Health and the Food and Drug Admin-
12 istration encouraged clinical research and commer-
13 cial product development for products that target
14 rare diseases. An Orphan Products Board was estab-
15 lished to promote the development of drugs and de-
16 vices for rare diseases or disorders.

17 (5) Before 1983, some 38 orphan drugs had
18 been developed. Since the enactment of the Orphan
19 Drug Act, more than 220 new orphan drugs have
20 been approved and marketed in the United States
21 and more than 800 additional drugs are in the re-
22 search pipeline.

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

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

20 (2) increase the national investment in the de-
21 velopment of diagnostics and treatments for patients
22 with rare diseases and disorders.

1 **SEC. 3. NIH OFFICE OF RARE DISEASES AT NATIONAL IN-**
2 **STITUTES OF HEALTH.**

3 Title IV of the Public Health Service Act (42 U.S.C.
4 281 et seq.), as amended by Public Law 107–84, is
5 amended by inserting after section 404E the following:

6 “OFFICE OF RARE DISEASES

7 “SEC. 404F. (a) ESTABLISHMENT.—There is estab-
8 lished within the Office of the Director of NIH an office
9 to be known as the Office of Rare Diseases (in this section
10 referred to as the ‘Office’), which shall be headed by a
11 Director (in this section referred to as the ‘Director’), ap-
12 pointed by the Director of NIH.

13 “(b) DUTIES.—

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

16 “(A) The Director shall recommend an
17 agenda for conducting and supporting research
18 on rare diseases through the national research
19 institutes and centers. The agenda shall provide
20 for a broad range of research and education ac-
21 tivities, including scientific workshops and
22 symposia to identify research opportunities for
23 rare diseases.

24 “(B) The Director shall, with respect to
25 rare diseases, promote coordination and co-
26 operation among the national research insti-

1 tutes and centers and entities whose research is
2 supported by such institutes.

3 “(C) The Director, in collaboration with
4 the directors of the other relevant institutes and
5 centers of the National Institutes of Health,
6 may enter into cooperative agreements with and
7 make grants for regional centers of excellence
8 on rare diseases in accordance with section
9 404G.

10 “(D) The Director shall promote the suffi-
11 cient allocation of the resources of the National
12 Institutes of Health for conducting and sup-
13 porting research on rare diseases.

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

20 “(F) The Director shall biennially prepare
21 a report that describes the research and edu-
22 cation activities on rare diseases being con-
23 ducted or supported through the national re-
24 search institutes and centers, and that identi-
25 fies particular projects or types of projects that

1 should in the future be conducted or supported
2 by the national research institutes and centers
3 or other entities in the field of research on rare
4 diseases.

5 “(G) The Director shall prepare the NIH
6 Director’s annual report to Congress on rare
7 disease research conducted by or supported
8 through the national research institutes and
9 centers.

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

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

20 “(d) AUTHORIZATION OF APPROPRIATIONS.—For the
21 purpose of carrying out this section, there are authorized
22 to be appropriated such sums as already have been appro-
23 priated for fiscal year 2002, and \$4,000,000 for each of
24 the fiscal years 2003 through 2006.”.

1 **SEC. 4. RARE DISEASE REGIONAL CENTERS OF EXCEL-**
2 **LENCE.**

3 Title IV of the Public Health Service Act (42 U.S.C.
4 281 et seq.), as amended by section 3, is further amended
5 by inserting after section 404F the following:

6 “RARE DISEASE REGIONAL CENTERS OF EXCELLENCE

7 “SEC. 404G. (a) COOPERATIVE AGREEMENTS AND
8 GRANTS.—

9 “(1) IN GENERAL.—The Director of the Office
10 of Rare Diseases (in this section referred to as the
11 ‘Director’), in collaboration with the directors of the
12 other relevant institutes and centers of the National
13 Institutes of Health, may enter into cooperative
14 agreements with and make grants to public or pri-
15 vate nonprofit entities to pay all or part of the cost
16 of planning, establishing, or strengthening, and pro-
17 viding basic operating support for regional centers of
18 excellence for clinical research into, training in, and
19 demonstration of diagnostic, prevention, control, and
20 treatment methods for rare diseases.

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

25 “(b) COORDINATION WITH OTHER INSTITUTES.—
26 The Director shall coordinate the activities under this sec-

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

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

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

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

18 “(3) clinical research and demonstration pro-
19 grams.

20 “(d) PERIOD OF SUPPORT; ADDITIONAL PERIODS.—
21 Support of a center under subsection (a) may be for a
22 period of not to exceed 5 years. Such period may be ex-
23 tended by the Director for additional periods of not more
24 than 5 years if the operations of such center have been
25 reviewed by an appropriate technical and scientific peer

1 review group established by the Director and if such group
2 has recommended to the Director that such period should
3 be extended.

4 “(e) AUTHORIZATION OF APPROPRIATIONS.—For the
5 purpose of carrying out this section, there are authorized
6 to be appropriated such sums as already have been appro-
7 priated for fiscal year 2002, and \$20,000,000 for each of
8 the fiscal years 2003 through 2006.”.

