II

107TH CONGRESS
1ST SESSION

# S. 1379

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

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IN THE SENATE OF THE UNITED STATES

AUGUST 3, 2001

Mr. KENNEDY (for himself and Mr. HATCH) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

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# 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.

*Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,*

**SECTION 1. SHORT TITLE.**

This Act may be cited as the ''Rare Diseases Act of 2001''.

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

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

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

(2) For many years, the 25,000,000 Americans suffering from the over 6,000 rare diseases and disorders were denied access to effective medicines because prescription drug manufacturers could rarely make a profit from marketing drugs for such small groups of patients. The prescription drug industry did not adequately fund research into such treatments. Despite the urgent health need for these medicines, they came to be known as ''orphan drugs'' because no companies would commercialize them.

(3) During the 1970s, an organization called the National Organization for Rare Disorders (NORD) was founded to provide services and to lobby on behalf of patients with rare diseases and disorders. NORD was instrumental in pressing Con-

gress for legislation to encourage the development of orphan drugs.

(4) The Orphan Drug Act created financial incentives for the research and production of such orphan drugs. New federal programs at the National Institutes of Health and the Food and Drug Administration encouraged clinical research and commercial product development for products that target rare diseases. An Orphan Products Board was established to promote the development of drugs and devices for rare diseases or disorders.

(5) Before 1983, some 38 orphan drugs had been developed. Since the enactment of the Orphan Drug Act, more than 220 new orphan drugs have been approved and marketed in the United States and more than 800 additional drugs are in the research pipeline.

(6) Despite the tremendous success of the Orphan Drug Act, rare diseases and disorders deserve greater emphasis in the national biomedical research enterprise. The Office of Rare Diseases at the National Institutes of Health was created in 1993, but lacks a statutory authorization.

(7) The National Institutes of Health has received a substantial increase in research funding

from Congress for the purpose of expanding the national investment of the United States in behavioral and biomedical research.

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

(9) To redress this oversight, the Department of Health and Human Services has proposed the establishment of a network of regional centers of excellence for research on rare diseases.

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

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

(1) amend the Public Health Service Act to establish an Office of Rare Diseases at the National Institutes of Health; and

(2) increase the national investment in the development of diagnostics and treatments for patients with rare diseases and disorders.

# TITLE I—NATIONAL INSTITUTES OF HEALTH

**SEC. 101. NIH OFFICE OF RARE DISEASES.**

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

''OFFICE OF RARE DISEASES

''SEC. 404E. (a) ESTABLISHMENT.—There is established within the Office of the Director of NIH an office to be known as the Office of Rare Diseases (in this section referred to as the 'Office'), which shall be headed by a Director (in this section referred to as the 'Director'), appointed by the Director of NIH.

''(b) DUTIES.—

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

''(A) The Director shall recommend an agenda for conducting and supporting research on rare diseases through the national research institutes and centers. The agenda shall provide for a broad range of research and education activities, including scientific workshops and symposia to identify research opportunities for rare diseases.

"(B) The Director shall, with respect to rare diseases, promote coordination and cooperation among the national research institutes and centers and entities whose research is supported by such institutes.

"(C) The Director shall enter into cooperative agreements with and make grants for regional centers of excellence on rare diseases in accordance with section 404F.

"(D) The Director shall promote the sufficient allocation of the resources of the National Institutes of Health for conducting and supporting research on rare diseases.

"(E) The Director shall promote and encourage the establishment of a centralized clearinghouse for rare and genetic disease information that will provide understandable information about these diseases to the public, medical professionals, patients and families.

"(F) The Director shall biennially prepare a report that describes the research and education activities on rare diseases being conducted or supported through the national research institutes and centers, and that identifies particular projects or types of projects that

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

''(G) The Director shall prepare the NIH Director's annual report to Congress on rare disease research conducted by or supported through the national research institutes and centers.

''(2) PRINCIPAL ADVISOR REGARDING ORPHAN DISEASES.—With respect to rare diseases, the Director shall serve as the principal advisor to the Director of NIH and shall provide advice to other relevant agencies. The Director shall provide liaison with national and international patient, health and scientific organizations concerned with rare diseases.

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

''(d) AUTHORIZATION OF APPROPRIATIONS.—For the purpose of carrying out this section, there are authorized to be appropriated $4,000,000 for fiscal year 2002, and such sums as may be necessary for each subsequent fiscal year.''.

**SEC. 102. RARE DISEASE REGIONAL CENTERS OF EXCELLENCE.**

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

''RARE DISEASE REGIONAL CENTERS OF EXCELLENCE

''SEC. 404F. (a) COOPERATIVE AGREEMENTS AND GRANTS.—

''(1) IN GENERAL.—The Director of the Office of Rare Diseases (in this section referred to as the 'Director') shall enter into cooperative agreements with and make grants to public or private nonprofit entities to pay all or part of the cost of planning, establishing, or strengthening, and providing basic operating support for regional centers of excellence for clinical research into, training in, and demonstration of diagnostic, prevention, control, and treatment methods for rare diseases.

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

''(b) COORDINATION WITH OTHER INSTITUTES.—The Director shall coordinate the activities under this section with similar activities conducted by other national research institutes, centers and agencies of the National In-

stitutes of Health and by the Food and Drug Administration to the extent that such institutes, centers and agencies have responsibilities that are related to rare diseases.

''(c) USES FOR FEDERAL PAYMENTS UNDER COOPERATIVE AGREEMENTS OR GRANTS.—Federal payments made under a cooperative agreement or grant under subsection (a) may be used for—

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

''(2) clinical training, including training for allied health professionals, continuing education for health professionals and allied health professions personnel, and information programs for the public with respect to rare diseases; and

''(3) clinical research and demonstration programs.

''(d) PERIOD OF SUPPORT; ADDITIONAL PERIODS.—Support of a center under subsection (a) may be for a period of not to exceed 5 years. Such period may be extended by the Director for additional periods of not more than 5 years if the operations of such center have been reviewed by an appropriate technical and scientific peer review group established by the Director and if such group

has recommended to the Director that such period should be extended.

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

# TITLE II—FOOD AND DRUG ADMINISTRATION

**SEC. 201. GRANTS AND CONTRACTS FOR THE DEVELOPMENT OF ORPHAN DRUGS.**

Subsection (c) of section 5 of the Orphan Drug Act (21 U.S.C. 360ee(c)) is amended to read as follows:

''(c) For grants and contracts under subsection (a) there are authorized to be appropriated $25,000,000 for fiscal year 2002, and such sums as may be necessary for each subsequent fiscal year.''.

**SEC. 202. TECHNICAL AMENDMENT.**

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

(1) by striking '', of such certification,''; and

(2) by striking '', the issuance of the certification,''.

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