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RARE DISEASES ACT OF 2001

DECEMBER 18, 2001.—Ordered to be printed

Mr. KENNEDY, from the Committee on Health, Education, Labor,
and Pensions, submitted the following

R E P O R T

[To accompany S. 1379]

The Committee on Health, Education, Labor, and Pensions, to which was referred the bill (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, having considered the same, reports favorably thereon with an amendment and recommends that the bill (as amended) do pass.

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I. PURPOSE AND SUMMARY OF BILL

To address a longstanding unmet need to develop new treatments, diagnostics, and cures for rare diseases and disorders, Congress enacted the Orphan Drug Act of 1983 (Pub. L. 97-414). This Act created financial incentives, such as market exclusivity, tax credits, and research grants, for the research and production of orphan drugs, and established the Orphan Products Board at the Food and Drug Administration (FDA). Congress sought through the

Act to encourage the development of new “orphan” treatments, diagnostics, and cures for the millions of Americans with rare diseases who did not have access to effective medicines because prescription drug manufacturers were unlikely to develop and market drugs for such small groups of patients. The term “orphan drug” refers to a product that treats a rare disease or disorder affecting fewer than 200,000 Americans. Although the prevalence of each disease is rare, approximately 25 million Americans suffer from at least one of the 6,000 known rare diseases and disorders. Increasing our knowledge of these diseases deepens our understanding of the human body, and serves to benefit all Americans.

Before the Orphan Drug Act, approximately 38 drugs existed that were considered orphan drugs. Passage and implementation of the Orphan Drug Act has led to the development of over 220 treatments for rare diseases and disorders. However, patients with rare diseases or disorders face continuing challenges in receiving appropriate and adequate treatment. Despite dramatic increases in behavioral and biomedical research funding from Congress for the National Institutes of Health (NIH) since 1998, significant opportunities for rare disease research remain unmet. The Rare Diseases Act of 2001 (S. 1379) is intended to build on the successes of, and improve upon, current law in generating urgently needed treatments, diagnostics, and cures for rare diseases and disorders. The Act seeks to further stimulate the research and development of orphan drugs, by giving statutory authorization to the Office of Rare Diseases (ORD) at the NIH, and authorizing new funds for rare disease research centers of excellence and the FDA’s Orphan Products Grant program.

1. THE LEGISLATION AUTHORIZES THE ESTABLISHMENT OF AN OFFICE OF RARE DISEASES

The Office of Rare Diseases (ORD) was established in 1993 within the Office of the Director at the NIH to promote research and collaboration on orphan diseases and respond to requests for information. The ORD also has developed and maintains a centralized database on rare diseases. The office serves to bridge the gap between basic and translational research, and to stimulate new research on rare diseases.

The Rare Diseases Act of 2001 provides a statutory authorization for this important office. It authorizes the ORD to recommend a research agenda through the national research institutes and centers, and provide a broad range of research and education activities, including scientific workshops and symposia to identify research opportunities for rare diseases. In addition, the Director of the ORD shall promote coordination and cooperation among the national research institutes and collaboration among the directors of the relevant institutes and centers of NIH. The Act doubles the authorization for the ORD’s budget to \$4 million for FY02 and such sums as may be necessary for each subsequent fiscal year.

2. THE LEGISLATION AUTHORIZES REGIONAL CENTERS OF EXCELLENCE FOR RARE DISEASE RESEARCH AND TRAINING

The Act authorizes the NIH to make cooperative agreements with, and make grants to, public or private nonprofit entities for regional centers of excellence for clinical research into, training in,

and demonstration of diagnostic, prevention, control, and treatment methods for rare diseases. It authorizes \$20 million for FY02 and such sums as may be necessary for each subsequent fiscal year. Support of a center may not exceed 5 years, though extensions of not more than 5 years may be granted.

3. THE LEGISLATION INCREASES FUNDING FOR THE ORPHAN PRODUCT RESEARCH GRANT PROGRAM

The Act doubles the funding authorization for the FDA's Orphan Product Research Grant program from \$12.5 million currently to \$25 million for fiscal year 2002 and such sums as may be necessary for each subsequent fiscal year. This will provide crucial additional support for clinical research on new treatments for rare diseases and disorders.

4. THE LEGISLATION INCLUDES A TECHNICAL AMENDMENT TO THE FEDERAL FOOD, DRUG, AND COSMETIC ACT

The Act includes a technical amendment to section 527(a) of the Federal Food, Drug, and Cosmetic Act.

II. BACKGROUND AND NEED FOR THE LEGISLATION

Prior to the passage of the Orphan Drug Act in 1983, Americans suffering from any of the 6,000 known rare diseases largely did not have access to effective medicines because prescription drug manufacturers did not have adequate incentives to develop or market drugs for such small groups of patients. Patients with rare diseases, such as Lou Gehrig's disease and urea cycle disorders, went without treatment or hope for a cure. Despite the need for these medicines, they came to be known as "orphan drugs" because companies would not or could not commercialize them.

The Orphan Drug Act provided seven years of market exclusivity and expanded tax credits to companies for the development and marketing of orphan drugs. The Act also established the Office of Orphan Product Development at the Food and Drug Administration, and created the Orphan Products Grant Program, providing financial incentives to small companies and research grants to academic scientists to support pivotal clinical trials, research, and production of orphan drugs, biologics, and devices. New programs at the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) were created to encourage clinical research and commercial product development of products that target rare diseases. Since 1983, the Orphan Drug Act has been amended five times to clarify terms, improve the implementation and effectiveness of the Act, and convene a National Commission on Orphan Diseases to assess the activities of public and private agencies related to rare diseases.

The Orphan Drug Act is widely recognized as having successfully encouraged greater research and the development of new treatments of rare diseases. This has had substantial benefits for the health and well-being of patients, improving their quality of life and allowing them to return to school or work, while reducing long-term health care costs. In the decade before 1983, less than ten orphan drugs had been marketed; since 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 under development.

The FDA's Orphan Products Research Grants generally support small clinical trials at academic institutions throughout the nation to develop the preliminary evidence that is necessary to attract commercial sponsors. It is a model of successful government-industry partnership, and fills a major gap between academic research and the private sector. The grants help to create lifesaving products that are needed throughout the world. Drug treatments for sickle-cell anemia and narcolepsy are on the market today only because these grants supported some of their research.

The creation of the Office of Rare Diseases (ORD) in 1993 formalized a mechanism within the NIH to stimulate research, bridge the gap between basic and translational research, and encourage the exchange of ideas among investigators, voluntary patient support groups and NIH staff. In 1995, recognizing a need to enhance ORD's operations, Senator Mark Hatfield introduced the Office for Rare Disease Research Act, a bill to formally establish this office at the NIH. This bill passed through the Senate, but was not enacted into law.

The Orphan Drug Act both laid the foundation for, and initiated a major leap in, our understanding and treatment of rare diseases. Yet, more needs to be done. Without statutory authorization, the Office of Rare Diseases and its mission have received less attention than an NIH office might otherwise receive from the biomedical research community. While the NIH has received a substantial increase in funding from Congress for the purpose of expanding the national investment of the United States in behavioral and biomedical research, significant opportunities for rare disease research remain unmet.

Moreover, although the Orphan Products Grant Program continues to attract research proposals, the funding for this program is insufficient. Today, about 100 grant applications are received annually, but those that would otherwise be funded are not funded because of limited resources. The investment necessary for research and development of new drugs and devices is large in comparison to the size of the potential market for a rare disease. For example, only 30,000 people suffer from cystic fibrosis, and 600 from cystinosis; yet, for each of these people, the need for treatment is urgent. Investing in research on rare diseases is an important health priority. When the Orphan Drug Act passed in 1983, only a few thousand people had been diagnosed with AIDS. According to former Health and Human Services Secretary Louis Sullivan, the response to the HIV/AIDS pandemic, in terms of research and drug development, was likely enhanced by the incentives authorized by the Orphan Drug Act.

The Rare Diseases Act of 2001, which was introduced in conjunction with legislation to expand the orphan drug tax credit (S. 1341), addresses current obstacles in combating rare diseases by providing statutory authorization and increasing funding for the ORD to promote research and develop our understanding and treatment of rare diseases; authorizing \$20 million for regional centers of excellence to further stimulate research and diagnose diseases; and increasing funding for the Orphan Products Research Grant pro-

gram to \$25 million to fund clinical trials that translate research into treatment and cures.

III. LEGISLATIVE HISTORY AND COMMITTEE ACTION

On August 3rd, 2001, Senator Kennedy and Senator Hatch introduced S. 1379, the “Rare Diseases Act of 2001.” The bill was referred to the Senate Committee on Health, Education, Labor, and Pensions.

On October 16, 2001, the committee held an executive session to consider S. 1379, and ordered the bill to be reported favorably with an amendment in the nature of a substitute. The committee approved S. 1379, as amended, by unanimous voice vote.

IV. EXPLANATION OF THE LEGISLATION AND COMMITTEE VIEWS

The committee recognizes that approximately 25 million Americans suffer from rare diseases every day. The pain and suffering from these diseases are compounded by well-grounded concerns that research on, and the ongoing development of, new cures and treatments for rare diseases is limited and needs to be accelerated.

Statutory authorization for the Office of Rare Disorders

Before the creation of the Office of Rare Diseases (ORD) in 1993, information regarding rare diseases was spread throughout the NIH based upon disease type and classification. As many rare diseases affect multiple systems in the human body, funding and responsibility often was diffused across different institutes. In 1989, the National Commission on Orphan Diseases, recommended that Congress establish a “Central Office of Orphan and Rare Diseases” at the NIH. In response to concerns raised by the Congress and patient advocates, the Director of the NIH created the ORD to develop a research agenda and clinical database to promote orphan drug product research and development.

In January 2001, the NIH issued a report on steps to expand and coordinate rare disease research, which included recommendations of an NIH Special Emphasis Panel (SEP) on the coordination of this research. The SEP recommended that the NIH “establish a permanent presence for the ORD and its activities at the NIH.” The committee supports this recommendation. The Rare Diseases Act of 2001 provides the ORD with increased funding and statutory authorization to help meet these goals.

Grants for Centers of Excellence

The NIH Special Emphasis Panel also recommended that “the NIH should support the establishment of Specialized Research and Diagnostic Centers of Excellence for Rare Diseases to stimulate research and aid in the diagnosis of rare diseases.” The Rare Diseases Act of 2001 authorizes the creation of, and \$20 million in funding for, such regional centers of excellence. The committee supports this provision, which was proposed in the Administration’s FY 2002 budget request. Regional centers of excellence provide critical opportunities for research and collaboration throughout the country, which are of particular importance to the study and treatment of rare diseases where researchers may not be aware of each others’ efforts. These centers would also help patients receive an

accurate diagnosis, which often takes years. In 1989, the National Commission on Orphan Diseases estimated that only a third of patients receive an accurate diagnosis in the 3 to 5 years after the onset of symptoms; 15 percent of the population is not accurately diagnosed until 7 or more years after the onset of symptoms.

Orphan Products Grant Program

The FDA's Orphan Products Grant Program plays a critical role in securing commercial sponsors for developing drugs. The grants fund small clinical trials at academic institutions throughout the nation to develop preliminary evidence, and are a model of a successful government-industry partnership. However, the funding for this program has not met the demand created by qualified proposals. For these reasons, the committee supports the provision in the Rare Diseases Act of 2001 that increases the funding for the Orphan Products Grant Program from \$12.5 million to \$25 million for fiscal year 2002 and such sums as may be necessary for each subsequent fiscal year. This will provide needed additional support for clinical research on new treatments for rare diseases and disorders.

V. COST ESTIMATE

U.S. CONGRESS,
CONGRESSIONAL BUDGET OFFICE,
Washington, DC, December 5, 2001.

Hon. EDWARD M. KENNEDY,
*Chairman, Committee on Health, Education, Labor, and Pensions,
U.S. Senate, Washington, DC.*

DEAR MR. CHAIRMAN: The Congressional Budget Office has prepared the enclosed estimate of S. 1379, the Rare Diseases Act of 2001.

If you wish further details on this estimate, we will be pleased to provide them. The CBO staff contact is Christopher J. Topoleski.
Sincerely,

BARRY B. ANDERSON
(For Dan L. Crippen, Director).

Enclosure.

CONGRESSIONAL BUDGET OFFICE COST ESTIMATE

S. 1379—Rare Diseases Act of 2001

Summary: S. 1379 would provide for a statutory authorization of the Office of Rare Diseases (ORD) within the Office of the Director of the National Institutes of Health (NIH). The bill would authorize NIH to provide grants to regional "Centers of Excellence" to conduct research and training related to rare diseases and it would authorize funding for an existing grant program administered by the Food and Drug Administration (FDA) that sponsors clinical testing of the safety and effectiveness of new products to treat or diagnose rare diseases.

CBO estimates that implementing S. 1379 would cost \$6 million in 2002 and \$190 million over the 2002–2006 period, assuming the appropriation of the necessary amounts including annual adjustments for inflation. (The five-year total would be \$181 million if

such inflation adjustments are not made.) The legislation would not affect direct spending or receipts; therefore, pay-as-you-go procedures would not apply.

S. 1379 contains no intergovernmental or private sector mandates as defined in the Unfunded Mandates Reform Act (UMRA). State, local, and tribal governments could apply for and receive grants authorized by the bill, and any costs they incur would be voluntary.

Estimated cost to the Federal Government: The estimated budgetary impact of S. 1379 is shown in the following table. The costs of this legislation fall within budget function 550 (health).

	By fiscal year in millions of dollars—				
	2002	2003	2004	2005	2006
CHANGES IN SPENDING SUBJECT TO APPROPRIATION ¹					
Estimated Authorization Level:					
National Institutes of Health ²	24	25	25	26	27
Food and Drug Administration ³	12	26	27	28	28
Total	36	51	52	54	55
Estimated Outlays:					
National Institutes of Health	4	14	23	25	27
Food and Drug Administration	2	16	26	27	26
Total	6	30	49	52	53

¹The amounts shown reflect adjustments for anticipation for those activities for which the bill would authorize such sums as necessary. Without such inflation adjustments, the five-year changes in authorization levels would total \$232 million (instead of \$248 million) and the changes in outlays would total \$181 million (instead of \$190 million).

²The 2002–2006 levels are CBO baseline projections, including adjustments for anticipated inflation, for the NIH.

³Estimated authorization level includes the incremental change from amounts appropriated thus far for 2002 for the FDA. The 2003–2006 levels are CBO baseline projections of the change in FDA authorization adjustments for anticipated inflation.

Basis of estimate: The bill would provide statutory authorization for the ORD, which was established in 1993 within the Office of the Director at the NIH. The ORD was established to respond to the reporting requirements of the Orphan Drug Act, to implement the recommendations of the National Commission on Orphan Diseases, and to respond to requests for information on rare diseases. A rare disease is one which typically affects fewer than 200,000 individuals in the United States.

The bill would require ORD to promote the establishment of a centralized clearinghouse to make data and information on rare diseases available to the public, researchers, and clinicians. The director of the ORD would be responsible for preparing biennial and annual reports on the activities of the office, its Centers for Excellence, and future research opportunities.

The bill would authorize NIH to award grants and contracts to public and nonprofit private entities known as Centers of Excellence to conduct clinical research, training, diagnostic prevention, control, and or treatment activities with respect to rare diseases. The centers would be awarded renewable contracts for up to five years for each contract period.

S. 1379 would authorize appropriation of \$24 million for those activities in 2002 and such sums as necessary for each subsequent year. CBO estimates that implementing the provisions affecting NIH would cost \$2 million in 2002 and \$97 million over the 2002–2006 period, assuming appropriations of the necessary amounts.

S. 1379 also would authorize funding for an existing grant program administered by FDA that sponsors clinical studies on the safety and effectiveness of new products to treat or diagnose rare diseases. The amount appropriated thus far for fiscal year 2002 for the current program is \$13 million. The bill would authorize the appropriation of \$25 million in 2002 and such sums as necessary for each subsequent fiscal year.

Research grants awarded under the program would defray some of the costs associated with clinical testing of certain orphan drugs, biological, medical devices, and medical foods. An orphan drug is a drug or biological that is used to treat or diagnose an illness usually affecting fewer than 200,000 people in the United States. Eligible medical devices and medical foods include products for which there is no reasonable expectation development without grant assistance because the condition occurs relatively infrequently in the United States.

CBO estimates that implementing this provision for FDA grants would cost an additional \$4 million in 2002 and \$93 million over the 2002–2006 period, assuming appropriation of the necessary amounts.

Pay-as-you-go considerations: None.

Estimated impact on state, local, and tribal governments: S. 1379 contains no intergovernmental mandates as defined in UMRA. State, local, and tribal governments could apply for and receive grants authorized by the bill, and any costs they incur would be voluntary.

Estimated impact on the private sector: The bill contains no private-sector mandates as defined in UMRA.

Estimates prepared by: Federal Costs: Christopher J. Topoleski and Julia Christensen. Impact on State, Local, and Tribal Governments: Leo Lex. Impact on the Private Sector: Jennifer Bowman.

Estimate approved by: Peter H. Fontaine, Deputy Assistant Director for Budget Analysis.

VI. APPLICATION OF LAW TO THE LEGISLATIVE BRANCH

The Rare Diseases Act of 2001 authorizes and amends sections of the Public Health Service Act and the Federal Food, Drug, and Cosmetic Act. As such, it has no application to the legislative branch.

VII. REGULATORY IMPACT STATEMENT

The committee has determined that this bill is unlikely to impose any new increases in the regulatory burden of paperwork.

VIII. SECTION-BY-SECTION ANALYSIS

Section 1. Short title

This section provides the short title of the bill, the “Rare Diseases Act of 2001.”

Section 2. Findings and purposes

Subsection (a) Findings. This subsection finds that: (1) rare diseases affect small patient populations, typically less than 200,000; (2) treatments for rare diseases were not researched and developed

by prescription drug manufacturers in the past because the industry could rarely make a profit from marketing such “orphan drugs” to small groups of patients; (3) the National Organization for Rare Disorders (NORD) was instrumental in pressing Congress for legislation to encourage the development of orphan drugs; (4) The Orphan Drug Act and new federal programs at NIH and FDA encouraged the development of drugs for rare diseases; (5) since enactment of the Orphan Drug Act, more than 220 new orphan drugs have been approved and more than 800 other such drugs are in the research pipeline; (6) rare diseases deserve greater emphasis and the Office of Rare Diseases at NIH, created in 1993, lacks statutory authorization; (7) NIH has received substantial increases in research funding; (8) funding for rare disease at NIH has not increased appreciably; (9) to redress this oversight, HHS has proposed establishing a network of regional centers of excellence for research on rare diseases; (10) the Orphan Products Research Grants funded by FDA have led to the development of 23 drugs and 4 medical devices for rare diseases, yet the appropriation in FY2001 for such grants was less than in FY1995.

Subsection (b) Purposes. This subsection states that the purposes of the Act are to: (1) amend the Public Health Service Act to establish an Office of Rare Diseases at NIH; and, (2) increase the national investment in the development of diagnostics and treatments for patients with rare diseases.

TITLE I—NATIONAL INSTITUTES OF HEALTH

Section 101. NIH Office of Rare Diseases

The act adds a new section 404E to Title IV of the Public Health Service Act. The new section 404E establishes, within the Office of the Director of NIH, the Office of Rare Diseases which would be headed by a Director appointed by the Director of NIH.

The Director of the Office of Rare Diseases would recommend an agenda for conducting and supporting research on rare diseases, including scientific workshops and symposia. The Director would promote coordination and cooperation on the topic of rare diseases research among the national research institutes and centers and entities whose research is supported by such institutes. The Director, in collaboration with the directors of the other relevant institutes and centers of the NIH, would enter into cooperative agreements with and make grants for regional centers of excellence on rare diseases. Sufficient allocation of NIH resources for research on rare diseases would be promoted by the Director. A centralized clearinghouse for rare and genetic disease information would be established to provide information about these diseases to the public and medical professionals. The Director would 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 these institutes and centers, or other entities in the field of research on rare diseases. An annual report to Congress would be prepared on rare disease research conducted or supported by NIH. The Director of the Office of Rare Diseases would serve as the

principal advisor to the Director of NIH on the topic of rare diseases and would serve as a liaison with outside organizations.

Rare diseases are defined as any disease or condition that affects less than 200,000 persons in the United States.

An appropriation of \$4,000,000 is authorized for fiscal year 2002 and such sums as may be necessary for subsequent fiscal years.

Section 102. Rare Disease Regional Centers of Excellence

The act adds a new section 404F to Title IV of the Public Health Service Act. The new section 404F establishes the Rare Disease Regional Centers of Excellence.

The Director of the Office of Rare Diseases, along with the Directors of other relevant NIH institutes and centers, would enter into cooperative agreements and make grants to public and private non-profit entities to plan, establish, or strengthen, and provide basic operating support for regional centers of excellence for clinical research on rare diseases. Such clinical research would include training in and demonstration of diagnostic, prevention, control, and treatment methods for rare diseases.

The Director of the Office of Rare Disease would coordinate the activities under section 404F with similar activities conducted by the other national research institutes, centers, and agencies of the NIH and by the FDA to the extent that such institutes, centers, and agencies have responsibilities that are related to rare diseases.

Federal payments made under such cooperative agreements or grants may be used for staffing, administrative, and other basic operating costs including such patient care costs as are required for research. Federal payments may also be used for clinical training, continuing education for health professionals, and information programs for the public on rare diseases, as well as clinical research and demonstration programs.

Support of such centers would not exceed a period of 5 years. The Director may extend support for additional periods of not more than 5 years if the operations of such a center have been peer reviewed by an appropriate technical and scientific group established by the Director, and the group has recommended that support should be extended.

An appropriation of \$20,000,000 is authorized for fiscal year 2002 and such sums as may be necessary for subsequent fiscal years.

TITLE II—FOOD AND DRUG ADMINISTRATION

Section 201. Grants and contracts for the development of orphan drugs

The act amends subsection (c) of section 5 of the Orphan Drug Act, authorizing \$25,000,000 for fiscal year 2002 for grants and contracts, and such sums as may be necessary for subsequent fiscal years.

Section 202. Technical amendment

In section 527(a) of the Federal Food, Drug, and Cosmetic Act, two phrases containing the word “certification” are struck from the matter following paragraph (2).

IX. CHANGES IN EXISTING LAW

In compliance with rule XXVI paragraph 12 of the Standing Rules of the Senate, the following provides a print of the statute or the part or section thereof to be amended or replaced (existing law proposed to be omitted is enclosed in black brackets, new matter is printed in italic, existing law in which no change is proposed is shown in roman):

PUBLIC HEALTH SERVICE ACT

* * * * *

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, in collaboration with the directors of the other relevant institutes and centers of the National Institutes of Health, 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 disease 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.*

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, in collaboration with the directors of the other relevant institutes and centers of the National Institutes of Health, 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 Institutes 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.*

(3) *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.

SECTION 5 OF THE ORPHAN DRUG ACT

GRANTS AND CONTRACTS FOR DEVELOPMENT OF DRUGS FOR RARE DISEASES AND CONDITIONS

SEC. 5. [21 U.S.C. 360ee] (a) * * *

* * * * *

[(c) For grants and contracts under subsection (a) there are authorized to be appropriated \$10,000,000 for fiscal year 1988, \$12,000,000 for fiscal year 1989, \$14,000,000 for fiscal year 1990.]

(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.*

* * * * *

FEDERAL FOOD, DRUG, AND COSMETIC ACT

* * * * *

PROTECTION FOR DRUGS FOR RARE DISEASES OR CONDITIONS

SEC. 527. [21 U.S.C. 360cc] (a) Except as provided in subsection (b), if the Secretary—

(1) * * *

* * * * *

for a drug designated under section 526 for a rare disease or condition, the Secretary may not approve another application under section 505 or issue another license under section 351 of the Public Health Service Act for such drug for such disease or condition for a person who is not the holder of such approved application[, of such certification,] or of such license until the expiration of seven years from the date of the approval of the approved application[, the issuance of the certification,] or the issuance of the license. Section 505(c)(2) does not apply to the refusal to approve an application under the preceding sentence.

* * * * *

