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107TH CONGRESS, 2d Session | HOUSE OF REPRESENTATIVES | REPORT 107–543

# RARE DISEASES ACT OF 2002

JUNE 26, 2002.—Committed to the Committee of the Whole House on the State of the Union and ordered to be printed

Mr. TAUZIN, from the Committee on Energy and Commerce, submitted the following

## R E P O R T

[To accompany H.R. 4013]

The Committee on Energy and Commerce, to whom was referred the bill (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, having considered the same, report favorably thereon without amendment and recommend that the bill do pass.

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## PURPOSE AND SUMMARY

The purpose of H.R. 4013, the Rare Diseases Act of 2002, is to authorize in statute the Office of Rare Diseases at the National Institutes of Health ("NIH"). Rare diseases are diseases affecting fewer than 200,000 individuals in the United States. The Director of the Office of Rare Diseases will serve as the principal advisor to

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the Director of the NIH with respect to rare diseases, and shall serve to promote sufficient allocation of NIH resources to rare disease research, and promote and encourage the establishment of a centralized clearinghouse for rare disease information for the benefit of the public, medical professionals, patients, and families.

## BACKGROUND AND NEED FOR LEGISLATION

In 1993, the NIH created an Office of Rare Diseases within the Office of the Director of NIH, to respond to the reporting requirements of the Orphan Drug Act of 1983. The Office of Rare Diseases implements the recommendations of the National Commission on Orphan Diseases, and responds to requests for information on rare diseases.

Rare, or Orphan, Diseases affect fewer than 200,000 individuals in America. Presently, more than 6,000 rare diseases affecting more than 25 million Americans are known. Prior to the enactment of the Orphan Disease Act of 1983, there was not enough of an incentive for drug and biological manufacturers to invest in treatments for these diseases. The Orphan Drug Act created greater incentives through increased exclusivity (7 years) for drugs and biologicals which treat Orphan Diseases, among other things.

While the Orphan Drug Act has achieved dramatic increases in research into, and treatments for, rare diseases, more still needs to be done. One positive step includes authorizing in statute the Office of Rare Diseases. This action sends the strong signal of the Congress' commitment for both this Office as well as for rare disease research generally. Also, authorizing regional centers of excellence for rare disease research is a positive step, as it will enable the NIH to select sites to concentrate on finding cures and treatment methods for rare diseases. Both of these proposals are contained within H.R. 4013.

## HEARINGS

The Subcommittee on Health held a hearing on "The National Institutes of Health: Investing in Research to Prevent and Cure Disease" on June 6, 2002. The Subcommittee received testimony from Claude Lenfant, M.D., Director, National Heart, Lung, and Blood Institute; Audrey S. Penn, M.D., Acting Director, National Institute of Neurological Disorders and Stroke; Robert O. Bonow, M.D., President-elect, American Heart Association; Eric Hargis, President and CEO, The Epilepsy Foundation; Edward Sanchez, M.D., M.P.H., Commissioner, Texas Department of Health; Daniel Jones, M.D., Vice Chancellor, University Medical Center, University of Mississippi.

## COMMITTEE CONSIDERATION

On Wednesday, June 19, 2002, the Full Committee met in open markup session and favorably ordered reported H.R. 4013, the Rare Diseases Act of 2002, by voice vote, a quorum being present.

## COMMITTEE VOTES

Clause 3(b) of rule XIII of the Rules of the House of Representatives requires the Committee to list the record votes on the motion

to report legislation and amendments thereto. There were no record votes taken in connection with ordering H.R. 4013 reported. A motion by Mr. Tauzin to order H.R. 4013 reported to the House, without amendment, was agreed to by a voice vote.

## COMMITTEE OVERSIGHT FINDINGS

Pursuant to clause 3(c)(1) of rule XIII of the Rules of the House of Representatives, the Committee held an oversight hearing and made findings that are reflected in this report.

## STATEMENT OF GENERAL PERFORMANCE GOALS AND OBJECTIVES

The objective of this legislation is to increase support for rare disease research within and without the National Institutes of Health through the statutory authorization of the Office of Rare Diseases and by allowing the Secretary to establish rare disease regional centers of excellence.

## NEW BUDGET AUTHORITY, ENTITLEMENT AUTHORITY, AND TAX EXPENDITURES

In compliance with clause 3(c)(2) of rule XIII of the Rules of the House of Representatives, the Committee finds that H.R. 4103, would result in no new or increased budget authority, entitlement authority, or tax expenditures or revenues.

## COMMITTEE COST ESTIMATE

The Committee adopts as its own the cost estimate prepared by the Director of the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974, which is included in the report to accompany H.R. 4984.

## CONGRESSIONAL BUDGET OFFICE ESTIMATE

Pursuant to clause 3(c)(3) of rule XIII of the Rules of the House of Representatives, the cost estimate provided by the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974 is included in the report to accompany H.R. 4984.

## FEDERAL MANDATES STATEMENT

The Committee adopts as its own the estimate of Federal mandates prepared by the Director of the Congressional Budget Office pursuant to section 423 of the Unfunded Mandates Reform Act. The estimate is included in the report to accompany H.R. 4984.

## ADVISORY COMMITTEE STATEMENT

No advisory committees within the meaning of section 5(b) of the Federal Advisory Committee Act were created by this legislation.

## CONSTITUTIONAL AUTHORITY STATEMENT

Pursuant to clause 3(d)(1) of rule XIII of the Rules of the House of Representatives, the Committee finds that the Constitutional authority for this legislation is provided in Article I, section 8, clause 3, which grants Congress the power to regulate commerce with foreign nations, among the several States, and with the Indian tribes.

## APPLICABILITY TO LEGISLATIVE BRANCH

The Committee finds that the legislation does not relate to the terms and conditions of employment or access to public services or accommodations within the meaning of section 102(b)(3) of the Congressional Accountability Act.

## SECTION-BY-SECTION ANALYSIS OF THE LEGISLATION

*Section 1. Short Title*

Section 1 establishes that the short title for this legislation is the Rare Diseases Act of 2002.

*Section 2. Findings and Purposes*

Section 2 contains Congressional findings.

*Section 3. NIH Office of Rare Diseases at National Institutes of Health*

Section 3 establishes within the Office of the Director of the NIH an office to be known as the Office of Rare Diseases, and ensures that the Director of the Office of Rare Diseases will (1) recommend an agenda for conducting and supporting research on rare diseases; (2) promote, with respect to rare diseases, coordination and cooperation among NIH institutes and centers; and, (3) promote the sufficient allocation of the resources of the NIH for conducting and supporting research on rare diseases. The section also authorizes $4 million in each of Fiscal Years 2003 through 2006 for these purposes.

*Section 4. Rare Disease Regional Centers of Excellence*

Section 4 empowers the Director of the Office of Rare Diseases, in collaboration with the directors of the other relevant institutes and centers of the NIH, to enter into cooperative agreements and make grants for the establishment of regional centers of excellence for clinical research into, training in, and demonstration of diagnostic, prevention, control, and treatment methods for rare diseases. This section authorizes $20 million in each of Fiscal Years 2003 through 2006 for these purposes.

## CHANGES IN EXISTING LAW MADE BY THE BILL, AS REPORTED

In compliance with clause 3(e) of rule XIII of the Rules of the House of Representatives, changes in existing law made by the bill, as reported, are shown as follows (new matter is printed in italic and existing law in which no change is proposed is shown in roman):

# PUBLIC HEALTH SERVICE ACT

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## TITLE IV—NATIONAL RESEARCH INSTITUTES

### PART A—NATIONAL INSTITUTES OF HEALTH

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*OFFICE OF RARE DISEASES*

*SEC. 404F. (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, may enter into cooperative agreements with and make grants for regional centers of excellence on rare diseases in accordance with section 404G.*

*(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 such sums as already have been appropriated for fiscal year 2002, and $4,000,000 for each of the fiscal years 2003 through 2006.*

*RARE DISEASE REGIONAL CENTERS OF EXCELLENCE*

*SEC. 404G. (a) COOPERATIVE AGREEMENTS AND GRANTS.—*

*(1) IN GENERAL.—The Director of the Office of Rare Diseases (in this section referred to as the "Director"), in collaboration with the directors of the other relevant institutes and centers of the National Institutes of Health, may 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.*

*(e) AUTHORIZATION OF APPROPRIATIONS.—For the purpose of carrying out this section, there are authorized to be appropriated such sums as already have been appropriated for fiscal year 2002, and $20,000,000 for each of the fiscal years 2003 through 2006.*

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