

RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF  
2002

---

OCTOBER 1, 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. 4014]

[Including cost estimate of the Congressional Budget Office]

The Committee on Energy and Commerce, to whom was referred the bill (H.R. 4014) to amend the Federal Food, Drug, and Cosmetic Act with respect to the development of products for rare diseases, having considered the same, report favorably thereon without amendment and recommend that the bill do pass.

CONTENTS

	Page
Purpose and Summary .....	1
Background and Need for Legislation .....	2
Hearings .....	2
Committee Consideration .....	2
Committee Votes .....	2
Committee Oversight Findings .....	2
Statement of General Performance Goals and Objectives .....	3
New Budget Authority, Entitlement Authority, and Tax Expenditures .....	3
Committee Cost Estimate .....	3
Congressional Budget Office Estimate .....	3
Federal Mandates Statement .....	5
Advisory Committee Statement .....	5
Constitutional Authority Statement .....	5
Applicability to Legislative Branch .....	5
Section-by-Section Analysis of the Legislation .....	5
Changes in Existing Law Made by the Bill, as Reported .....	5

PURPOSE AND SUMMARY

H.R. 4014, the Rare Diseases Orphan Product Development Act, reauthorizes the grant program originally established under the Orphan Drug Act through FY 2006. Grants awarded pursuant to

this Act are used by researchers to investigate therapies to treat rare diseases, which are diseases affecting fewer than 200,000 individuals in the United States.

#### BACKGROUND AND NEED FOR LEGISLATION

An “orphan drug” is a drug intended to treat a rare disease. Prior to 1983, few manufacturers sought to develop cures and treatments for these diseases due to the limited return on investment of such therapies. To respond to the fact that few drugs were developed to treat rare diseases, Congress passed the Orphan Drug Act, which created various incentives for developing orphan drugs. Included in the Act were provisions which provided a seven year exclusivity period (instead of the normal five year exclusivity period granted by the Food and Drug Administration for new chemical entities) for orphan drugs; a grant program which funds researcher for developing cures and therapies for orphan diseases; and tax incentives for such researchers.

There are more than 6,000 rare diseases (diseases which affect fewer than 200,000 individuals in the United States) affecting roughly 25 million individuals in the United States. Prior to 1983, only 38 orphan drugs had been developed to treat orphan diseases. Since the Orphan Drug Act was passed, more than 220 orphan drugs have been approved and marketed in the United States.

H.R. 4014 amends the Orphan Drug Act by reauthorizing the Orphan Products Research Grant program contained in the Act at the amounts already-appropriated in this year, and at \$25 million per year for the Fiscal Years 2003–2006. The new monies authorized under this program will result in more clinical trials for therapies for orphan diseases to be funded.

#### HEARINGS

The Committee on Energy and Commerce has not held hearings on the legislation.

#### COMMITTEE CONSIDERATION

On Thursday, September 5, the Full Committee met in open markup session and without objection, ordered H.R. 4014 favorably reported to the House, without amendment, by unanimous consent, 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. 4014 reported. A motion by Mr. Tauzin to order H.R. 4014 reported to the House, without amendment, was agreed to by unanimous consent.

#### COMMITTEE OVERSIGHT FINDINGS

Pursuant to clause 3(c)(1) of rule XIII of the Rules of the House of Representatives, the Committee has not held oversight or legislative hearings on this legislation.

## STATEMENT OF GENERAL PERFORMANCE GOALS AND OBJECTIVES

The goals of the legislation are to provide for more treatments and cures for rare diseases by funding more researchers who are conducting clinical trials designed to discover such cures and treatments.

## 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. 4014, the Rare Diseases Orphan Product Development Act of 2002, 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.

## CONGRESSIONAL BUDGET OFFICE ESTIMATE

Pursuant to clause 3(c)(3) of rule XIII of the Rules of the House of Representatives, the following is the cost estimate provided by the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974:

U.S. CONGRESS,  
CONGRESSIONAL BUDGET OFFICE,  
*Washington, DC, September 27, 2002.*

Hon. W.J. "BILLY" TAUZIN,  
*Chairman, Committee on Energy and Commerce,  
House of Representatives, Washington, DC.*

DEAR MR. CHAIRMAN: The Congressional Budget Office has prepared the enclosed estimate of H.R. 4014, the Rare Diseases Orphan Product Development Act of 2002.

If you wish further details on this estimate, we will be pleased to provide them. The CBO staff contact is Julia M. Christensen.

Sincerely,

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

Enclosure.

*H.R. 4014—Rare Diseases Orphan Product Development Act of 2002*

Summary: H.R. 4014 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.

The bill would authorize the appropriation of \$25 million a year for fiscal years 2003 through 2006. CBO estimates that implementing H.R. 4014 would cost \$8 million in 2003 and \$93 million over the 2003–2007 period, assuming the appropriation of the authorized amounts. The legislation would not affect direct spending or receipts; therefore, pay-as-you-go procedures would not apply.

H.R. 4014 contains no intergovernmental or private-sector mandates as defined in the Unfunded Mandates Reform Act (UMRA). State, local, and tribal governments could enter into contracts 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 H.R. 4014 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	2007
CHANGES IN SPENDING SUBJECT TO APPROPRIATION						
Authorization Level .....	0	25	25	25	25	0
Estimated Outlays .....	0	8	21	23	24	17

**Basis of estimate:** H.R. 4014 would authorize funding for an existing grant program administered by the FDA that sponsors clinical studies on the safety and effectiveness of new products to treat or diagnose rare diseases. The amount appropriated for fiscal year 2002 for the current program is \$13 million. The bill would authorize the appropriation of such sums as already have been appropriated for fiscal year 2002, and \$25 million for each of the fiscal years 2003 through 2006.

Research grants awarded under the program would defray some of the costs associated with clinical testing of certain orphan drugs, biologicals, 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 of development without grant assistance because the condition occurs relatively infrequently in the United States.

CBO estimates that implementing H.R. 4014 would cost \$8 million in 2003 and \$93 million over the 2003–2007 period, assuming appropriation of the necessary amounts. This estimate incorporates general spending patterns for research grant programs administered within the Public Health Service.

Pay-as-you-go considerations: None.

**Estimated impact on state, local, and tribal governments:** H.R. 4014 contains no intergovernmental mandates as defined in UMRA. State, local, and tribal governments enter into contracts 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.

**Previous estimate:** On December 5, 2001, CBO transmitted a cost estimate for S. 1379, the Rare Diseases Act of 2001, as ordered reported by the Senate Committee on Health, Education, Labor, and Pensions on November 1, 2002. S. 1379 contains a provision very similar to H.R. 4014 that funds FDA's grant program for the development of orphan products. The main difference between the provision in the two bills is that S. 1379 would authorize the appropriation of \$25 million in 2002 and such sums as necessary for each subsequent year while H.R. 4014 would authorize such sums as al-

ready have been appropriated for fiscal year 2002, and \$25 million in funding each year from 2003 through 2006.

Estimate prepared by: Federal Costs: Julia Christensen, Impact on State, Local, and Tribal Governments: Leo Lex; and Impact on the Private Sector: Jennifer Bowman.

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

#### 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.

#### 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 of the legislation provides the short title for the bill.

##### *Section 2. Findings and purposes*

Section 2 provides the findings and purposes of the bill.

##### *Section 3. Food and Drug Administration; grants and contracts for the development of orphan drugs*

Section 3 reauthorizes the Orphan Products Research Grant program at such sums which have already been appropriated for FY 2002, and at \$25,000,000 for each of Fiscal Years 2003 through 2006.

##### *Section 4. Technical amendment*

Section 4 provides for a technical amendment to the Federal Food, Drug, and Cosmetic Act.

#### 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 (existing law proposed to be omit-

ted is enclosed in black brackets, new matter is printed in italic, existing law in which no change is proposed is shown in roman):

**SECTION 5 OF THE ORPHAN DRUG ACT**

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

SEC. 5. (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 such sums as already have been appropriated for fiscal year 2002, and \$25,000,000 for each of the fiscal years 2003 through 2006.*

\* \* \* \* \*

---

**SECTION 527 OF THE FEDERAL FOOD, DRUG, AND COSMETIC ACT**

PROTECTION FOR DRUGS FOR RARE DISEASES OR CONDITIONS

SEC. 527. (a) Except as provided in subsection (b), if the Secretary—

- (1) approves an application filed pursuant to section 505, or
- (2) issues a license under section 351 of the Public Health Service Act

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.

\* \* \* \* \*