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Folder Title: 11/06/2002 [H.R. 4014] [771588]

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FG006-01

Barcode Scanning Sheet

Collection Code: **SECLOG**

Staff Name:

Document Date:

Correspondent: **TIM SAUNDERS**

Subject/Description: **OVERSIZE ATTACHMENT # 9469 NARA # 9360 BOX 5 FOLDER 6C BILL FILE RECEIVED FROM THE OFFICE OF THE EXECUTIVE CLERK - NOV 05 02 ENROLLED BILL H.R. 4014 - RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF 2002**

EXECUTIVE OFFICE OF THE PRESIDENT
OFFICE OF MANAGEMENT AND BUDGET
WASHINGTON, D.C. 20503

October 30, 2002

THE DIRECTOR

THE PRESIDENT HAS SEEN
~~6-11-02~~
11-6-02

MEMORANDUM FOR THE PRESIDENT

SUBJECT: Enrolled Bill H.R. 4014 - Rare Diseases Orphan Product Development Act of 2002
Sponsors - Rep. Foley (R) FL and 58 cosponsors

Last Day for Action

November 8, 2002 - Friday

Purpose

Authorizes appropriations through FY 2006 for the Food and Drug Administration's (FDA) program that helps defray the costs of developing and testing new products to treat or diagnose rare diseases.

Agency Recommendations

Office of Management and Budget	Approval
Department of Health and Human Services (HHS)	Approval

Discussion

The Orphan Drug Act of 1983 (P.L. 97-414) provides economic incentives, including tax credits, grants, and exclusive marketing rights, for drug manufacturers to develop drugs for rare or "orphan" diseases. A rare or "orphan" disease is one that afflicts fewer than 200,000 individuals in the United States. According to FDA, there are more than 6,000 rare diseases affecting 20 to 25 million individuals in the United States. FDA's current program makes grants to and enters into contracts with public and private entities and individuals to defray some of the costs associated with the development and human clinical testing of new drugs, diagnostics, medical devices, and medical foods that treat illnesses that do not affect enough people to make development of such products profitable without such assistance.

H.R. 4014 would authorize appropriations of $25 million for each of FYs 2003-2006 for grants and contracts for developing and conducting human clinical testing of products for rare diseases and conditions. The FY 2003 Budget requested $12 million for FDA's rare diseases program.

Conclusion and Recommendations

HHS recommends approval of H.R. 4014. In its views letter on the enrolled bill, the Department states that the programs created by the Orphan Drug Act of 1983, including the grant program authorized by H.R. 4014, "are credited with promoting a substantial increase in the numbers of orphan products successfully developed and marketed."

We join HHS in recommending approval of H.R. 4014, which passed the House by voice vote and the Senate by unanimous consent.

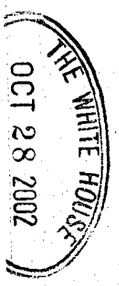
Mitchell E. Daniels, Jr.
Director

Enclosures

One Hundred Seventh Congress of the United States of America

AT THE SECOND SESSION

Begun and held at the City of Washington on Wednesday, the twenty-third day of January, two thousand and two



An Act

To amend the Federal Food, Drug, and Cosmetic Act with respect to the development of products for rare diseases.

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 Orphan Product Development Act of 2002".

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 Congress 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.

(7) 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 purpose of this Act is to increase the national investment in the development of diagnostics and treatments for patients with rare diseases and disorders.

SEC. 3. FOOD AND DRUG ADMINISTRATION; 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 such sums as already have been appropriated for fiscal year 2002, and $25,000,000 for each of the fiscal years 2003 through 2006.".

SEC. 4. 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,".

Speaker of the House of Representatives pro tempore.

~~*Vice President of the United States and*~~
President of the Senate. pro tempore.

APPROVED

NOV - 6 2002

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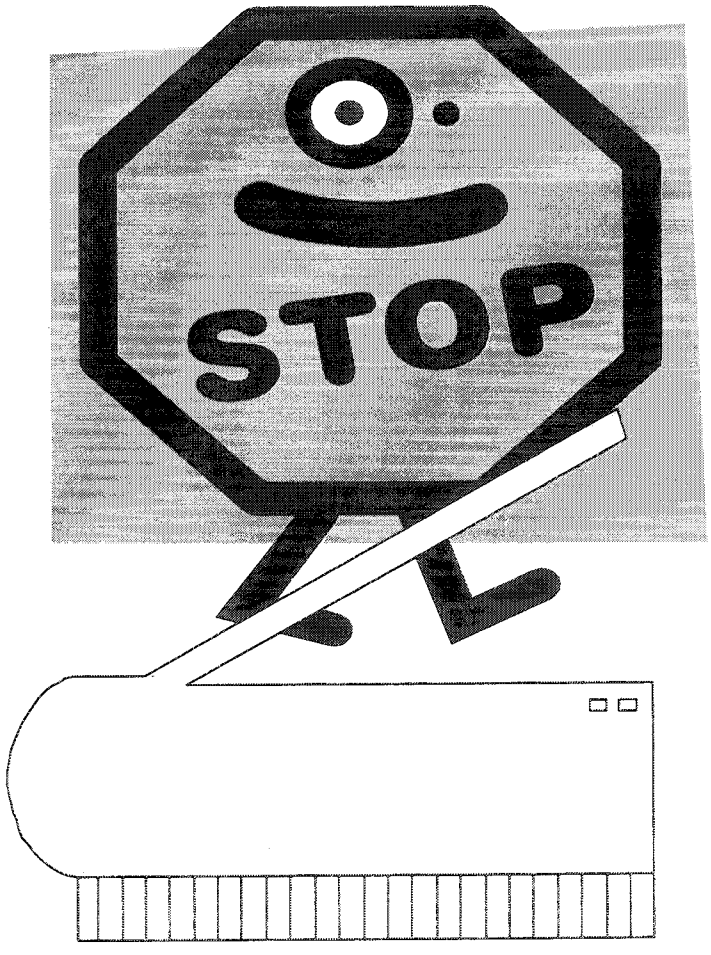

Remainder of case not scanned

Bills Received at the White House

Date received and notification to OMB: **10/28/2002**

Last day for action: **11/8/2002**

	Official Title	
H.R.4014	An Act to amend the Federal Food, Drug, and Cosmetic Act with respect to the development of products for rare diseases.	518757

SS/ RM NO. _____

WHITE HOUSE STAFFING MEMORANDUM

Date: 10-30-02 4:30 PM ACTION / CONCURRENCE / COMMENT DUE BY: 10-31-02 5:00 PM

Subject: ENROLLED BILL H.R. 4014 - RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF 2002

	ACTION	FYI		ACTION	FYI
VICE PRESIDENT	☑	☐	HUBBARD	☑	☐
CARD	☑	☐	IRASTORZA	☐	☐
BARTLETT	☑	☐	JOHNSON	☐	☐
BLAKEMAN	☐	☐	LINDSEY	☑	☐
BOLTEN	☑	☐	MARBURGER	☐	☐
BRIDGELAND	☑	☐	MIERS	☐	☐
CALIO	☑	☐	RICE	☑	☐
CONNAUGHTON	☑	☐	RIDGE	☑	☐
DANIELS	☐	☑	ROVE	☑	☐
FLEISCHER	☑	☐	SPELLINGS	☑	☐
GERSON	☐	☐	CLERK	☐	☑
GONZALES	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

REMARKS:

PLEASE FORWARD COMMENTS DIRECTLY TO JIM JUKES, X53458, FAX X56148, BY 5:00 P.M., THURSDAY, OCTOBER 31, 2002, WITH A COPY TO THE STAFF SECRETARY. THANK YOU.

RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

EXECUTIVE OFFICE OF THE PRESIDENT
OFFICE OF MANAGEMENT AND BUDGET
WASHINGTON, D.C. 20503

October 30, 2002

THE DIRECTOR

MEMORANDUM FOR THE PRESIDENT

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Sponsors - Rep. Foley (R) FL and 58 cosponsors

Last Day for Action

November 8, 2002 - Friday

Purpose

Authorizes appropriations through FY 2006 for the Food and Drug Administration's (FDA) program that helps defray the costs of developing and testing new products to treat or diagnose rare diseases.

Agency Recommendations

Office of Management and Budget	Approval
Department of Health and Human Services (HHS)	Approval

Discussion

The Orphan Drug Act of 1983 (P.L. 97-414) provides economic incentives, including tax credits, grants, and exclusive marketing rights, for drug manufacturers to develop drugs for rare or "orphan" diseases. A rare or "orphan" disease is one that afflicts fewer than 200,000 individuals in the United States. According to FDA, there are more than 6,000 rare diseases affecting 20 to 25 million individuals in the United States. FDA's current program makes grants to and enters into contracts with public and private entities and individuals to defray some of the costs associated with the development and human clinical testing of new drugs, diagnostics, medical devices, and medical foods that treat illnesses that do not affect enough people to make development of such products profitable without such assistance.

H.R. 4014 would authorize appropriations of $25 million for each of FYs 2003-2006 for grants and contracts for developing and conducting human clinical testing of products for rare diseases and conditions. The FY 2003 Budget requested $12 million for FDA's rare diseases program.

Conclusion and Recommendations

HHS recommends approval of H.R. 4014. In its views letter on the enrolled bill, the Department states that the programs created by the Orphan Drug Act of 1983, including the grant program authorized by H.R. 4014, "are credited with promoting a substantial increase in the numbers of orphan products successfully developed and marketed."

We join HHS in recommending approval of H.R. 4014, which passed the House by voice vote and the Senate by unanimous consent.

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VICE PRESIDENT no objection	☑	☐	HUBBARD ok	☑	☐
CARD ok	☑	☐	IRASTORZA	☐	☐
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BLAKEMAN	☐	☐	LINDSEY	☑	☐
BOLTEN ✓	☑	☐	MARBURGER	☐	☐
BRIDGELAND - N/C	☑	☐	MIERS	☐	☐
CALIO ok	☑	☐	RICE	☑	☐
CONNAUGHTON - N/C	☑	☐	RIDGE	☑	☐
DANIELS	☐	☑	ROVE N/C	☑	☐
FLEISCHER	☑	☐	SPELLINGS N/C ok	☑	☐
GERSON	☐	☐	CLERK	☐	☑
GONZALES - N/C	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

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H.R. 4014 would authorize appropriations of $25 million for each of FYs 2003-2006 for grants and contracts for developing and conducting human clinical testing of products for rare diseases and conditions. The FY 2003 Budget requested $12 million for FDA's rare diseases program.

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H.R. 4014 would authorize appropriations of $25 million for each of FYs 2003-2006 for grants and contracts for developing and conducting human clinical testing of products for rare diseases and conditions. The FY 2003 Budget requested $12 million for FDA's rare diseases program.

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We join HHS in recommending approval of H.R. 4014, which passed the House by voice vote and the Senate by unanimous consent.

Mitchell E. Daniels, Jr.
Director

Enclosures

THE SECRETARY OF HEALTH AND HUMAN SERVICES
WASHINGTON, D.C. 20201

October 25, 2002

The Honorable Mitchell E. Daniels, Jr.
Director, Office of Management and Budget
Washington, D.C. 20503

Dear Mr. Daniels:

This responds to your request for the views of the Department of Health and Human Services (HHS) on H.R. 4014, an enrolled bill entitled the "Rare Disease Orphan Product Development Act of 2002".

The bill extends authorizations through FY 2006 for grants under section 5 of the Orphan Drug Act for research on products for rare diseases.

The Orphan Drug Act of 1983 established several programs designed to eliminate financial barriers to development of treatments for rare diseases (defined as diseases which affect fewer than 200,000 persons in the United States). These small patient populations render it uneconomical for pharmaceutical companies to explore development of therapies for these conditions, as they cannot hope to recover their research investment through ultimate sales of the drugs. The Orphan Drug Act created an array of programs to overcome these disincentives. In addition to the section 5 grants to pharmaceutical companies sponsoring orphan product development, the Act provided for guaranteed periods of marketing exclusivity for orphan products and tax credits for clinical testing expenses. The provisions of the Act are credited with promoting a substantial increase in the numbers of orphan products successfully developed and marketed.

H.R. 4014 extends authority for the section 5 research grant program, authorizing appropriations of such sums as already have been appropriated for FY 2002 and $25,000,000 for each of FYs 2003 through 2006.

We recommend that the President approve the enrolled bill.

Sincerely,

Tommy G. Thompson

107TH CONGRESS
2d Session
HOUSE OF REPRESENTATIVES
REPORT
107–702

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.

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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.

* * * * * * *

○

H. R. 4014

One Hundred Seventh Congress of the United States of America

AT THE SECOND SESSION

Begun and held at the City of Washington on Wednesday, the twenty-third day of January, two thousand and two

An Act

To amend the Federal Food, Drug, and Cosmetic Act with respect to the development of products for rare diseases.

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 Orphan Product Development Act of 2002".

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 Congress 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.

(7) 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 purpose of this Act is to increase the national investment in the development of diagnostics and treatments for patients with rare diseases and disorders.

SEC. 3. FOOD AND DRUG ADMINISTRATION; 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 such sums as already have been appropriated for fiscal year 2002, and $25,000,000 for each of the fiscal years 2003 through 2006.".

SEC. 4. 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,".

Speaker of the House of Representatives.

Vice President of the United States and
President of the Senate.

Linda

EXECUTIVE OFFICE OF THE PRESIDENT
OFFICE OF MANAGEMENT AND BUDGET
WASHINGTON, D.C. 20503

October 30, 2002

THE DIRECTOR

MEMORANDUM FOR THE PRESIDENT

SUBJECT: Enrolled Bill H.R. 4014 - Rare Diseases Orphan Product Development Act of 2002
Sponsors - Rep. Foley (R) FL and 58 cosponsors

Last Day for Action

November 8, 2002 - Friday

Purpose

Authorizes appropriations through FY 2006 for the Food and Drug Administration's (FDA) program that helps defray the costs of developing and testing new products to treat or diagnose rare diseases.

Agency Recommendations

Office of Management and Budget	Approval
Department of Health and Human Services (HHS)	Approval

Discussion

The Orphan Drug Act of 1983 (P.L. 97-414) provides economic incentives, including tax credits, grants, and exclusive marketing rights, for drug manufacturers to develop drugs for rare or "orphan" diseases. A rare or "orphan" disease is one that afflicts fewer than 200,000 individuals in the United States. According to FDA, there are more than 6,000 rare diseases affecting 20 to 25 million individuals in the United States. FDA's current program makes grants to and enters into contracts with public and private entities and individuals to defray some of the costs associated with the development and human clinical testing of new drugs, diagnostics, medical devices, and medical foods that treat illnesses that do not affect enough people to make development of such products profitable without such assistance.

H.R. 4014 would authorize appropriations of $25 million for each of FYs 2003-2006 for grants and contracts for developing and conducting human clinical testing of products for rare diseases and conditions. The FY 2003 Budget requested $12 million for FDA's rare diseases program.

Conclusion and Recommendations

HHS recommends approval of H.R. 4014. In its views letter on the enrolled bill, the Department states that the programs created by the Orphan Drug Act of 1983, including the grant program authorized by H.R. 4014, "are credited with promoting a substantial increase in the numbers of orphan products successfully developed and marketed."

We join HHS in recommending approval of H.R. 4014, which passed the House by voice vote and the Senate by unanimous consent.

Mitchell E. Daniels, Jr.
Director

Enclosures

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*** TX REPORT ***
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TRANSMISSION OK

TX/RX NO	1143	
CONNECTION TEL		66212
CONNECTION ID		
ST. TIME	10/30 05:48	
USAGE T	01'07	
PGS. SENT	3	
RESULT	OK	

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WHITE HOUSE STAFFING MEMORANDUM

Date: 10-30-02 4:30 PM ACTION / CONCURRENCE / COMMENT DUE BY: 10-31-02 5:00 PM

Subject: ENROLLED BILL H.R. 4014 - RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF 2002

	ACTION	FYI		ACTION	FYI
VICE PRESIDENT	☑	☐	HUBBARD	☑	☐
CARD	☑	☐	IRASTORZA	☐	☐
BARTLETT	☑	☐	JOHNSON	☐	☐
BLAKEMAN	☐	☐	LINDSEY	☑	☐
BOLTEN	☑	☐	MARBURGER	☐	☐
BRIDGELAND	☑	☐	MIERS	☐	☐
CALIO	☑	☐	RICE	☑	☐
CONNAUGHTON	☑	☐	RIDGE	☑	☐
DANIELS	☐	☑	ROVE	☑	☐
FLEISCHER	☑	☐	SPELLINGS	☑	☐
GERSON	☐	☐	CLERK	☐	☑
GONZALES	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

REMARKS:
PLEASE FORWARD COMMENTS DIRECTLY TO JIM JUKES, X53458, FAX X56148,

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RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

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*** TX REPORT ***
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TRANSMISSION OK

TX/RX NO	1155	
CONNECTION TEL		62710
CONNECTION ID	CEQ	
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PGS. SENT	3	
RESULT	OK	

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RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

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*** TX REPORT ***
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TRANSMISSION OK

TX/RX NO	1149
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WHITE HOUSE STAFFING MEMORANDUM

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HAWKINS	☑	☐	_____	☐	☐

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RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

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TRANSMISSION OK

TX/RX NO	1152
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WHITE HOUSE STAFFING MEMORANDUM

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HAWKINS	☑	☐	_____	☐	☐

REMARKS:

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RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

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*** TX REPORT ***
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TRANSMISSION OK

TX/RX NO	1159
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WHITE HOUSE STAFFING MEMORANDUM

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VICE PRESIDENT	☑	☐	HUBBARD	☑	☐
CARD	☑	☐	IRASTORZA	☐	☐
BARTLETT	☑	☐	JOHNSON	☐	☐
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REMARKS:
PLEASE FORWARD COMMENTS DIRECTLY TO JIM JUKES, X53458, FAX X56148,

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RESULT	OK	

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WHITE HOUSE STAFFING MEMORANDUM

Date: 10-30-02 4:30 PM ACTION / CONCURRENCE / COMMENT DUE BY: 10-31-02 5:00 PM

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CARD	☑	☐	IRASTORZA	☐	☐
BARTLETT	☑	☐	JOHNSON	☐	☐
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HAWKINS	☑	☐	_____	☐	☐

REMARKS: FORWARD COMMENTS DIRECTLY TO JIM JUKES, X53458, FAX X56148, NLT OCT 31 2002, WITH A COPY TO THE STAFF SECRETARY.

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RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

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*** TX REPORT ***
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TRANSMISSION OK

TX/RX NO	1160	
CONNECTION TEL		56958
CONNECTION ID		
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RESULT	OK	

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FLEISCHER	☑	☐	SPELLINGS	☑	☐
GERSON	☐	☐	CLERK	☐	☑
GONZALES	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

REMARKS:
PLEASE FORWARD COMMENTS DIRECTLY TO JIM JUKES, X53458, FAX X56148,

SS/ RM NO. _____

WHITE HOUSE STAFFING MEMORANDUM

Date: 10-30-02 4:30 PM ACTION / CONCURRENCE / COMMENT DUE BY: 10-31-02 5:00 PM

Subject: ENROLLED BILL H.R. 4014 - RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF 2002

	ACTION	FYI		ACTION	FYI
VICE PRESIDENT	☑	☐	HUBBARD	☑	☐
CARD	☑	☐	IRASTORZA	☐	☐
BARTLETT	☑	☐	JOHNSON	☐	☐
BLAKEMAN	☐	☐	LINDSEY	☑	☐
BOLTEN	☑	☐	MARBURGER	☐	☐
BRIDGELAND	☑	☐	MIERS	☐	☐
CALIO	☑	☐	RICE	☑	☐
CONNAUGHTON	☑	☐	RIDGE	☑	☐
DANIELS	☐	☑	ROVE	☑	☐
FLEISCHER	☑	☐	SPELLINGS	☑	☐
GERSON	☐	☐	CLERK	☐	☑
GONZALES	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

REMARKS:

PLEASE FORWARD COMMENTS DIRECTLY TO JIM JUKES, X53458, FAX X56148, BY 5:00 P.M., THURSDAY, OCTOBER 31, 2002, WITH A COPY TO THE STAFF SECRETARY. THANK YOU.

RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

****
*** TX REPORT ***
****

TRANSMISSION OK

TX/RX NO	1163
CONNECTION TEL	67090
CONNECTION ID	ADMIN
ST. TIME	10/30 06:20
USAGE T	01'08
PGS. SENT	3
RESULT	OK

SS/ RM NO. _____

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RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

SS/ RM NO. _____

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RESPONSE:

BL AHC 10/30 6:30PM

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

EXECUTIVE OFFICE OF THE PRESIDENT
OFFICE OF MANAGEMENT AND BUDGET
WASHINGTON, D.C. 20503

October 30, 2002

THE DIRECTOR

MEMORANDUM FOR THE PRESIDENT

SUBJECT: Enrolled Bill H.R. 4014 - Rare Diseases Orphan Product Development Act of 2002
Sponsors - Rep. Foley (R) FL and 58 cosponsors

Last Day for Action

November 8, 2002 - Friday

Purpose

Authorizes appropriations through FY 2006 for the Food and Drug Administration's (FDA) program that helps defray the costs of developing and testing new products to treat or diagnose rare diseases.

Agency Recommendations

Office of Management and Budget	Approval
Department of Health and Human Services (HHS)	Approval

Discussion

The Orphan Drug Act of 1983 (P.L. 97-414) provides economic incentives, including tax credits, grants, and exclusive marketing rights, for drug manufacturers to develop drugs for rare or "orphan" diseases. A rare or "orphan" disease is one that afflicts fewer than 200,000 individuals in the United States. According to FDA, there are more than 6,000 rare diseases affecting 20 to 25 million individuals in the United States. FDA's current program makes grants to and enters into contracts with public and private entities and individuals to defray some of the costs associated with the development and human clinical testing of new drugs, diagnostics, medical devices, and medical foods that treat illnesses that do not affect enough people to make development of such products profitable without such assistance.

H.R. 4014 would authorize appropriations of $25 million for each of FYs 2003-2006 for grants and contracts for developing and conducting human clinical testing of products for rare diseases and conditions. The FY 2003 Budget requested $12 million for FDA's rare diseases program.

Conclusion and Recommendations

HHS recommends approval of H.R. 4014. In its views letter on the enrolled bill, the Department states that the programs created by the Orphan Drug Act of 1983, including the grant program authorized by H.R. 4014, "are credited with promoting a substantial increase in the numbers of orphan products successfully developed and marketed."

We join HHS in recommending approval of H.R. 4014, which passed the House by voice vote and the Senate by unanimous consent.

Mitchell E. Daniels, Jr.
Director

Enclosures

SS/ RM NO. _____

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GERSON	☐	☐	CLERK	☐	☑
GONZALES	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

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RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

EXECUTIVE OFFICE OF THE PRESIDENT
OFFICE OF MANAGEMENT AND BUDGET
WASHINGTON, D.C. 20503

October 30, 2002

THE DIRECTOR

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Mitchell E. Daniels, Jr.
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HAWKINS	☑	☐	_____	☐	☐

REMARKS:
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No comments

RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

SS/ RM NO. Jen

WHITE HOUSE STAFFING MEMORANDUM

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GONZALES	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

02 OCT 31 AM 9:01

REMARKS:

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RESPONSE: Viewed 10/30/02 JDN

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

SS/ RM NO. _____

WHITE HOUSE STAFFING MEMORANDUM

Date: 10-30-02 4:30 PM ACTION / CONCURRENCE / COMMENT DUE BY: 10-31-02 5:00 PM

Subject: ENROLLED BILL H.R. 4014 - RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF 2002

	ACTION	FYI		ACTION	FYI
VICE PRESIDENT	☑	☐	HUBBARD	☑	☐
CARD	☑	☐	IRASTORZA	☐	☐
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RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

SS/ RM NO. _____

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GONZALES	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

'02 OCT 31 AM 9:02

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No Comment

RESPONSE:

PA Cooley, CEQ
10/30/02

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

SS/ RM NO. _____

WHITE HOUSE STAFFING MEMORANDUM

Date: 10-30-02 4:30 PM ACTION / CONCURRENCE / COMMENT DUE BY: 10-31-02 5:00 PM

Subject: ENROLLED BILL H.R. 4014 - RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF 2002

'02 OCT 31 PM 5:50

	ACTION	FYI		ACTION	FYI
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CARD	☑	☐	IRASTORZA	☐	☐
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Ok - Phillip Swagel CEA

RESPONSE:

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

OFFICE OF THE VICE PRESIDENT

WASHINGTON

'02 OCT 31 PM 7:09

October 31, 2002

MEMORANDUM FOR JAMES JUKES
ASSISTANT DIRECTOR, OMB

FROM: **JONATHAN BURKS**
DEPUTY STAFF SECRETARY TO THE VICE PRESIDENT

SUBJECT: **Enrolled Bill H.R. 4014 – Rare Diseases Orphan Product Development Act of 2002**

The Office of the Vice President has reviewed the above-referenced draft and has no objection to the President signing the enrolled bill.

cc: **Harriet Miers**
Staff Secretary

SS/ RM NO. _____

WHITE HOUSE STAFFING MEMORANDUM

Date: 10-30-02 4:30 PM ACTION / CONCURRENCE / COMMENT DUE BY: 10-31-02 5:00 PM

'02 OCT 31 PM 7:09

Subject: ENROLLED BILL H.R. 4014 - RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF 2002

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RESPONSE:

cc Jay
Alan Philo
Tim

OK - No Comments

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

SS/ RM NO. _____

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RESPONSE:

Legislative Affairs (Howard) - ok

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

EXECUTIVE OFFICE OF THE PRESIDENT
OFFICE OF MANAGEMENT AND BUDGET
WASHINGTON, D.C. 20503

October 30, 2002

THE DIRECTOR

MEMORANDUM FOR THE PRESIDENT

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M E Daniels, J

Mitchell E. Daniels, Jr.
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Enclosures

SS/ RM NO. _____

WHITE HOUSE STAFFING MEMORANDUM

Date: 10-30-02 4:30 PM ACTION / CONCURRENCE / COMMENT DUE BY: 10-31-02 5:00 PM

Subject: ENROLLED BILL H.R. 4014 - RARE DISEASES ORPHAN PRODUCT DEVELOPMENT ACT OF 2002

	ACTION	FYI		ACTION	FYI
VICE PRESIDENT	☑	☐	HUBBARD	☑	☐
CARD	☑	☐	IRASTORZA	☐	☐
BARTLETT	☑	☐	JOHNSON	☐	☐
BLAKEMAN	☐	☐	LINDSEY	☑	☐
BOLTEN	☑	☐	MARBURGER	☐	☐
BRIDGELAND	☑	☐	MIERS	☐	☐
CALIO	☑	☐	RICE	☑	☐
CONNAUGHTON	☑	☐	RIDGE	☑	☐
DANIELS	☐	☑	ROVE	☑	☐
FLEISCHER	☑	☐	SPELLINGS	☑	☐
GERSON	☐	☐	CLERK	☐	☑
GONZALES	☑	☐	_____	☐	☐
HAGIN	☐	☐	_____	☐	☐
HAWKINS	☑	☐	_____	☐	☐

REMARKS:

PLEASE FORWARD COMMENTS DIRECTLY TO JIM JUKES, X53458, FAX X56148, BY 5:00 P.M., THURSDAY, OCTOBER 31, 2002, WITH A COPY TO THE STAFF SECRETARY. THANK YOU.

ok

RESPONSE:

cc Jay
Alan I Philo
Terri

Harriet E. Miers
Assistant to the President
and Staff Secretary
Ext. 62702

EXECUTIVE OFFICE OF THE PRESIDENT
OFFICE OF MANAGEMENT AND BUDGET
WASHINGTON, D.C. 20503

October 30, 2002

THE DIRECTOR

MEMORANDUM FOR THE PRESIDENT

SUBJECT: Enrolled Bill H.R. 4014 - Rare Diseases Orphan Product Development Act of 2002
Sponsors - Rep. Foley (R) FL and 58 cosponsors

Last Day for Action

November 8, 2002 - Friday

Purpose

Authorizes appropriations through FY 2006 for the Food and Drug Administration's (FDA) program that helps defray the costs of developing and testing new products to treat or diagnose rare diseases.

Agency Recommendations

Office of Management and Budget	Approval
Department of Health and Human Services (HHS)	Approval

Discussion

The Orphan Drug Act of 1983 (P.L. 97-414) provides economic incentives, including tax credits, grants, and exclusive marketing rights, for drug manufacturers to develop drugs for rare or "orphan" diseases. A rare or "orphan" disease is one that afflicts fewer than 200,000 individuals in the United States. According to FDA, there are more than 6,000 rare diseases affecting 20 to 25 million individuals in the United States. FDA's current program makes grants to and enters into contracts with public and private entities and individuals to defray some of the costs associated with the development and human clinical testing of new drugs, diagnostics, medical devices, and medical foods that treat illnesses that do not affect enough people to make development of such products profitable without such assistance.

H.R. 4014 would authorize appropriations of $25 million for each of FYs 2003-2006 for grants and contracts for developing and conducting human clinical testing of products for rare diseases and conditions. The FY 2003 Budget requested $12 million for FDA's rare diseases program.

Conclusion and Recommendations

HHS recommends approval of H.R. 4014. In its views letter on the enrolled bill, the Department states that the programs created by the Orphan Drug Act of 1983, including the grant program authorized by H.R. 4014, "are credited with promoting a substantial increase in the numbers of orphan products successfully developed and marketed."

We join HHS in recommending approval of H.R. 4014, which passed the House by voice vote and the Senate by unanimous consent.

Mitchell E. Daniels, Jr.
Director

Enclosures