

Union Calendar No. 435

107TH CONGRESS
2^D SESSION

H. R. 4014

[Report No. 107-702]

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

IN THE HOUSE OF REPRESENTATIVES

MARCH 20, 2002

Mr. FOLEY (for himself, Mr. WAXMAN, Mr. SHIMKUS, Mr. BROWN of Ohio, Mrs. ROUKEMA, Mr. RUSH, Mr. KING, Mr. GREENWOOD, and Mr. DINGELL) introduced the following bill; which was referred to the Committee on Energy and Commerce

OCTOBER 1, 2002

Additional sponsors: Mr. MCGOVERN, Mr. HORN, Mr. SMITH of New Jersey, Ms. MCKINNEY, Mrs. MORELLA, Mr. WYNN, Mr. GREEN of Texas, Mr. TOWNS, Mr. FRANK, Mr. LYNCH, Mr. PALLONE, Ms. RIVERS, Mr. GEORGE MILLER of California, Mrs. CAPPS, Ms. DEGETTE, Mr. WELDON of Florida, Mr. HOFFEL, Mr. LANTOS, Ms. DELAURO, Mr. STUPAK, Mrs. MINK of Hawaii, Mrs. KELLY, Ms. SLAUGHTER, Mr. McDERMOTT, Ms. WOOLSEY, Mr. PLATTS, Ms. VELÁZQUEZ, Mr. KIND, Mrs. JOHNSON of Connecticut, Mr. BONIOR, Mr. WEXLER, Ms. NORTON, Mr. LUCAS of Oklahoma, Mr. ISAKSON, Mrs. LOWEY, Ms. SCHAKOWSKY, Mr. KENNEDY of Rhode Island, Mr. COOKSEY, Mr. PRICE of North Carolina, Mr. WOLF, Mr. DEUTSCH, Ms. CARSON of Indiana, Mr. SANDERS, Mr. WATT of North Carolina, Mr. ENGEL, Mr. JENKINS, Mr. BALDACCI, Ms. HARMAN, Mr. SAWYER, and Mr. TOM DAVIS of Virginia

OCTOBER 1, 2002

Committed to the Committee of the Whole House on the State of the Union and ordered to be printed

A BILL

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

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Rare Diseases Orphan
5 Product Development Act of 2002”.

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

7 (a) FINDINGS.—Congress makes the following find-
8 ings:

9 (1) Rare diseases and disorders are those which
10 affect small patient populations, typically popu-
11 lations smaller than 200,000 individuals in the
12 United States. Such diseases and conditions include
13 Huntington’s disease, amyotrophic lateral sclerosis
14 (Lou Gehrig’s disease), Tourette syndrome, Crohn’s
15 disease, cystic fibrosis, cystinosis, and Duchenne
16 muscular dystrophy.

17 (2) For many years, the 25,000,000 Americans
18 suffering from the over 6,000 rare diseases and dis-
19 orders were denied access to effective medicines be-
20 cause prescription drug manufacturers could rarely
21 make a profit from marketing drugs for such small
22 groups of patients. The prescription drug industry

1 did not adequately fund research into such treat-
2 ments. Despite the urgent health need for these
3 medicines, they came to be known as “orphan
4 drugs” because no companies would commercialize
5 them.

6 (3) During the 1970s, an organization called
7 the National Organization for Rare Disorders
8 (NORD) was founded to provide services and to
9 lobby on behalf of patients with rare diseases and
10 disorders. NORD was instrumental in pressing Con-
11 gress for legislation to encourage the development of
12 orphan drugs.

13 (4) The Orphan Drug Act created financial in-
14 centives for the research and production of such or-
15phan drugs. New Federal programs at the National
16 Institutes of Health and the Food and Drug Admin-
17 istration encouraged clinical research and commer-
18 cial product development for products that target
19 rare diseases. An Orphan Products Board was estab-
20 lished to promote the development of drugs and de-
21 vices for rare diseases or disorders.

22 (5) Before 1983, some 38 orphan drugs had
23 been developed. Since the enactment of the Orphan
24 Drug Act, more than 220 new orphan drugs have
25 been approved and marketed in the United States

1 and more than 800 additional drugs are in the re-
2 search pipeline.

3 (6) Despite the tremendous success of the Or-
4 phan Drug Act, rare diseases and disorders deserve
5 greater emphasis in the national biomedical research
6 enterprise.

7 (7) The Food and Drug Administration sup-
8 ports small clinical trials through Orphan Products
9 Research Grants. Such grants embody successful
10 partnerships of government and industry, and have
11 led to the development of at least 23 drugs and four
12 medical devices for rare diseases and disorders. Yet
13 the appropriations in fiscal year 2001 for such
14 grants were less than in fiscal year 1995.

15 (b) PURPOSES.—The purpose of this Act is to in-
16 crease the national investment in the development of
17 diagnostics and treatments for patients with rare diseases
18 and disorders.

19 **SEC. 3. FOOD AND DRUG ADMINISTRATION; GRANTS AND**
20 **CONTRACTS FOR THE DEVELOPMENT OF OR-**
21 **PHAN DRUGS.**

22 Subsection (c) of section 5 of the Orphan Drug Act
23 (21 U.S.C. 360ee(e)) is amended to read as follows:

24 “(c) For grants and contracts under subsection (a),
25 there are authorized to be appropriated such sums as al-

1 ready have been appropriated for fiscal year 2002, and
2 \$25,000,000 for each of the fiscal years 2003 through
3 2006.”.

4 **SEC. 4. TECHNICAL AMENDMENT.**

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

8 (1) by striking “, of such certification,”; and

9 (2) by striking “, the issuance of the certifi-
10 cation,”.

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