

107TH CONGRESS
2^D SESSION

H. R. 4014

IN THE SENATE OF THE UNITED STATES

OCTOBER 2, 2002

Received

AN ACT

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,*

1 **SECTION 1. SHORT TITLE.**

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

4 **SEC. 2. FINDINGS AND PURPOSES.**

5 (a) FINDINGS.—Congress makes the following find-
6 ings:

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

15 (2) For many years, the 25,000,000 Americans
16 suffering from the over 6,000 rare diseases and dis-
17 orders were denied access to effective medicines be-
18 cause prescription drug manufacturers could rarely
19 make a profit from marketing drugs for such small
20 groups of patients. The prescription drug industry
21 did not adequately fund research into such treat-
22 ments. Despite the urgent health need for these
23 medicines, they came to be known as “orphan
24 drugs” because no companies would commercialize
25 them.

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

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

17 (5) Before 1983, some 38 orphan drugs had
18 been developed. Since the enactment of the Orphan
19 Drug Act, more than 220 new orphan drugs have
20 been approved and marketed in the United States
21 and more than 800 additional drugs are in the re-
22 search pipeline.

23 (6) Despite the tremendous success of the Or-
24 phan Drug Act, rare diseases and disorders deserve

1 greater emphasis in the national biomedical research
2 enterprise.

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

11 (b) PURPOSES.—The purpose of this Act is to in-
12 crease the national investment in the development of
13 diagnostics and treatments for patients with rare diseases
14 and disorders.

15 **SEC. 3. FOOD AND DRUG ADMINISTRATION; GRANTS AND**
16 **CONTRACTS FOR THE DEVELOPMENT OF OR-**
17 **PHAN DRUGS.**

18 Subsection (c) of section 5 of the Orphan Drug Act
19 (21 U.S.C. 360ee(c)) is amended to read as follows:

20 “(c) For grants and contracts under subsection (a),
21 there are authorized to be appropriated such sums as al-
22 ready have been appropriated for fiscal year 2002, and
23 \$25,000,000 for each of the fiscal years 2003 through
24 2006.”.

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

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

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

6 (2) by striking “, the issuance of the certifi-
7 cation,”.

Passed the House of Representatives October 1,
2002.

Attest:

JEFF TRANDAHL,

Clerk.