Public Law 107–281
107th Congress

An Act

Nov. 6, 2002
[H.R. 4014]

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,

Rare Diseases Orphan Product Development Act of 2002.
21 USC 301 note.

SECTION 1. SHORT TITLE.

This Act may be cited as the "Rare Diseases Orphan Product Development Act of 2002".

21 USC 360ee note.

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.". Appropriation authorization.

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,".

Approved November 6, 2002.

LEGISLATIVE HISTORY—H.R. 4014:

HOUSE REPORTS: No. 107–702 (Comm. on Energy and Commerce).
CONGRESSIONAL RECORD, Vol. 148 (2002):
Oct. 1, considered and passed House.
Oct. 17, considered and passed Senate.