

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 [see Short Title of 1983 Amendments note set out under section 301 of this title] 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 [Jan. 4, 1983], 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 [see Short Title of 2002 Amendments note set out under section 301 of this title] is to increase the national investment in the development of diagnostics and treatments for patients with rare diseases and disorders.”

§ 360ee-1. FDA rare neurodegenerative disease grant program

The Secretary of Health and Human Services, acting through the Commissioner of Food and Drugs, shall award grants and contracts to public and private entities to cover the costs of research on, and development of interventions intended to prevent, diagnose, mitigate, treat, or cure, amyotrophic lateral sclerosis and other rare neurodegenerative diseases in adults and children, including costs incurred with respect to the development and critical evaluation of tools, methods, and processes—

- (1) to characterize such neurodegenerative diseases and their natural history;
- (2) to identify molecular targets for such neurodegenerative diseases; and
- (3) to increase efficiency and productivity of clinical development of therapies, including through—
 - (A) the use of master protocols and adaptive and add-on clinical trial designs; and
 - (B) efforts to establish new or leverage existing clinical trial networks.

(Pub. L. 117–79, § 5, Dec. 23, 2021, 135 Stat. 1537.)

Editorial Notes

CODIFICATION

Section was enacted as part of the Accelerating Access to Critical Therapies for ALS Act, and not as part of the Federal Food, Drug, and Cosmetic Act which comprises this chapter.

§ 360ff. Priority review to encourage treatments for rare pediatric diseases

(a) Definitions

In this section:

(1) Priority review

The term “priority review”, with respect to a human drug application as defined in section 379g(1) of this title, means review and action by the Secretary on such application not later than 6 months after receipt by the Secretary of such application, as described in the Manual of Policies and Procedures of the Food and Drug Administration and goals identified in the letters described in section 101(b) of the Prescription Drug User Fee Amendments of 2012.

(2) Priority review voucher

The term “priority review voucher” means a voucher issued by the Secretary to the sponsor of a rare pediatric disease product application that entitles the holder of such voucher to priority review of a single human drug application submitted under section 355(b)(1) of this title or section 351(a) of the Public Health Service Act [42 U.S.C. 262(a)] after the date of approval of the rare pediatric disease product application.

(3) Rare pediatric disease

The term “rare pediatric disease” means a disease that meets each of the following criteria:

- (A) The disease is a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents.
- (B) The disease is a rare disease or condition, within the meaning of section 360bb of this title.

(4) Rare pediatric disease product application

The term “rare pediatric disease product application” means a human drug application, as defined in section 379g(1) of this title, that—

- (A) is for a drug or biological product that is for the prevention or treatment of a rare pediatric disease;
- (B)(i) is for such a drug—
 - (I) that contains no active moiety (as defined by the Secretary in section 314.3 of title 21, Code of Federal Regulations (or any successor regulations)) that has been previously approved in any other application under subsection (b)(1), (b)(2), or (j) of section 355 of this title; and
 - (II) that is the subject of an application submitted under section 355(b)(1) of this title; or
- (ii) is for such a biological product—
 - (I) that contains no active ingredient that has been previously approved in any