

Orphan Drugs

The term "orphan drug" refers to a product that treats a rare disease affecting fewer than 200,000 Americans. The Orphan Drug Act was signed into law on January 4, 1983. Since the Orphan Drug Act passed, over 100 orphan drugs and biological products have been brought to market.

The intent of the Orphan Drug Act is to stimulate the research, development, and approval of products that treat rare diseases. This mission is accomplished through several mechanisms:

- Sponsors are granted seven years of marketing exclusivity after approval of its orphan drug product.
- Sponsors also are granted tax incentives for clinical research they have undertaken.
- FDA's [Office of Orphan Products Development](#) coordinates research study design assistance for sponsors of drugs for rare diseases
- The Office of Orphan Products Development also encourages sponsors to conduct open protocols, allowing patients to be added to ongoing studies.
- Grant funding is available to defray costs of qualified clinical testing expenses incurred in connection with the development of orphan products.

See Title 21 CFR 316 for the regulations governing the use of orphan drugs. In order to be considered an orphan drug, FDA certification must be obtained.

Orphan drug protocols are treated as regular protocol submissions by the IRB. A protocol application is required, a consent form must be reviewed, and available supporting documents must be provided, such as the Investigator's Brochure and the Sponsor Protocol. In addition, the investigator must provide certification that the drug is considered an orphan drug by the FDA.