FDA/CDER
SMALL BUSINESS CHRONICLES
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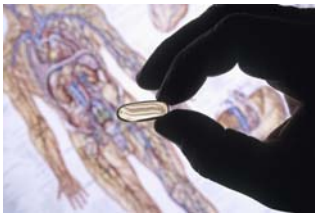


An orphan drug is a drug intended to treat a condition affecting fewer than 200,000 persons in the US, or which will not be profitable within 7 years following approval by the FDA

Orphan Drugs

When Daddy Warbucks gave Little Orphan Annie some attention, it transformed her life. Similarly, when pharmaceutical companies turn their attention to rare and often neglected diseases, they have the potential to touch the lives of people that often have no alternatives. To encourage development of these types of drugs FDA has developed several incentives. In fact, we have a name for these drugs – orphan drugs.

The FDA Office of Orphan Products Development (OOPD) mission is to advance the evaluation and development of products (drugs, biologics, devices, or medical foods) that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions.

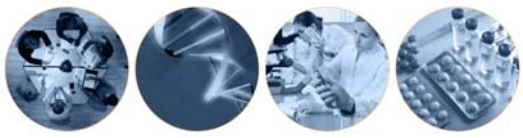
Orphan Drug Definition An orphan drug is defined in the 1984 amendments of the U.S. Orphan Drug Act (ODA) as a drug intended to treat a condition affecting fewer than 200,000 persons in the United States, or which will not be profitable within 7 years following approval by the FDA.

Incentives Orphan drugs are desperately needed by patients with rare diseases. The ODA provides for granting special status, orphan drug designation, to a product to treat a rare disease or condition upon request of a sponsor. The product to treat the rare disease or condition must meet certain criteria. Orphan designation qualifies the sponsor of the product for:

- Seven-year marketing exclusivity to the first sponsor obtaining FDA approval of a designated drug
- Tax credit equal to 50% of clinical investigation expenses
- Exemption/Waiver of PDUFA application (filing) fees
- Assistance in the drug development process
- Orphan Products Grant funding

Eligibility A previously unapproved drug may be eligible for orphan drug status if it meets the definition of an orphan drug and is:
- A previously unapproved drug
- A new orphan indication for an approved drug
- The "same drug" as one already approved but with a potential to be "clinically superior"

Sometimes, a drug may be granted orphan status when it is under development for only a subset of persons with a particular disease or condition, a demonstration that the subset is medically plausible. A medically plausible subset of a more common disease has been defined only in terms of a characteristic of the drug that would limit its use to just a segment of those afflicted. An example of a medically plausible subset is of a drug so toxic that only the most severely ill patients would use it (e.g. stage IV cancer); there would be too many risks for a less ill patient to use that therapy.



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Requesting Orphan Designation The sponsor must submit the request for orphan designation to OOPD before filing the NDA/BLA. If OOPD grants orphan designation, then the drug is said to have "orphan status." Sponsors requesting designation of the same drug for the same indication as a previously designated product must submit their own data in support of their designation request.

FDA does not require a form to apply for orphan drug designation. Our webpage outlines the information sponsors should submit. Don't forget to check out our submission tips. Sponsors may use FDA Form 3671to apply for orphan designation in both Europe and the US at the same time.

Sponsors can submit an electronic orphan designation application on a single CD-ROM disk with a signed cover letter. You can find additional information in our Draft Guidance for Providing Regulatory Submissions in Electronic Format — Orphan-Drug and Humanitarian Use Device Designation Requests and Related Submissions.

Orphan Drug Status Orphan status does not mean that FDA has approved the drug. Orphan status and FDA approval are not the same. The approval of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Sponsors must establish safety and efficacy of a compound to treat a rare disease through adequate and well-controlled studies.

Resources The following databases contain information about drugs with orphan status:

- Orphan Designated and or Approved Products searchable database: includes information such as the sponsor's name, address and contact information, name of drug, proposed indication and date of designation on orphan designated products.

- Repurposing of Products for Rare Diseases (RDRD) database: includes products that have received orphan status designation AND are already market-approved for the treatment of some other diseases up through June 2010. This database is a useful tool for finding special opportunities to develop niche therapies that are already well-advanced through development.

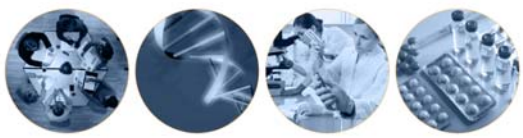
Grants The Orphan Products Grants Program provides funding for clinical research that tests the safety and efficacy of drugs and other products in rare diseases or conditions. Grants ensure that product development occurs in a timely manner with a very modest investment. Since the program's inception in 1983, it has been used to bring 45 products to marketing approval. At any one time, there are typically 60 to 85 ongoing grant-funded projects, and in general, OOPD grant funding lasts for three-four years. A major portion of the appropriated funds (typically approximately $14 million) for a given fiscal year go towards continued funding of prior approved grants. OOPD usually funds between 10-15 new grants per fiscal year. You may refer to the Searchable Database of Research Grants Funded by OOPD.

We hope you take advantage of these opportunities.

Cheers,

The CDER Small Business Assistance Team

Issues of this newsletter are archived at http://www.fda.gov/cdersmallbusinesschronicles



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