



“DO A DESIGNATION” FDA Orphan Drug Workshops

FDA announces the launch of its orphan drug workshop series, an opportunity for academics, biotechnology companies and larger pharmaceutical firms to spend two days in creation of applications for orphan status designation. Participants will be expected to bring specific products for at least one candidate orphan drug that holds promise for the treatment of a rare disease. Over two days, FDA staff from the Office of Orphan Products Development (OOPD) will provide regulatory assistance to sponsors to find regulatory paths forward. There will be an introductory lecture, but most of the time will be spent in application writing and individual one-on-one guidance sessions to develop the strongest possible orphan designation application to be submitted at the close of the workshop.

Who should attend?

This workshop is designed to simplify and demystify entry into orphan drug development by small biotechnology companies, academics and larger industry entities. It is a *workshop*, not a *listenshop*, and participants are expected to directly engage in the practical matter of constructing an application for orphan

status designation, and so should come prepared to work on a particular promising therapy, not just therapies in general. Participants need not be the holders of intellectual property rights of the compounds for which they seek orphan designation, but need only to be a “party of interest” and will be considered to be “sponsors” of designations that may be ultimately granted (though naturally, post-designation product development is dependent upon product access and testing). Sponsors should note that participation in this workshop confers neither expedited processing nor preferential consideration of their applications, however a good-faith effort will be made by FDA staff to provide regulatory assistance to aid your work.

Historically, orphan status designation has been an extremely productive means of advancing good ideas into regulatory realities. Moreover, as an FDA affirmation of a drug’s promise, orphan designation has become an early-stage regulatory concurrence that has a track record of provoking investor interest. With about 2,100 orphan designated drugs (of which 344 have become approved products), your product could have its advancement aided by FDA staff devoted to advancing new

products for people with rare diseases who need them.

Are you ready for orphan status designation?

The chief task of the orphan status designation application is to convince FDA/OOPD of two things: that the proposed drug is for a rare disease (i.e. that fewer than 200,000 persons in the US have the rare disease or condition) and that there is a medical rationale for believing that proposed drug has “promise” for treating the rare disease/condition (i.e. clinical data, animal model data OR rarely in vitro data, but not exclusively theoretical considerations). This major content of an orphan status designation can often be gleaned from the extant medical literature alone.

Orphan status designation can be secured anytime prior to the submission of a marketing application; it need not be under consideration as an investigational new drug (IND) nor are pre-clinical toxicology studies nor manufacturing certifications required. If you have a product envisioned for a rare disease you like to see move forward and there is sufficient data to show it may be promising, then you’re ready.

Worried about creating an orphan drug designation application?

Then you're not alone, typically submissions to the FDA evoke considerable consternation among drug sponsors. This workshop is designed to make the process of orphan drug development transparent and accessible to those uninitiated in regulatory affairs.

How do I register?

Prospective participants should send to CDR Lucienne Nelson at 301-827-3666 or lucienne.nelson@fda.hhs.gov the following information:

- Names of proposed attendees
- Contact information
- Desired workshop location
- Proposed orphan product for which an application will be developed
- Proposed rare diseases for said product

FDA will keep product and disease information provided confidential. Acceptance at each workshop will be limited to no more than three participants who attend in support of each of the first 50 product proposals.



Workshop Locations:

The inaugural workshops will be held at:



Details on workshop fees, accommodations and other travel arrangements can be obtained from separate announcements made by these institutions.



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FDA Orphan Drug Workshops:

**Keck Graduate Institute
Claremont, California
February 25-26, 2010**

**Center for Orphan Drug Research
College of Pharmacy
University of Minnesota
Minneapolis, Minnesota
August 3-4, 2010**

**Sponsored by the
Office of Orphan Products
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