



“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 full days, FDA staff from the Office of Orphan Products Development (OOPD) will provide regulatory assistance to sponsors to find regulatory paths forward. The opening evening begins with a keynote on the orientation to the application process, 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 end of the workshop or within 30 days 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 with a working draft submission of 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; the drug need not be under consideration as an investigational new drug (IND) nor are pre-clinical toxicology studies or 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 CAPT Stephanie Donahoe at 301-796-8681 or stephanie.donahoe@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 the identity of participants, 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:



KECK GRADUATE INSTITUTE
of Applied Life Sciences

Keck Graduate Institute
Center for Rare Disease Therapies
Claremont, California
February 25-26, 2010



UNIVERSITY OF MINNESOTA

College of Pharmacy

Center for Orphan Drug Research
College of Pharmacy
University of Minnesota
Minneapolis, Minnesota
August 2-4, 2010

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



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Sponsored by the
Office of Orphan Products
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