"DO A DESIGNATION"
FDA Orphan Drug Workshop

FDA announces the continuation of its orphan drug workshop series, an opportunity for academics, biotechnology companies and pharmaceutical firms to spend a day preparing an application for orphan status designation. Participants will be expected to bring a draft designation application for at least one candidate orphan drug that holds promise for the treatment of a rare disease. FDA staff from the Office of Orphan Products Development (OOPD) will provide regulatory assistance to sponsors to help find a regulatory path forward. There will be an introductory lecture and an Orphan Grants Program presentation, 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. The application to FDA may be submitted at the close of the workshop.

Who should attend?

This workshop is designed to simplify and demystify entry into orphan drug development and to provide registrants assistance in the preparation of their designation request. It is a *work*shop, not a *listen*shop, 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). *Attendees should note that participation in this workshop confers neither expedited processing nor preferential consideration of their applications.*

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 2,300 orphan designated drugs (of which 362 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 U.S. 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 or manufacturing certifications required. If you have a product envisioned for a rare disease that you would like to see move forward and if there is sufficient data to show it may be promising, then you should consider this workshop.

Worried about preparing an orphan drug designation application?

Then you are 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 unfamiliar with regulatory affairs.

Interested in the OOPD Grants Program?

OOPD will present information concerning its Grants Program which provides funds to encourage clinical development of rare diseases products. Sessions will be available to address specific questions. OOPD funds Phase 1 clinical trials up to $200,000/year for up to 3 years and Phase 2 or 3 trials up to $400,000/year for up to 4 years.

How do I register?

Prospective participants should email to James Bona at james.bona@fda.hhs.gov or fax to 301-847-8621, the following information:

- Requested date of attendance
- Names of proposed attendees
- Contact information
- Proposed orphan product for which an application will be developed
- Proposed rare diseases for said product

Acceptance at each workshop will be limited to no more than three participants per team/sponsor who attend in support of each of the first 30 product proposals.

Upon notification of acceptance to the workshop, attendees must complete registration for the workshop with:

Benjamin Zaitz, DIA Program Manager at 1-215-293-5803 or benjamin.zaitz@diahome.org.

Details on workshop fees, accommodations and other travel arrangements can be obtained on the DIA Website:

Click here for more information

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FDA Orphan Drug Workshop:
National Conference Center
Lansdowne, Virginia
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Sponsored by:
Office of Orphan Products Development
Food and Drug Administration

and
Drug Information Association

In Partnership with:
NORD
and Genetic Alliance