FDA NOTE TO CORRESPONDENTS

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Fourth FDA orphan drug designation workshop scheduled for Feb. 28 - March 1, 2011
Agency experts to provide guidance on applying for orphan drug designation

The U.S. Food and Drug Administration has scheduled its fourth orphan drug designation workshop for academics, biotechnology companies, and those unfamiliar with the process for Feb. 28 – March 1, 2011, in Claremont, Calif. in collaboration with Keck Graduate Institute.

The workshop, co-sponsored by the National Organization of Rare Disorders and the Genetic Alliance, will focus on the process used by the FDA to grant a special status, known as orphan drug designation, for drug products intended to treat rare diseases. Three similar workshops were held in 2010.

Orphan drugs are either drugs or biologics intended for the treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the United States, or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing the drug. Orphan designation qualifies the applicant to receive certain benefits, such as tax credits and marketing incentives, from the federal government in exchange for developing the drug.

During the workshop, participants will propose a specific drug for a specific rare disease and work on an orphan designation application to submit to the FDA at the conclusion of the workshop. FDA staff will provide one-on-one guidance to help participants develop strong applications. Designation requires there be a scientific rationale for expecting the proposed drug to be effective in the treatment, prevention, or diagnosis of that disease or condition.

For more information:

FDA: Orphan Drug Workshop
http://www.fda.gov/downloads/ForIndustry/DevelopingProductsforRareDiseasesConditions/UCM215487.pdf

Developing Products for Rare Diseases and Conditions
http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm