4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2016-N-0012]

Natural History Studies for Rare Disease Product Development: Orphan Products Research Project Grant (R01)

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the availability of grant funds for the support of FDA's Office of Orphan Products Development (OOPD) Natural History Grants Program. The goal of the Orphan Products Natural History Grants Program is to support studies that advance rare disease medical product development through characterization of the natural history of rare diseases/conditions, identification of genotypic and phenotypic subpopulations, and development and/or validation of clinical outcome measures, biomarkers and/or companion diagnostics. The ultimate goal of these natural history studies is to support clinical development of products for use in rare diseases or conditions where no current therapy exists or where the proposed product will be superior to the existing therapy. FDA provides grants for natural history studies that will either assist or substantially contribute to market approval of these products. Applicants must include in the application's Background and Significance section documentation to support that the estimated prevalence of the orphan disease or condition in the United States is less than 200,000 (or in the case of a vaccine or diagnostic, information to support that the product will be administered to fewer than 200,000

people in the United States per year), and an explanation of how the proposed study will either help support product approval or provide essential data needed for product development.

DATES: Important dates are as follows:

1. The application due dates are October 14, 2016 and October 15, 2018.
2. The anticipated start dates are March 2017 and March 2019.
3. The opening dates are August 15, 2016 and August 15, 2018.
4. The expiration date is October 16, 2018.

ADDRESSES: Submit electronic applications to: http://www.grants.gov. For more information, see section III of the SUPPLEMENTARY INFORMATION section of this notice.

FOR FURTHER INFORMATION AND ADDITIONAL REQUIREMENTS CONTACT: Katherine Needleman, Office of Orphan Products Development, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 32, rm. 5295, Silver Spring, MD 20993-0002, 301-796-8660, email: katherine.needleman@fda.hhs.gov; or Daniel Lukash, Office of Acquisitions and Grant Services, 5630 Fishers Lane, Rockville, MD 20857, 240-402-7596, email: daniel.lukash@fda.hhs.gov.

For more information on this funding opportunity announcement (FOA) and to obtain detailed requirements, please refer to the full FOA located at http://grants.nih.gov/grants/guide (select the "Request for Applications" link), http://www.grants.gov (see "For Applicants" section), and http://www.fda.gov/orphan.

SUPPLEMENTARY INFORMATION:

I. Funding Opportunity Description

RFA-FD-16-043

93.103

A. Background

The OOPD was created to identify and promote the development of orphan products. Orphan products are drugs, biologics, medical devices, and medical foods that are indicated for a rare disease or condition. The term "rare disease or condition" is defined in section 528 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ee). FDA generally considers drugs, devices, and medical foods potentially eligible for grants under the OOPD grant program if they are indicated for a disease or condition that has a prevalence, not incidence, of fewer than 200,000 people in the United States. Diagnostics and vaccines are considered potentially eligible for such grants only if the U.S. population to whom they will be administered is fewer than 200,000 people in the United States per year.

The natural history of a disease is the natural course of a disease from the time immediately prior to its inception, progressing through its pre-symptomatic phase and different clinical stages to the point where the disease has ended without external intervention. Natural history studies track the course of disease over time, identifying demographic, genetic, environmental, and other variables that correlate with its development and outcomes in the absence of treatment. Thorough understanding of disease natural history is the foundation upon which a clinical development program for drugs, biologics, medical foods or medical devices is built.

Rare diseases, as defined in the United States Orphan Drug Act (ODA) (Pub. L. 97-414), are diseases or conditions with a prevalence of fewer than 200,000 persons in the United States. Though individually rare, together there are approximately 30 million Americans affected by 7,000 known rare diseases. Unlike common diseases, there is little existing knowledge on the natural history of most rare diseases, which makes natural history studies of particular

importance for rare diseases product development. In January 2014, the FDA organized a Public Workshop on Complex Issues in Developing Drugs for Rare Diseases. During the workshop, the lack of natural history studies was reconfirmed by all stakeholders (patients, industry, researchers and the FDA) as one of the most common and urgent issues that hinder treatment development for rare diseases. The need for natural history studies was also emphasized in the recently published (August 17, 2015) draft FDA Guidance for Industry, "Rare Diseases: Common Issues in Drug Development," available at http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM458485.pdf.

B. Research Objectives

The objective of FDA's Orphan Products Natural History Grants Program is to support studies that characterize the natural history of rare diseases/conditions, identify genotypic and phenotypic subpopulations, and develop and/or validate clinical outcome measures, biomarkers and/or companion diagnostics. The ultimate goal of these natural history studies is to support clinical development of products for use in serious rare diseases or conditions where no current therapy exists or where the proposed product will be superior to the existing therapy. FDA provides grants for natural history studies that will either assist or substantially contribute to market approval of these products. Applicants must include in the application's Background and Significance section documentation to support that the estimated prevalence of the orphan disease or condition in the United States is less than 200,000 (or in the case of a vaccine or diagnostic, information to support that the product will be administered to fewer than 200,000 people in the United States per year), and an explanation of how the proposed study will either help support product approval or provide essential data needed for product development.

C. Eligibility Information

The grants are available to any foreign or domestic, public or private, for-profit or nonprofit entity (including State and local units of government). Federal Agencies may not apply.

II. Award Information/Funds Available

A. Award Amount

Of the estimated FY 2017 funding ($17.7 million), approximately $2 million will fund 2 to 5 new awards, subject to availability of funds. Prospective Natural History Studies are eligible for grants of up to $400,000 per year for up to 5 years. Retrospective Natural History Studies or Surveys are eligible for grants of up to $150,000 per year for up to 2 years. Please note that the dollar limitation will apply to total costs (direct plus indirect). Budgets for each year of requested support may not exceed the $150,000 or $400,000 total cost limit, whichever is applicable.

B. Length of Support

The length of support will depend on the nature of the study. For those studies with an expected duration of more than 1 year, all future years of noncompetitive continuation of support will depend on the following factors: (1) Performance during the preceding year; (2) compliance with regulatory requirements as applicable; and (3) availability of Federal funds.

III. Electronic Application, Registration, and Submission

Only electronic applications will be accepted. To submit an electronic application in response to this FOA, applicants should first review the full announcement located at http://grants.nih.gov/grants/guide. For all electronically submitted applications, the following steps are required.

- Step 1: Obtain a Dun and Bradstreet (DUNS) Number
- Step 2: Register With System for Award Management (SAM) (formerly Central Contractor Registration (CCR))
- Step 3: Obtain Username & Password on Grants.gov
- Step 4: Authorized Organization Representative (AOR) Authorization
- Step 5: Track AOR Status
- Step 6: Register With Electronic Research Administration (eRA) Commons

Steps 1 through 5, in detail, can be found at http://www07.grants.gov/applicants/organization_registration.jsp. Step 6, in detail, can be found at https://commons.era.nih.gov/commons/registration/registrationInstructions.jsp. After you have followed these steps, submit electronic applications to: http://www.grants.gov.

Dated: April 28, 2016.

Leslie Kux,

Associate Commissioner for Policy.

[FR Doc. 2016-10398 Filed: 5/3/2016 8:45 am; Publication Date: 5/4/2016]