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DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2015-N-0012]

Disease Natural History Database Development--(U24)

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 Natural History Database Development. The National Organization for Rare Disorders (NORD) is developing an Internet-based data collection tool with promise to further the accumulation of natural history data for many rare diseases. The goal of this grant is to enable NORD to further develop, refine, and disseminate the database tool.

DATES: Important dates are as follows:

1. The application due date is September 4, 2015.
2. The anticipated start date is September 2015.
3. The opening date is July 2015.
4. The expiration date is September 5, 2015.

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 CONTACT:

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For more information on this funding opportunity announcement (FOA) and to obtain detailed requirements, please refer to the full FOA located at www.grants.gov. Search by Funding Opportunity Number: RFA-FD-15-038.

SUPPLEMENTARY INFORMATION:

I. Funding Opportunity Description

RFA-FD-15-038

93.103

A. Background

There are an estimated 7,000 rare diseases, in total affecting approximately 30 million Americans. Most of these are serious conditions with no approved therapies. Rare diseases constitute an enormous unmet medical need.

Drug development for rare diseases, as well as for common diseases, relies on an indepth knowledge of the diseases' natural histories. Natural history is the course of the disease in the absence of a clinical intervention (that is, treatment under clinical care or study). Natural history knowledge makes possible the design of successful and efficient drug development programs. This knowledge has wide-ranging applications at every stage of drug development, for example, insight into the mechanism of disease, which can inform proof-of-concept studies; development of biomarkers that can expedite clinical studies at every stage of drug development; recognition and understanding of phenotypes of disease that may respond more (or less) to a therapy; and knowledge of the aspects of disease that matter to patients, with an impact on developing drugs that have a meaningful impact on how a patient feels, functions, or survives. The lack of natural history knowledge can result in the failure of drug programs, even for drugs with great promise. Unfortunately, the natural history of rare diseases is often poorly understood.

Impediments to the understanding of the natural history of a rare disease include the small numbers of patients and the sparse dispersal of clinical experience even among the chief clinical referral centers. The rare disease community is largely composed of small, diverse groups including patient and patient-family support, nonprofit disease groups (including umbrella groups), academic researchers, and small- to medium-sized biotechnology and pharmaceutical companies. For most rare diseases there has been no mechanism to systematically collect rare disease knowledge. In addition, it has become increasingly clear that

it is vitally important to collect more knowledge from living patients over time, not simply to collect currently available information. This "longitudinal" information about individual patients is invaluable to the design of a drug development program. The rare disease community is in need of a means of collecting and analyzing this knowledge: A natural history database tool.

B. Research Objectives

The development of natural history databases will directly further FDA's public health mission. We anticipate that the successful implementation of a natural history database will have profound and far-reaching effects on development of therapies for rare diseases. As a basis for solid natural history knowledge of a disease it may help to make a clinical development program for a candidate therapy appear feasible, and thus a more attractive area to pharmaceutical companies for devoting a portion of their drug discovery resources. This too will lead to greater numbers of therapies for rare diseases.

C. Eligibility Information

Only the following organization is eligible to apply: The National Organization for Rare Disorders. NORD is uniquely qualified to apply for this grant as the only applicant. Natural history studies is an area of unmet need and there are very few efforts towards building these studies. Those efforts that exist are very limited to specific diseases (e.g., cystic fibrosis, urea cycle disorders). These individual efforts cannot and do not support other patient groups starting their own studies. Most efforts are largely focused on patient communication and patient reports through Web-based self-reporting and are not likely to conform to sufficient scientific rigor to be able to support drug development. Although patient registries exist, these are not the same thing as natural history studies, and can often be very broad and general and cannot be customized to the depth and scope needed to support multiple natural history studies in a diverse group of rare

diseases. The rigor, scope, and flexibility of NORD's platform, which comes from approximately 15 years of working with the rare disease community on these efforts, is unique and directly suited to the needs of FDA.

II. Award Information/Funds Available

A. Award Amount

FDA/Center for Drug Evaluation and Research intends to fund up to $250,000, for fiscal year 2015 in support of this grant program. It is anticipated that one award will be made, not to exceed $250,000 in total costs (direct plus indirect).

B. Length of Support

The maximum project period is 1 year.

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 www.grants.gov. Search by Funding Opportunity Number: RFA-FD-15-038. 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)
- Step 3: Obtain Username & Password
- 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: August 10, 2015.

Leslie Kux,

Associate Commissioner for Policy.

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