Natural History Studies Registry Project

Summary of Presentation given by Jacqueline Kraska
NORD Research Programs Manager

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Online Seminar Overview

This session will provide participants with an overview on:

- A description of the project
- An overview of Natural History Studies
- Application process
- Q&A
“To empower patient organizations, patients, and families, NORD is collaborating with NIH and FDA to advance the development of more and better natural history data. The cornerstone of this effort is a practical and affordable platform NORD developed for the design, launch, and maintenance of rare disease natural history studies.”

Pamela Gavin – NORD Chief Operating Officer
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 it has ended and the patient is either cured, chronically disabled, or dead without external intervention.

A well conducted NHS should:

- Describe the disease overtime
- Identify demographic, genetic, environmental and other variables that are associated with the disease
- Define the disease population, including a description of the full range of disease manifestations and subtypes
Natural History Study Data

Potential uses of NH study data:

- Inform patient care and best practices
- Assess experiences and preferences
- Contribute to disease understanding
- Identify research priorities
- Estimate the number of affected patients and patients potentially available to participate in research
- Evaluate the individual and global economic burden of disease
- Inform drug development
NORD’s online Registry:

• Easy to use
• Built with input from all stakeholders
• Study sponsors (NORD member organizations) have access and control of surveys/questions; quality data; and reporting tools
• Registries hosted on a custom developed system in concert with Microsoft Azure cloud services
In collaboration with NORD member organization will develop, implement and manage natural history study

NORD will provide:

- Standardized registry on its Platform
- Standardized content for registry:
  - overview of the study (“about section”)
  - governance material
  - core NHS data set
NHS Set up Process

With support from NORD member will:

1. Develop a Project Advisory Committee
2. Develop a Study Plan/Protocol
3. Develop disease specific survey questions
4. Develop Patient Engagement and Retention strategy
Application Process (1)

Fill in online Natural History Project Application

**Organization Detail**
- Name of Organization
- Key Contact
  - Name
  - Title
  - Phone Number
  - Email Address

**Disease Detail**
- Disease or diseases intended for natural history study
- Name
- Physiological system
- Link to disease information
- Patient Engagement
  - How large is the US patient population?
Application Process (1a)

Ensure fulfill application criteria:

• 501(c)(3) Organizational Member of NORD
• Commitment and resources to administer a registry and collaborate with NORD for at least five years to properly evaluate outcomes
• Support from disease community and experts who will be able to contribute to the design and success of the project, including selecting disease-specific questions and ensuring patient engagement and retention
Consider scoring criteria:

• Unmet medical needs
• No existing or limited natural history data
• Research gaps that could potentially be addressed by a natural history study
Selection Committee:

• NORD representation from Membership, Research, Education
• FDA Project team
• Committee has 3 weeks to review applications
• 20 Applicants will be selected
Be aware of Timelines:

- January 29, 2016, 11:59 EST
  - Application period closes
- February 29, 2016 – Rare Disease Day!
  - Awards will be announced
Questions or Concerns

Contact:

• Jacqueline Kraska
  research@rarediseases.org
  203-304-7205
Experts agree that these registries are transforming patient/caregiver support and advocacy groups into research organizations. They also provide patients and family members another way to become engaged in research beyond the role of adviser or informant to researcher-generated studies.
Alone we are rare. Together we are strong.