Clinical Trials Road Map

NATURAL HISTORY STUDIES

WHAT IS A NATURAL HISTORY STUDY?

The Purpose of a Natural History Study Includes:

- Provide information to help researchers and clinicians understand the disease course over time (natural history), as well as validate animal models
- Better characterize the disease
- Characterize patient population and identify subgroups within the population
- Understand the day-to-day effects of the disease on the lives of patients and their families from their perspective and highlight areas of unmet medical need
- Inform research priorities from patient and clinical perspectives
- Establish diagnostic criteria
- Develop clinical care guidelines
- Identify biomarkers
- Collect patient-reported outcomes and other clinical outcomes, which can be used to identify outcome measures to be used in clinical trials
- Increase patient participation and retention in clinical trials
- Compile data from the patient perspective that can be used by the FDA as part of their marketing review process
- Amass data to advocate for expanded insurance coverage for therapies and services

WHAT IS THE PURPOSE?

WHY PARTICIPATE?

What Is a Natural History Study?

AN OBSERVATIONAL STUDY INTENDED TO TRACK THE COURSE OF THE DISEASE.

Its purpose is to identify demographic, genetic, environmental and other variables that correlate with the disease's development and outcomes. Information obtained from a natural history study plays an essential role at every stage of product development, such as identifying the patient population, and identifying or developing clinical outcome assessments and biomarkers.

Due to the small numbers of people affected by rare disease, there are unique challenges in understanding the disease and drug development for these conditions. One way for better understanding is through a natural history study.

Why Participate in a Natural History Study?

- The data from a natural history study can:
  - be used in FDA review of new therapies for rare disease resulting in regulatory approval and making the therapy accessible to those with the rare disease
  - be used as evidence with government policy makers when expressing the needs of people with rare diseases
    - Helps with the development of clear diagnostic criteria or clinical management guidelines by considering patient/caregiver burden
    - Supports the development of clinical trials by helping to design better study criteria
    - Raises awareness of disease in medical, academic, and pharmaceutical communities
    - Provides your patient community with a sense of momentum and increase interest in participation in research
    - Long term rewards include development of more effective, safe treatments
    - Ultimate reward is a cure for the disease

RESOURCES

www.fda.gov/orphan
The FDA Orphan Products Natural History Grants Program is a program funding natural history studies for rare diseases in order to guide therapy development

www.rarediseases.org
National Organization of Rare Disorders (NORD)

Connect with resources to help guide you through this journey
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