Current Challenges in Rare Disease Drug Development: Articulation of the Problem Statement

Kathleen M. Donohue, MD
Division of Gastroenterology and Inborn Errors Products (DGIEP)
U.S. Food and Drug Administration

Rare Disease Cures Accelerator Data & Analytics Platform
September 17, 2019
Disclosure Statement

• No conflicts of interest
• Nothing to disclose
• This talk reflects the views of the author and should not be construed to represent FDA’s views or policies
• In this talk “drug” refers to both drugs and biologics
Rare Disease Research?

The upside down house in Bispingen, Germany
Cures Accelerator
Patient Privacy & Data Security
Who: Team Members

- Patients
- Epidemiologists
- Geneticists
- Drug developers
What: Data

• Symptoms at diagnosis
• Molecular genetic diagnosis
• Predictors of clinical outcomes
• Background therapies
  – Diet, mobility assist devices, surgical procedures, respiratory support, etc.
• Time to events
• Endpoints
  – Mobile data
• Adverse Events
What: STROBE

• The Strengthening the Reporting of Observational Studies in Epidemiology (STROBE) Statement: guidelines for reporting observational studies

• http://www.equator-network.org/reporting-guidelines/strobe/
How: Data Standards

CDISC, MedDRA, audit trails, regulatory inspections
Better Data = Faster Cures