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

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Rare Disease Cures Accelerator Data & Analytics Platform
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• 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