

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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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?



Cures Accelerator



Patient Privacy & Data Security



Who: Team Members



- Patients
- Epidemiologists
- Geneticists
- Drug developers

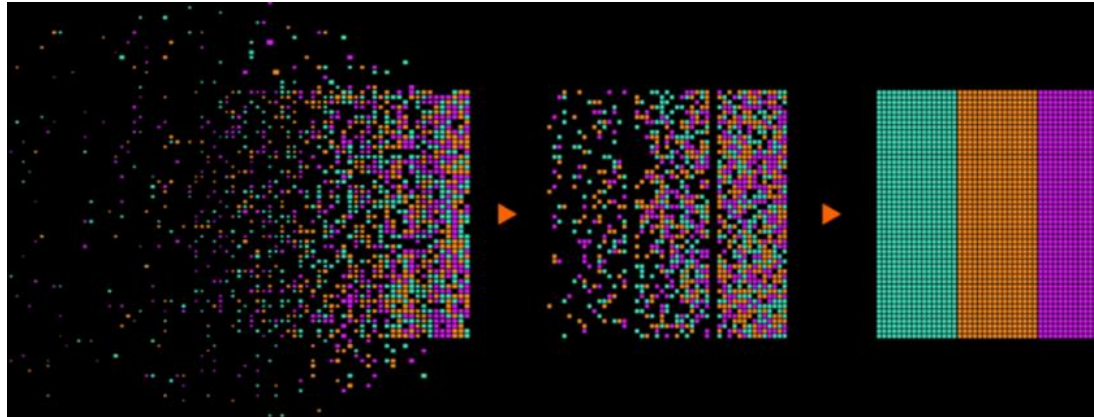
What: Data

- 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





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