WITH STANDARDS – UNLOCK THE POWER OF DATA

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Real world data as a bridge between industry and patients

Presented by Sophia Zilber, Rare Disease, Pfizer

Meet the Speaker

Sophia Zilber

Title: Real world data as a bridge between industry and patients

Organization: Pfizer

Sophia Zilber is a Sr. Statistical Programming Lead in Pfizer, where she's managing a team of statistical programmers, preparing the study and asset programming deliverables for submission. She has 20 years of experience with clinical data analysis. Her experience includes programming, creating study documentation, and establishing strategy, timelines, and resourcing of statistical programming and analysis deliverables, following CDISC and regulatory standards.

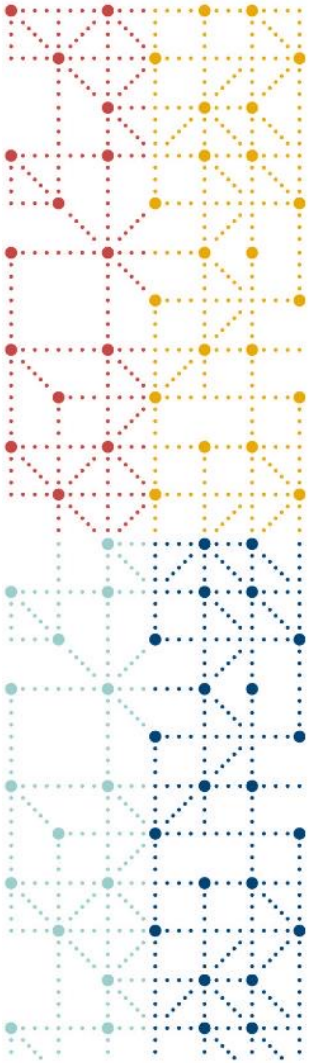
External to Pfizer, Sophia applies her passion for patient advocacy and data sharing through her involvement with rare disease community. Sophia is currently a board member for Cure Mito Foundation where she's also a patient registry director and has developed a global patient registry for Leigh Syndrome. Sophia has authored papers and has done multiple presentations on the topic of patient registries and data sharing.

Sophia believes that patients are critical to advancing treatments forward and that sharing knowledge, and open, honest and transparent communication are a key to a successful partnership.

Disclaimer and Disclosures

- *The views expressed in this presentation are the personal views of the author and may not be understood or quoted as being made on behalf of or reflecting the position of the regulatory agency/agencies or organizations with which the author is/are employed/affiliated and do not necessarily reflect the official policy or position of CDISC.*

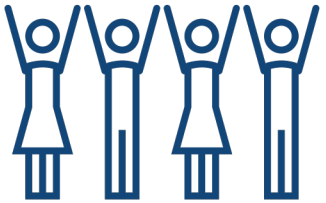
- *The author(s) have no real or apparent conflicts of interest to report.*



Agenda

1. Background
2. Patients-driven research: advantages, challenges
3. Patient registry for patient reported data – example
4. Aligning patient reported data to CDISC – use case
5. Discussion topics of above example
6. CDISC and Real World Data
7. Opportunities for pharma industry
8. Q&A

Background



Real World Data examples[1]:

- Electronic Health Records (EHRs);
- Medical claims and billing data;
- Data from product and disease registries; biobanks;
- Patient-generated data, including from in-home-use settings; and data gathered from other sources that can inform on health status, such as mobile devices

Patient Registries:

- Used to be fully maintained by hospitals or academic institutions
- As patients play a greater role in research, patient registries are frequently run by patients themselves, usually through patient advocacy foundations

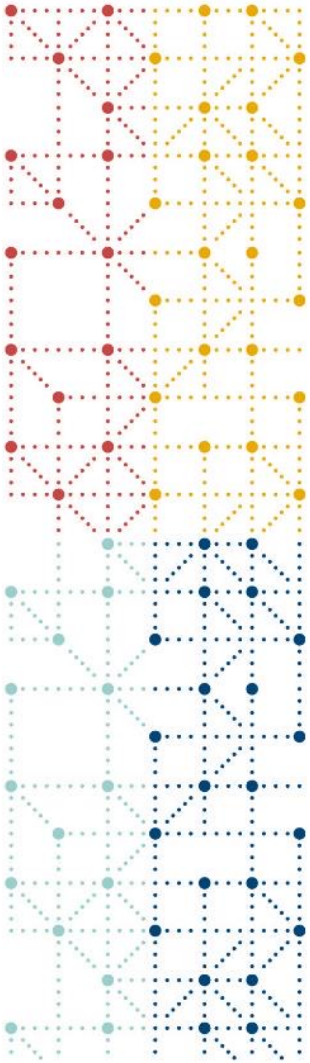
Advantages

- Patients have a voice in research
- Collecting data that's meaningful to patients
- Often quicker to collect data, easier outreach
- Data can be shared much easier than data collected in other ways (hospital, and other)
- Overall faster to advance clinical trials, treatments

Challenges

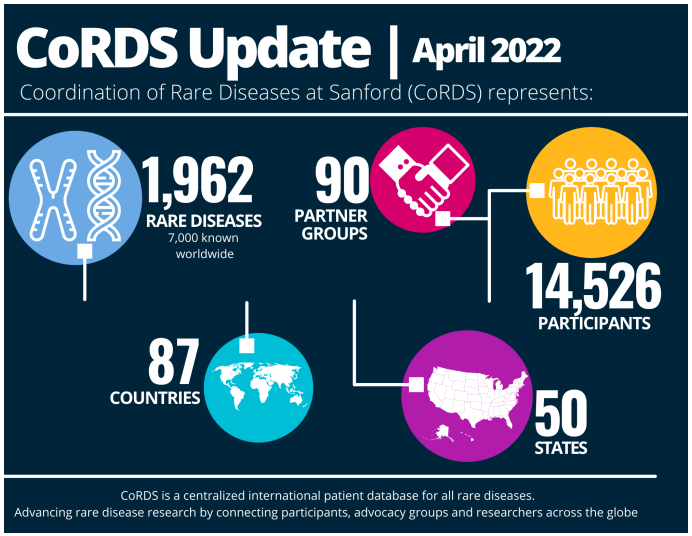
Quote from patient: *"I hope that something comes of this. I have done so many surveys and questionaires [sic] and NOTHING has ever come of it, NOTHING"*

- Lack of expertise (survey design, data management, data analysis)
- Poor data quality
- Data sometimes never analyzed or used
- Lack of clarity regarding data privacy and data ownership
- Inaccurate beliefs about data (examples: registry=cure, FDA/industry have high interest in the data, different datasets can be easily combined)
- Challenges resut in slowing down research, patients discouraged



Patient registry – patient reported data - example

Leigh Syndrome Global Registry – Cure Mito Foundation



Based at Sanford Research, a nonprofit research institution, CoRDS is a centralized international patient registry for all rare diseases.

We coordinate the advancement of research into 7,000 rare diseases. Here's how:

- We work with patient advocacy groups, individuals and researchers.
- We capture health information from individuals with a rare diagnosis, undiagnosed patients, unaffected carriers or at-risk patients.
- We connect researchers and patients and notify our participants of emerging clinical trials.
- We make the registry accessible. Participants can enroll for free and researchers can access it for free.

Source:https://research.sanfordhealth.org/rare-disease-registry

Leigh Syndrome Global Registry – Cure Mito Foundation

OBJECTIVES

Cure Mito Leigh Syndrome registry was started in September, 2021 to meet the following goals:

- Internationally available
- Identify and collect comprehensive information about LS patients population
- Facilitate clinical trials recruitment
- Share results and findings on an ongoing basis
- Build stronger patients and researchers community

Leigh Syndrome Global Registry – Cure Mito Foundation

General survey – based on NIH survey for rare diseases (Common Data Elements recommended by NIH)

- Exactly the same general/demographic survey for each rare disease supported by CoRDS
- Easy to harmonize data across rare diseases

Disease specific survey – developed separately for each disease

Linked to Orphanet list of rare diseases

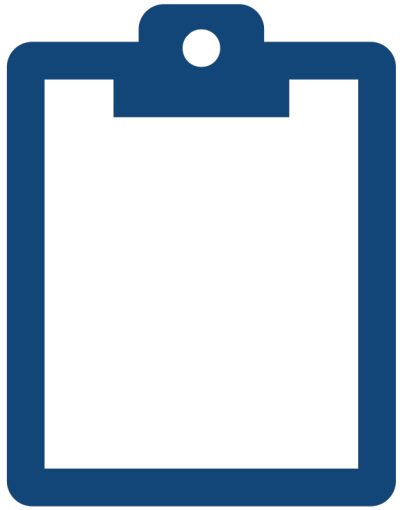
- Patient can join even if no advocacy group represented in CoRDS
- Joining based on disease

Multiple family members with same disease can be linked together

Leigh Syndrome Global Registry – Cure Mito Foundation

Information collected:

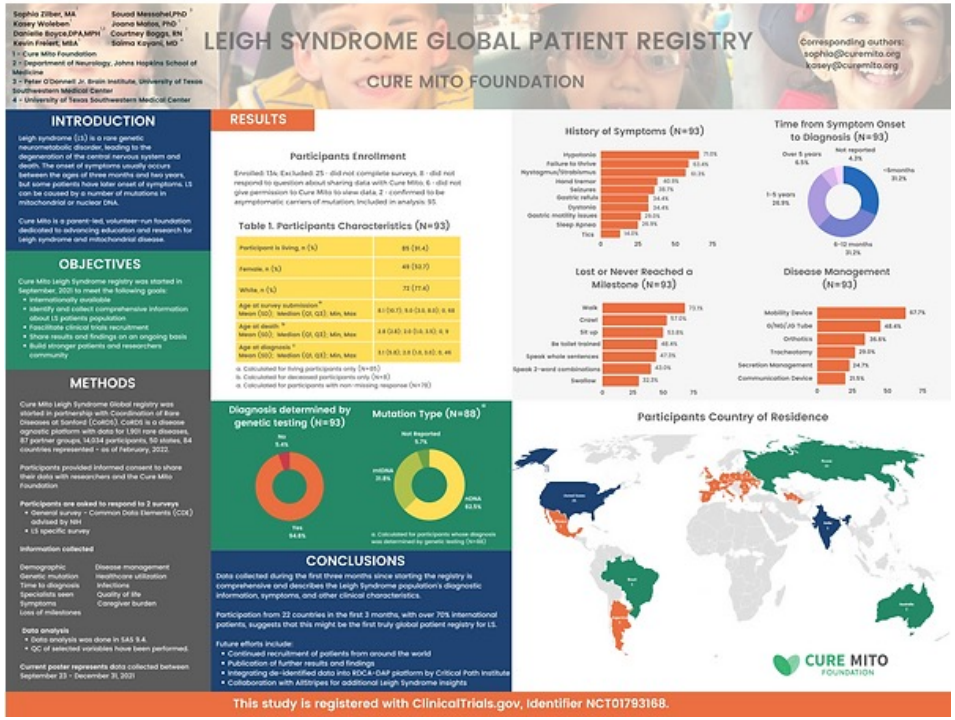
- Diagnosis and genetic mutation information
- Symptoms/concerns first noticed
- Loss of milestones
- Disease management
- Symptoms experienced
- Healthcare utilization
- Quality of life
- Caregiver burden



Poster

Mitochondria-Targeted Drug Development Summit, February 22-24, 2022

https://www.curemito.org/results



Poster

RESULTS

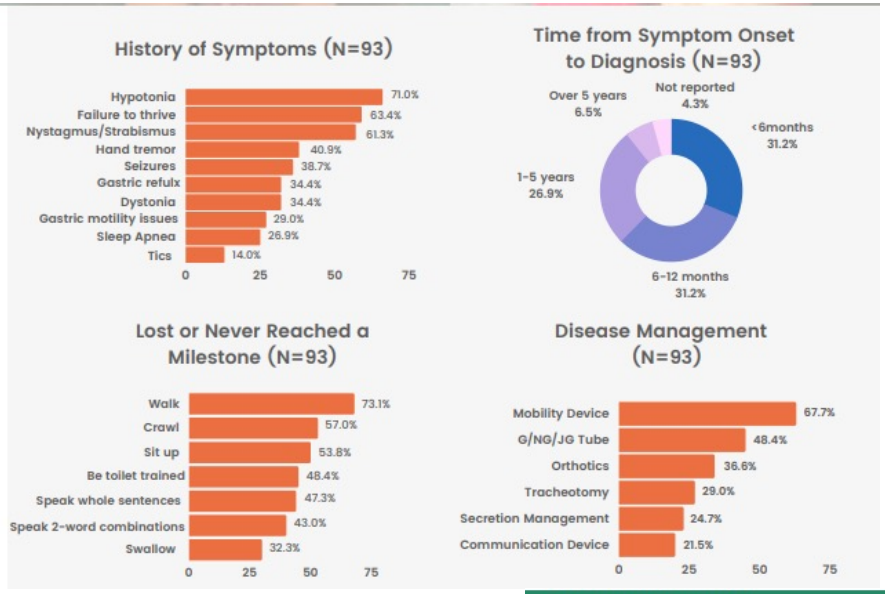
Participants Enrollment

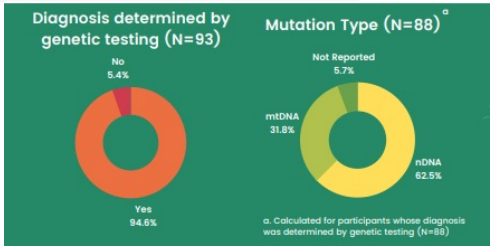
Enrolled: 134; Excluded: 25 - did not complete surveys, 8 - did not respond to question about sharing data with Cure Mito, 6 - did not give permission to Cure Mito to view data, 2 - confirmed to be asymptomatic carriers of mutation; Included in analysis: 93.

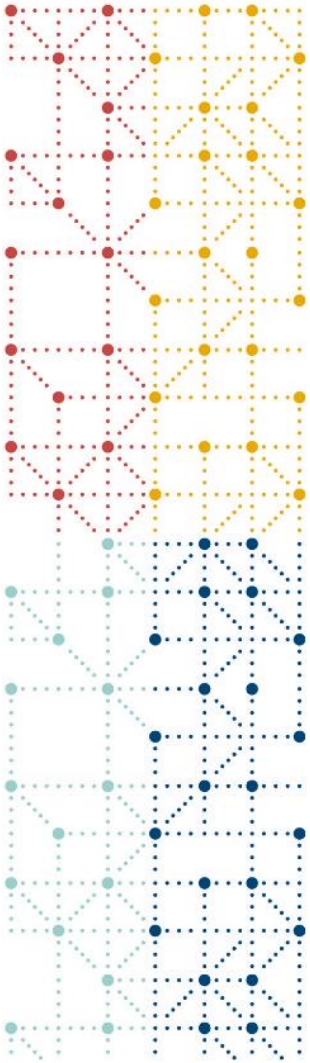
Table 1. Participants Characteristics (N=93)

Participant is living, n (%)	85 (91.4)
Female, n (%)	49 (52.7)
White, n (%)	72 (77.4)
Age at survey submission [a] Mean (SD); Median (Q1, Q3); Min, Max	8.1 (10.7); 5.0 (3.0, 8.0); 0, 68
Age at death [b] Mean (SD); Median (Q1, Q3); Min, Max	2.8 (2.8); 2.0 (1.0, 3.5); 0, 9
Age at diagnosis [c] Mean (SD); Median (Q1, Q3); Min, Max	3.1 (5.8); 2.0 (1.0, 3.0); 0, 46

a. Calculated for living participants only (N=85)
b. Calculated for deceased participants only (N=8)
a. Calculated for participants with non-missing response (N=78)



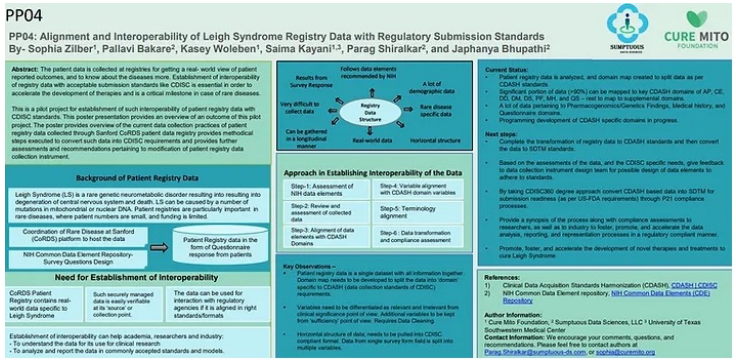




Interoperability of Leigh Syndrome Patient Registry Data with Regulatory Submission Standards

Interoperability of Leigh Syndrome Patient Registry Data with Regulatory Submission Standards

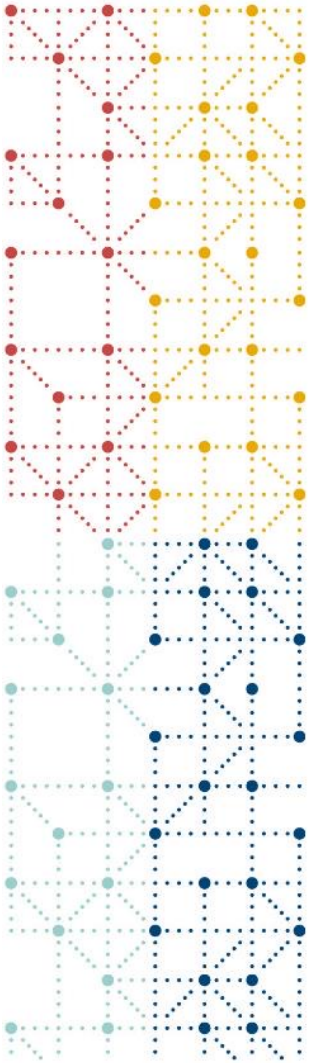
- Collaboration with Sumptuous Data Sciences (sumptuous-ds.com)
- CDISC Standards are required for regulatory submissions to FDA (U.S.) and PMDA (Japan) (cdisc.org).
- Goals:
  - Learn if the data is compatible with CDISC
  - Convert data to CDASH and then SDTM
  - Have data that is regulatory submission ready
- Results
  - >90% of data maps to existing CDISC domain
  - Conversion in progress
- Next steps?



Opportunities for pharma industry

Guidance for patients and patient advocacy groups	Guidance for researchers	Why is this important?
• Understanding of drug development process • Understanding of data and how it's used • Limitations of data • Explain data management, data analysis • Explain value/possible use of patient-reported data, electronic health records	• Explain data from industry perspective • Explain CDISC • Value of CDISC standards • Individual use cases	• Patients are highly motivated • A lot of money, time, effort already invested into collecting data • By identifying and addressing the gaps, we can put patients into the center and get to treatments and cures faster

PHUSE workgroup: Best Data Practices for Rare Disease Patient Foundations and Researchers
https://advance.phuse.global/display/WEL/Best+Data+Practices+for+Rare+Disease+Patient+Foundations+and+Researchers



Thank You!

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