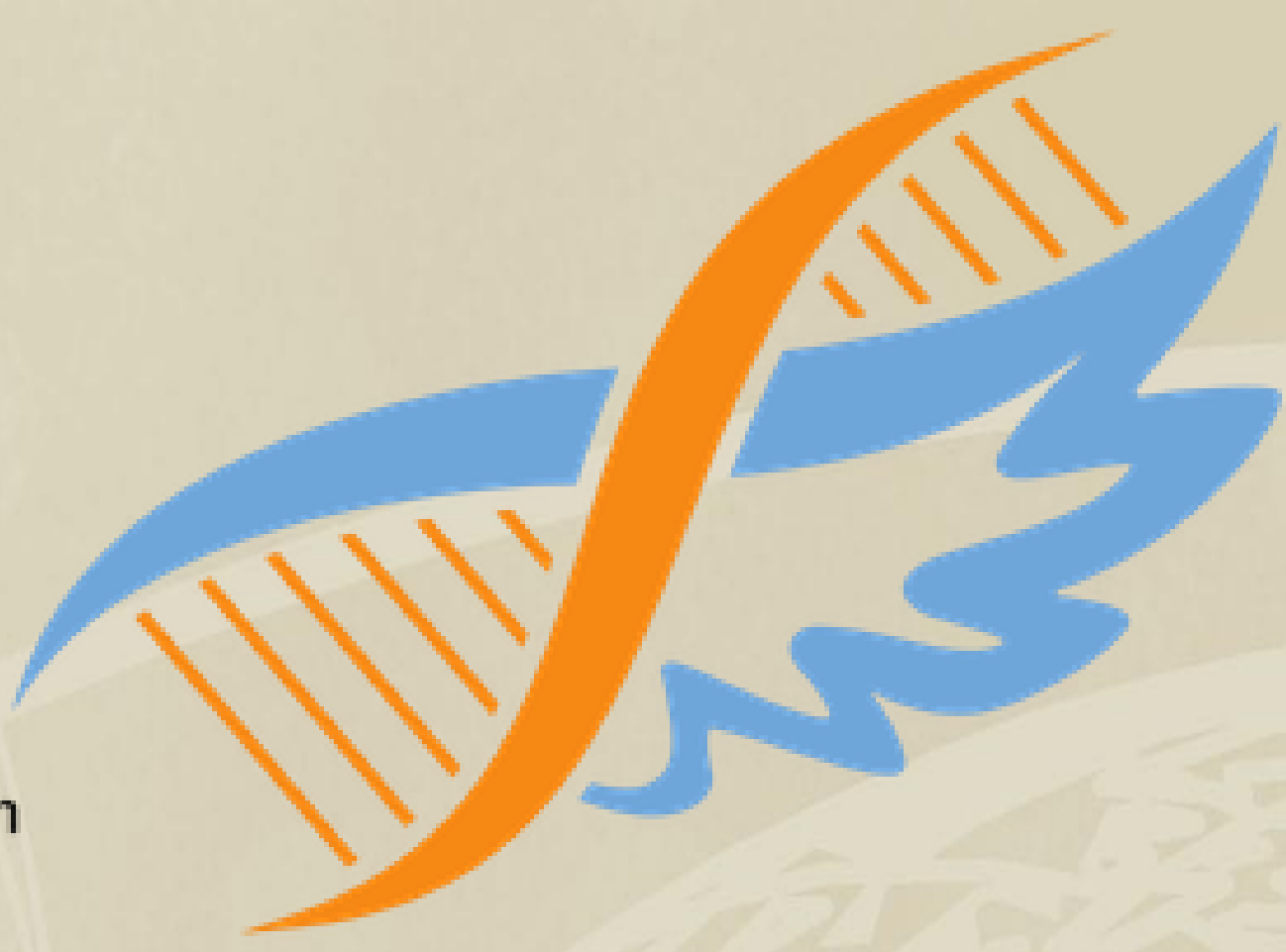


LADDER Database

Linking Angelman & Dup15q Data for Expanded Research



RTI INTERNATIONAL

angelman SYNDROME FOUNDATION

Dup15q Alliance
FOR 15Q 11.2-13.1 MULTIPLICATIONS

Goals of the LADDER Database

Curate and harmonize existing data on individuals with Angelman or Dup15q from multiple settings in order to:

- Accelerate knowledge about phenotypes, medical and developmental trajectories, and family needs
- Identify and promote effective treatment guidelines
- Facilitate outcome measure development and validation for use in clinical trials

Facilitate data access and sharing to:

- Protect privacy of the individuals and families
- Streamline use of the data to meet the priorities of the community

Synergize data collection across the LADDER clinic network

- Clinical Needs Survey—common data elements collected in all AS clinics

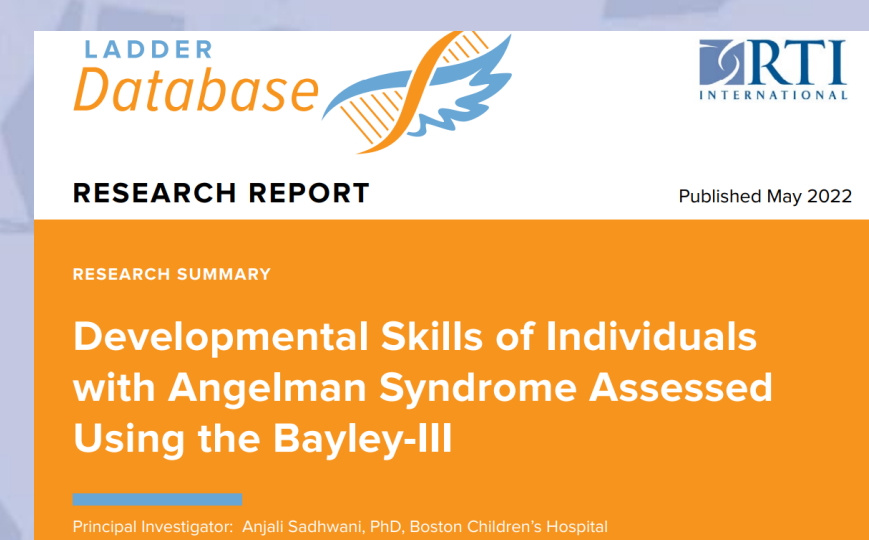


Data Curation and Facilitation

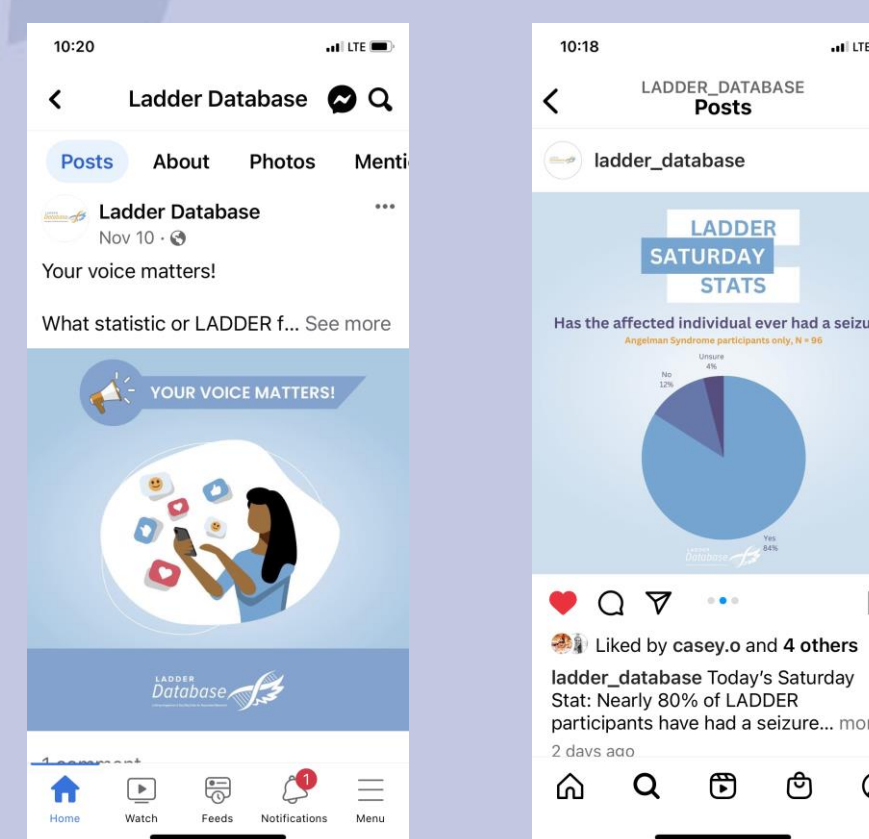
- ❖ Nearly **700** participants enrolled
- ❖ **81** unique datasets
- ❖ Over **60,000** historical data points
- ❖ The Data Access Committee has approved **twenty** applications to receive data
 - ✓ Industry/Pharma companies
 - ✓ Academic researchers
 - ✓ Graduate students
- ❖ **Two** applications for Level 3 (for recruitment and data collection) have been approved

Community Engagement

- ❖ Family-friendly reports on key research studies



- ❖ Weekly feedback through social media posts



Example of Data Use

Meaningful change on core outcome measures
Statistical study to identify the smallest change on the Bayley and Vineland measure that would be considered meaningful to caregivers

One Data Source: 107 Vinelands, 94 Bayleys

Two Data Sources: 319 Vinelands, 237 Bayleys

Three Data Sources: **1291 Vinelands, 1475 Bayleys**

- ❖ Rapid deployment of surveys to answer clinical questions and inform future research

- ❖ Seizures
- ❖ Quality of life
- ❖ Early Experiences

Acknowledgements

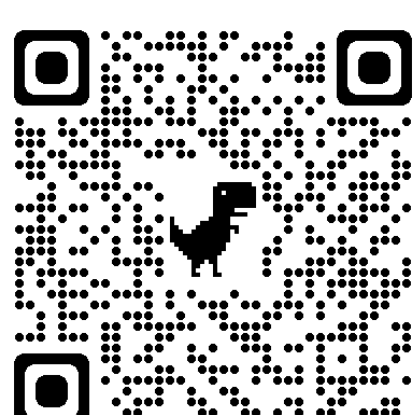
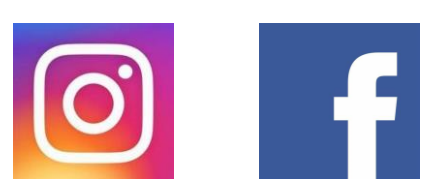
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Find out more:

<https://www.laddertotreatment.com/>



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