



Department of Population
Health Sciences

Duke University School of Medicine



Observer-Reported Communication Ability Measure

Novel caregiver-reported outcome measure for individuals with Rett syndrome

Bryce B. Reeve, PhD

Professor of Population Health Sciences

Professor of Pediatrics

Director of Center for Health Measurement

Bryce.Reeve@Duke.edu

Center
for | **Health
Measurement**

person centered • evidence driven



Overview

1. The Importance of Good Quality Measures in Clinical Research Settings.
2. Are there acceptable regulatory-grade measurement tools available to assess communication in Rett syndrome?
3. Overview of the Observer-Reported Communication Ability (ORCA) measure.
4. Evaluation of the ORCA measure in Rett syndrome.

Disclosures

The study team members have developed the technology being discussed. If the technology is commercially successful in the future, the developers and Duke University may benefit financially.

Views expressed are my own.

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Team



Bryce Reeve, PhD



Nicole Lucas, BS



Molly McFatrigh, MPH



Christy Zigler, PhD, MEd



Leslie Zapata Leiva



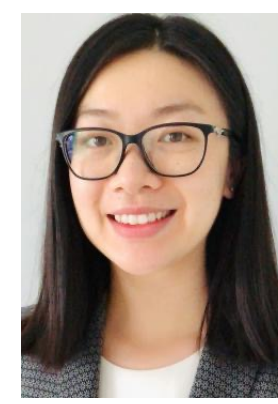
Kelly Gordon, CCC-SLP, MS



Harrison Jones, PhD



Li Lin, MS



Dandan Chen, PhD



1.

The Importance of Good Quality Measures in Clinical Research Settings.

Clinical Research: Helps us to learn more about a disease and ways to improve health care for people in the future.

- **Clinical Trials:** Determine if an intervention is safe and effective

Are we measuring what matters?

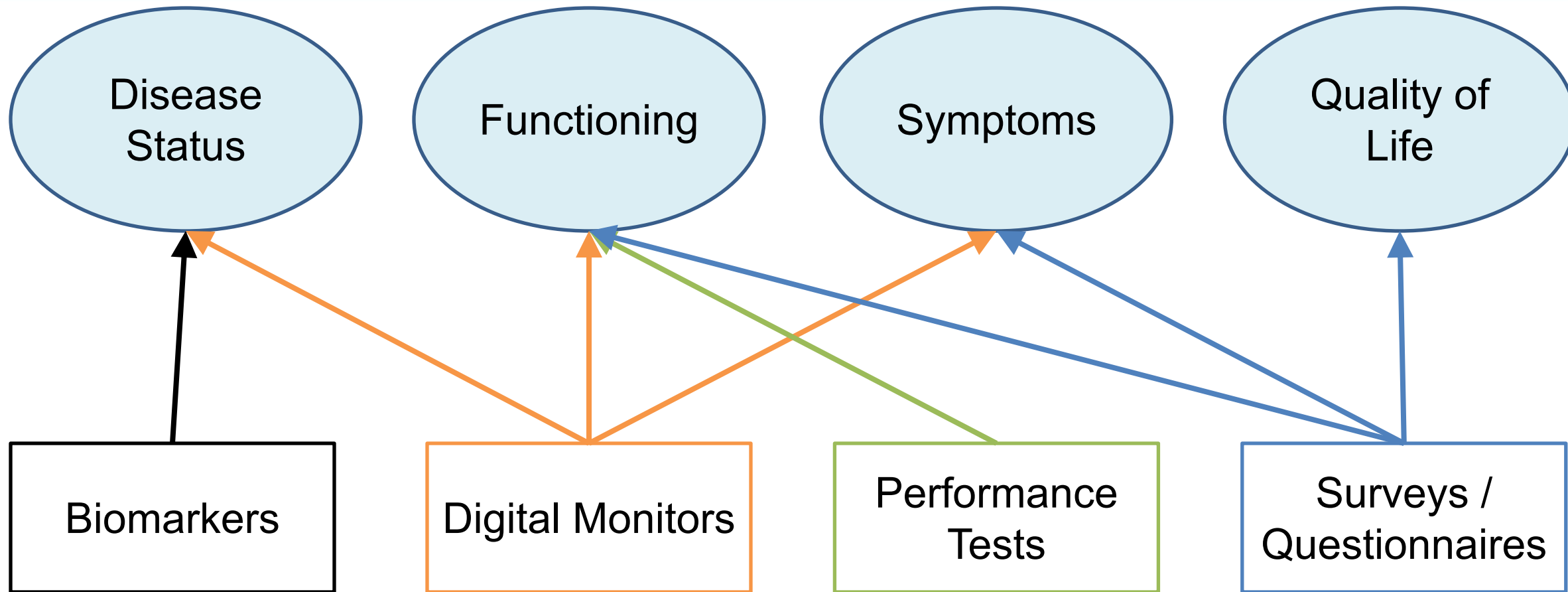
- Disease status: Brain function, Bone health
- Functioning: Cognition, Motor skills, Communication
- Symptoms: Seizures, Sleep disturbance, Gastro-intestinal distress, Depression, Anxiety
- Quality of Life: Relationships, Family impact



Patient-Centered
Outcomes Research



Patient-Focused
Drug Development





- Need regulatory-grade measures to assess change in outcomes over time.
- Measures should be validated for Rett syndrome.
- All measures have error.
- Tools for the same “outcome” should be related, but not perfectly as they assess different aspects.



2.

Are there acceptable regulatory-grade measurement tools available to assess communication in Rett syndrome?



Limitations of existing measures

1. Most measures require an SLP to complete. Children perform differently in a clinic/laboratory setting and cannot typically demonstrate their highest abilities.
2. Not designed as an outcome measure for a clinical trial.
3. Not designed based on best practices for designing and evaluating questionnaires.
4. Did not include parent advocates in oversight and direction of questionnaire design.
5. Most measures focus on verbal speech as indicator for ability.
6. Many children with Rett syndrome unable to be differentiated on the score metric (i.e., basement effect).
7. Not sufficient evidence for the quality of the measure for individuals with Rett syndrome.

3.

Overview of the Observer-Reported Communication Ability measure.



Observer-Reported Communication Ability Measure

Key Features of ORCA measure

- Caregiver/Parent completes questionnaire independently; does not require administrator
- Assesses a broad range of communication concepts
- Allows multiple communication modalities (important for non-verbal populations)
- Designed for use in clinical trials
- Originally developed for Angelman syndrome, could be applicable to other neurodevelopmental disorders

Concepts contributing to estimating communication ability scores on the ORCA measure

Expressive Communication	Receptive Communication	Pragmatic Communication
Seek Attention	Respond to Name	Greeting
Direct Attention	Understand Mood	Comfort Others
Refuse Object	Understand Isolated Words	Play Games
Request Object	Turns in Conversation	Use Names
Request Object Out of View	Make Choices	
Request "More"	Respond to Familiar Directions	"Vocabulary"
Communicate Understanding	Respond to New Directions	Number of verbal words
Asking Questions	Answer Questions	Number of symbols on an assistive device
Communicate with Others		
Telling About the Past*		

Refusal

Cry or fuss

Turn their head away

Push the object away

Throw the object

Shaking head "no"

Gesture for "no"

Indicated "No" on device

Say "no"

Refusal

Cry or fuss

Turn their head away

Push the object away

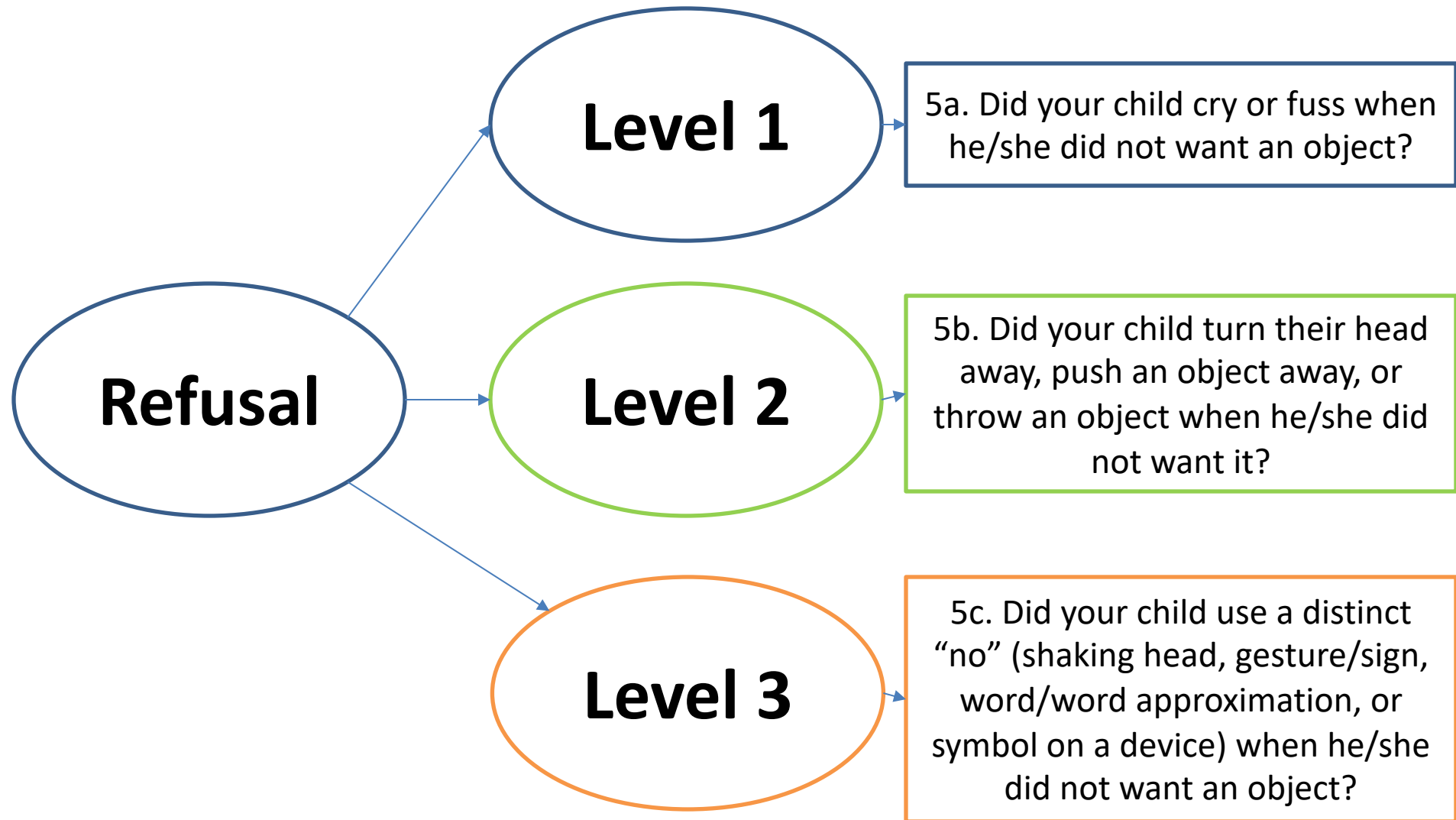
Throw the object

Shaking head "no"

Gesture for "no"

Indicated "No" on device

Say "no"



Refusal questions

5. Please tell us how your child refused an object like a book, toy, or food.

		No or only once	Sometimes	Yes, almost all the time
	In the past 30 days,			
5a.	Did your child <u>cry or fuss</u> when he/she did not want an object?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5b.	Did your child <u>turn their head away, push an object away, or throw an object</u> when he/she did not want it?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5c.	Did your child <u>use a distinct “no” (shaking head, gesture/sign, word/word approximation, or symbol on a device)</u> when he/she did not want an object?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

4.

Evaluation of the ORCA measure in Rett syndrome.



Approaches to Evaluate ORCA Measure with Caregivers of Individuals with Rett Syndrome

- A. Qualitative interviews
- B. Longitudinal observational study



Qualitative Interview Goals

- Understand typical communication types and behaviors observed in individuals with Rett syndrome.
- Determine if caregivers can understand the ORCA measure's instructions, questions and response options.
- Evaluate how well the ORCA measure captures key communication concepts in individuals with Rett syndrome.



Qualitative Interview Approach

- One-on-one interviews approximately one hour:
 - a) Concept Elicitation – typical communication
 - b) Complete ORCA measure independently
 - c) Cognitive Interviewing – comprehension & relevance
- Sampling by age: 2-7, 8-12, 13-17, 18+ years
- Two rounds of interviews to evaluate changes to the ORCA measure

Qualitative Interview Sample Characteristics (n = 19)

Individual with Rett syndrome	
Gender	19 females
Age (average, range)	13.3, 4-36 years
Race/Ethnicity	11 N-Hisp White, 4 Asian, 2 Black, 1 Middle Eastern, 1 Hispanic
Caregiver	
Gender	16 females
Age (average, range)	47.2, 34-73 years
Household Income (median)	\$100,000-\$250,000 (n=4 <\$80,000)

Qualitative Interview Key findings

- A. Gestures (eye gazes) heavily used. Device use not common among adults.
- B. Discussed variety of concepts included in ORCA (making choices, seeking attention).
- C. Completion time: 15-20 minutes
 - Somewhat (74%) or Very Easy (21%) to complete
 - Captured their child's communication Somewhat Well (32%) or Very Well (63%)

“Most of the questions were questions that I could answer without wanting to write down [my child is] “physically unable””

“It's much better than a normal questionnaire, where they would not give credit to an eye gaze gesture or the body movement or anything like that. So, this accommodated that and gave her credit that she is communicating this way.”

Qualitative Interview Key findings (continued)

- Minor modifications to ORCA measure - for example:
 - Emphasized eye gaze, pointing, and head nodding were specific gestures.
 - Some of the original examples required mobility; e.g., “Get your cup”
“Let’s take a bath”
 - Added “Give me a kiss”

Longitudinal Observational Study Goals

- Document evidence for the ORCA measure's
 - **Validity** – measure what it is supposed to measure.
 - **Reliability** – provide consistent and reproducible scores.

Longitudinal Observational Study Approach

- Survey with caregivers at two times
 - 279 Caregivers of females with Rett syndrome
 - 210 Caregivers @ follow-up (5-12 days later)
 - 8 Caregivers of males with Rett syndrome
 - Completed:
 - Refined ORCA measure
 - Communication and Symbolic Behaviors Scale (CSBS)

Demographic Data – Females with Rett syndrome (n = 279)

Age, years (Mean/SD)	13.7 (10.1)
Ethnicity	
Not Hispanic or Latino	90%
Hispanic- Latino	10%
Race	
White	92%
African-American or Black	3%
American Indian/Alaska Native	<1%
Asian	5%
Middle Eastern	1%
Native Hawaiian/Other Pacific Islander	<1%
More than one race	7%

Demographic Data – Females with Rett syndrome (n = 279)

Hours/wk in school (mean, SD)	21.9 (14.6)
Age start speech therapy (mean, SD)	2.2 (1.0)
Therapy	
Physical Therapy	80%
Occupational Therapy	70%
Speech Therapy	70%
Other	26%
AAC device	
No	30%
Yes	70%
Device	
High tech	67%
Low tech	11%
Places to use the device	
Home	53%
School	56%
Out in the community	11%
Other	6%

Demographic Data – Caregivers (n = 279)

Female	90%
Age, years (Mean, SD)	45.0 (10.4)
Ethnicity	
Not Hispanic or Latino	91%
Hispanic- Latino	8%
Race	
White	91%
African-American or Black	1%
Asian	5%
Middle Eastern	1%
More than one race	1%
Relationship status	
Single, never married	4%
Married, or living with domestic partner	86%
Separated, Divorced, Widowed	10%

Demographic Data – Caregivers (n = 279)

Occupational status	
Homemaker	25%
Unemployed	3%
Retired	5%
On disability	2%
On leave of absence	<1%
Full-time employed	44%
Part-time employed	20%
Full-time student only	<1%
Income	
Less than \$20,000	3%
Between \$20,001 and \$40,000	8%
Between \$40,001 and \$60,000	9%
Between \$60,001 and \$80,000	11%
Between \$80,001 and \$100,000	10%
Between \$100,001 and \$250,000	33%
Between \$250,001 and \$500,000	16%
\$500,000+	4%
I prefer not to answer / don't know	7%
Non-US	n = 89

Demographic Data – Caregivers (n = 279)

Highest grade in school	
Less than high school diploma	1%
High school degree or equivalent	7%
Some college/University	15%
College/University degree	42%
Postgraduate degree	36%

Language (spoken in home)	
English	85%
Spanish	3%
Hindi	1%
Portuguese	1%
French	1%
German	1%
Italian	2%
Other	6%

Demographic Data – Caregivers (n = 279)

Country live in	
United States	70%
Canada	6%
United Kingdom	7%
Australia	3%
Italy	2%
Israel	2%
18 other countries	10%

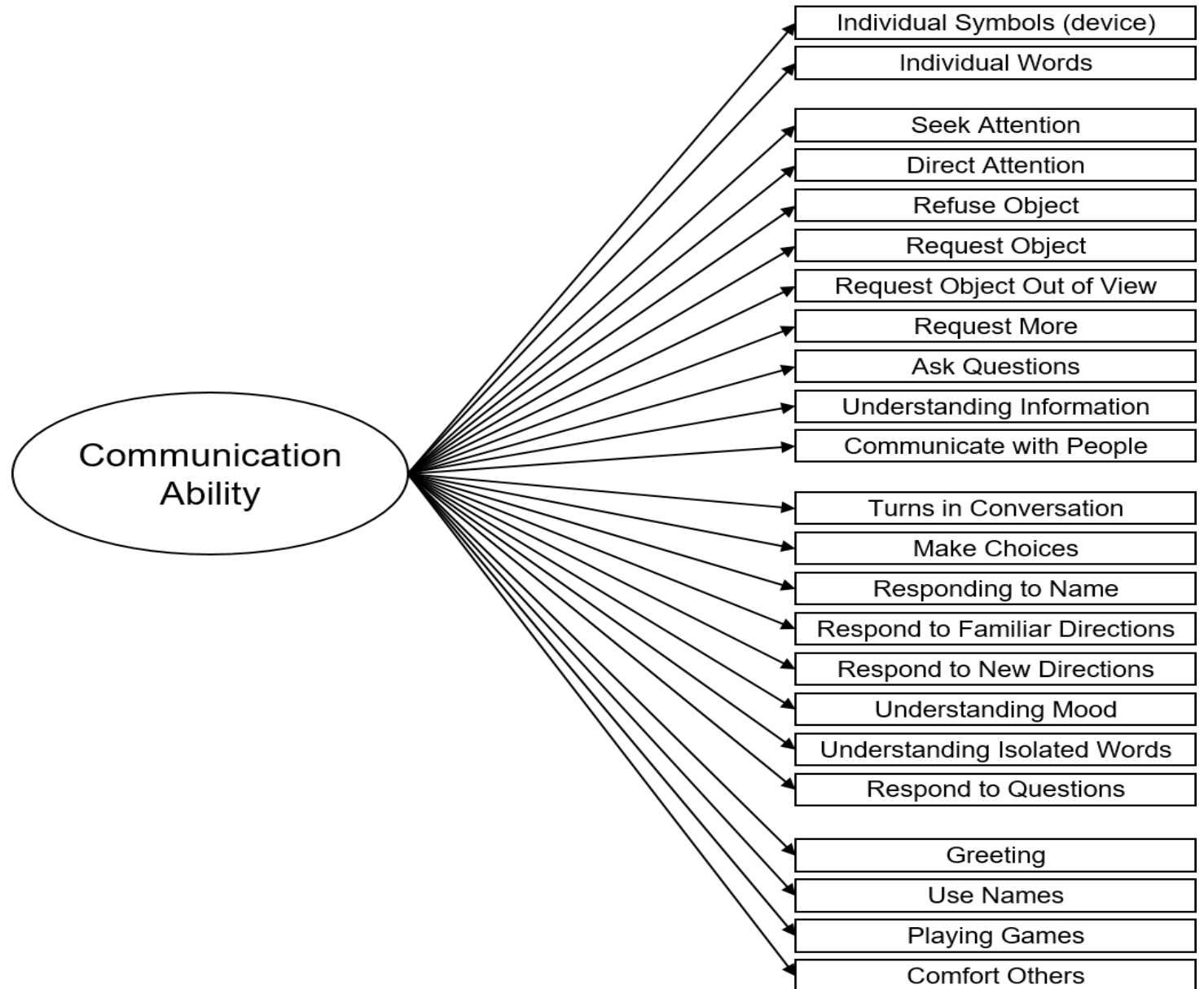
Evaluation of Validity and Reliability of ORCA Measure

1. Structural Validity
2. Score Distribution
3. Reliability
4. Convergent Validity
5. Known Groups Validity



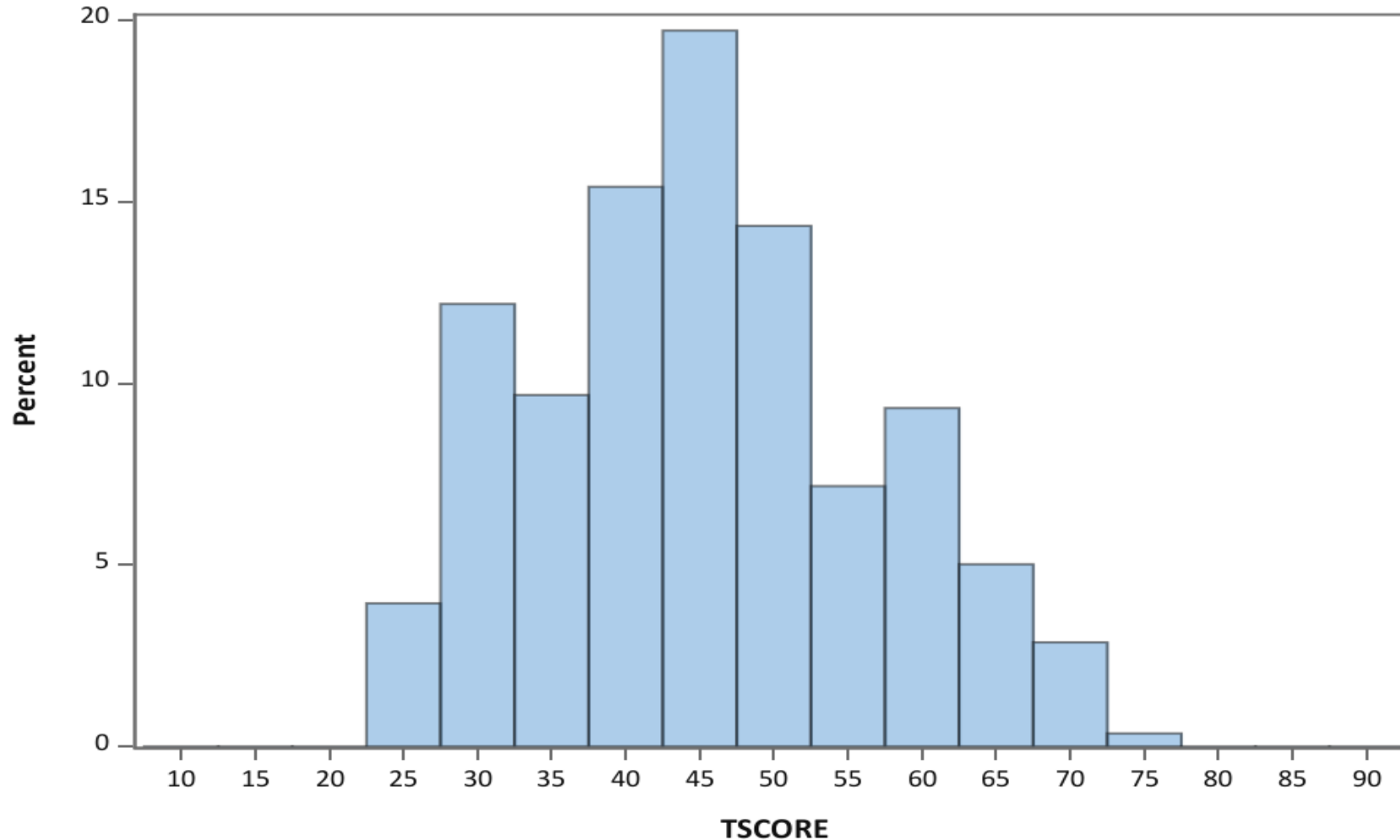
Structural Validity

- The 23 communication concepts included in the ORCA measure are associated; allowing us to calculate a single score that reflects their overall communication ability.



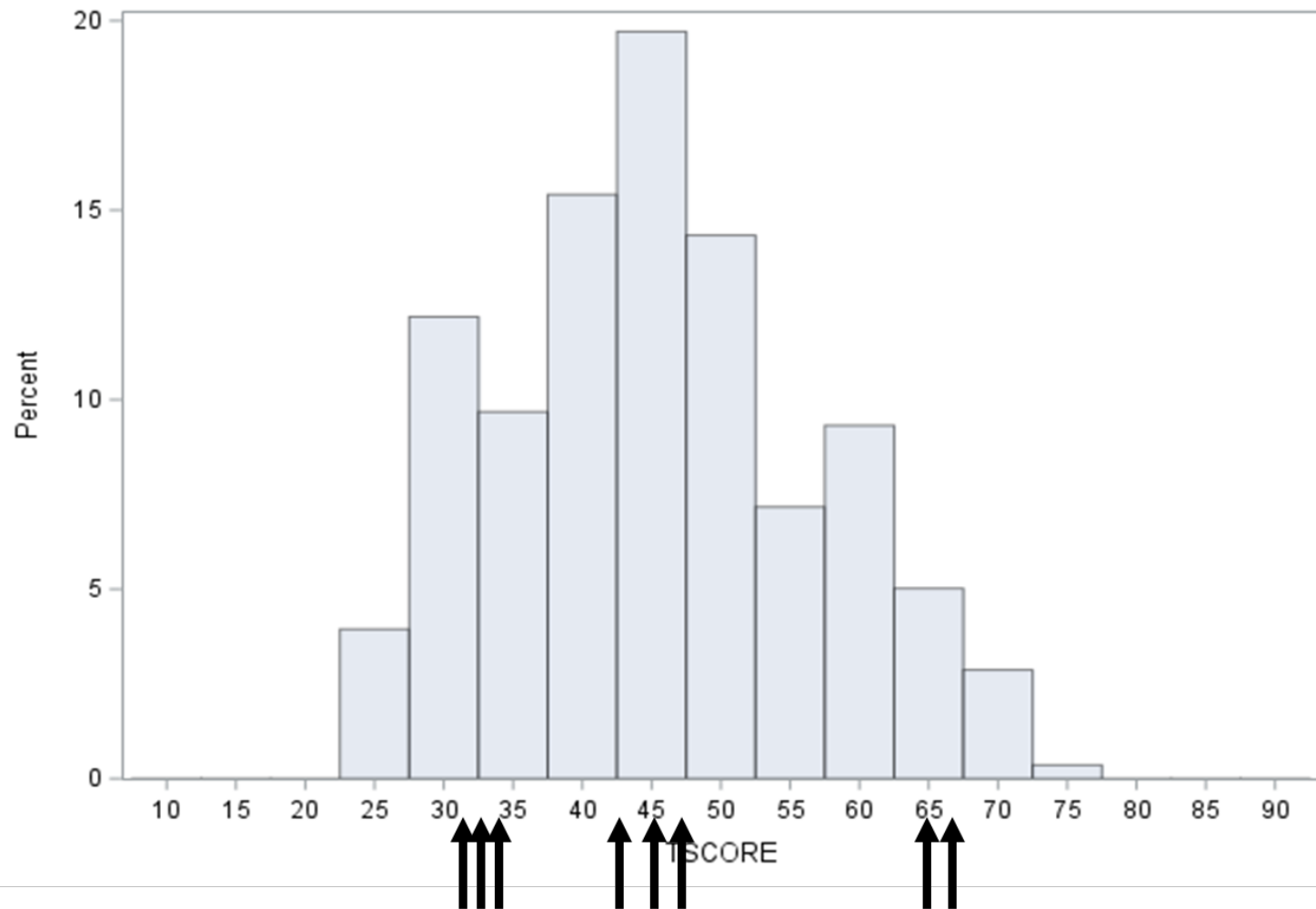
ORCA Score Distribution

(n = 279 females with Rett syndrome)



11 Females were at lowest score (26.8), and no females at highest score (83.2)

ORCA Score Distribution (n = 8 males with Rett syndrome)

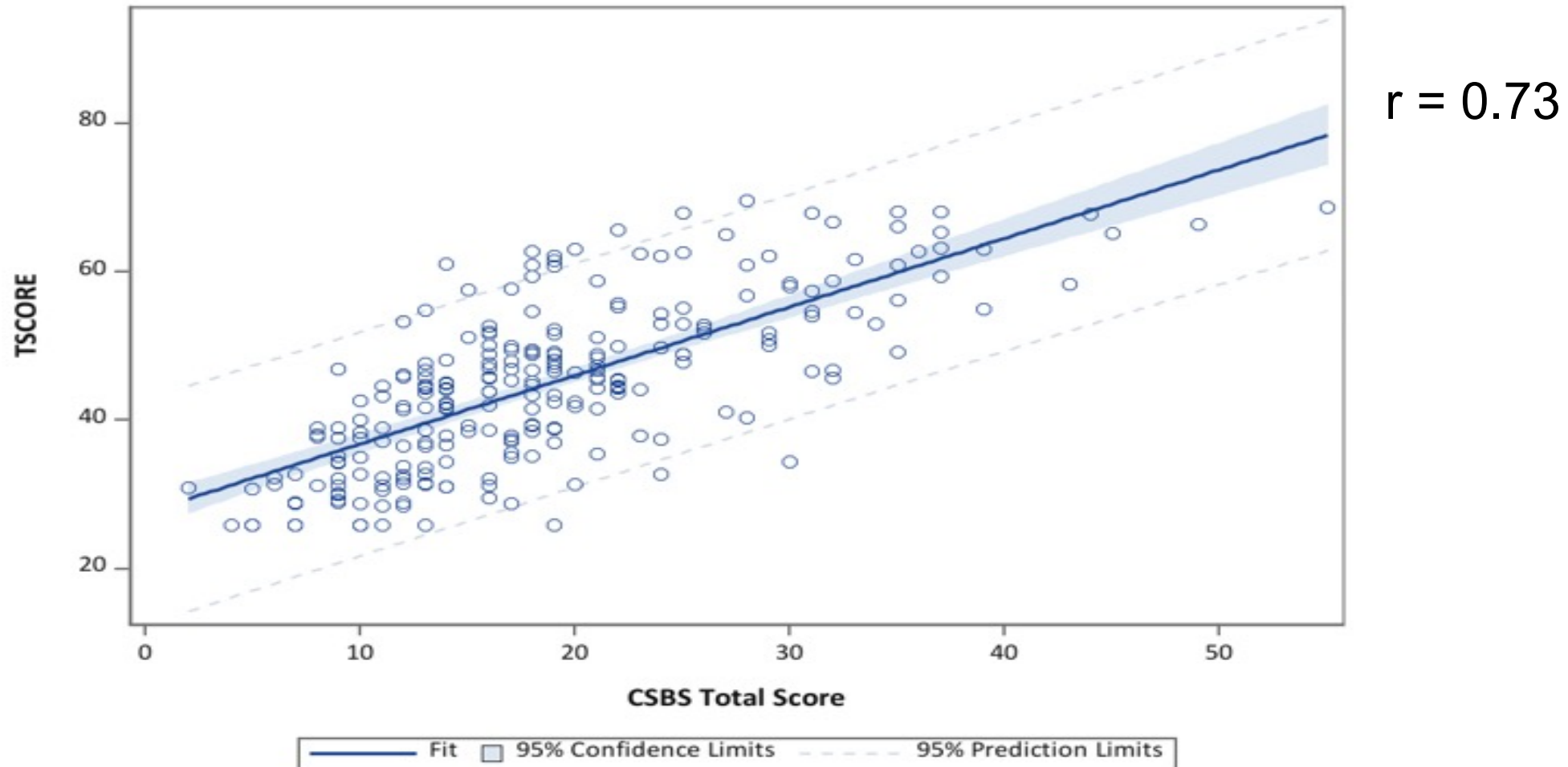


Age	Score
1	33.2
5	66.4
6	45.9
7	42.8
8	65.7
9	34.7
11	47.2
47	31.9

Reliability

- How well do individuals with Rett syndrome with true differences in their communication ability are able to be distinguished from each other, after accounting for the presence of measurement error.
- ORCA measure found to have high reliability:
 - Internal consistency: $\alpha = 0.90$
 - Test-retest reliability: ICC = 0.88

Convergent Validity (extent the ORCA measure associates with another communication measure (CSBS))



Known Groups Validity (extent the ORCA measure can differentiate between groups known to be different)

	n (%)	ORCA Mean (STD)	95% Confidence Limits for Mean
AAC device			
No	84 (30.1%)	39.3 (9.6)	37.2 – 41.4
Yes	195 (69.9%)	47.8 (10.8)	46.3 – 49.4

Conclusions

- A. ORCA measure was designed for use in trials and to be completed independently by caregivers.
- B. Initial evidence supports use of ORCA in Rett syndrome.
- C. More validation work could be done:
 - Responsiveness to change & meaningful change
 - Association with other communication measures
 - Diverse samples
- D. FDA funded our group to expand the ORCA to other rare neurodevelopmental disorders.

Access



<https://populationhealth.duke.edu/research/center-health-measurement/observer-reported-communication-ability-orca-measure>



<https://pattern.health/exchange/orca-observer-reported-communication-ability-measure/>



Observer-Reported Communication Ability Measure

- We are grateful for the **families** who participated in the development process for the ORCA measure and generously donated their time.
- Thank you to our funders and collaborators.