



# *Validation of the Observer-Reported Communication Ability (ORCA) Measure*

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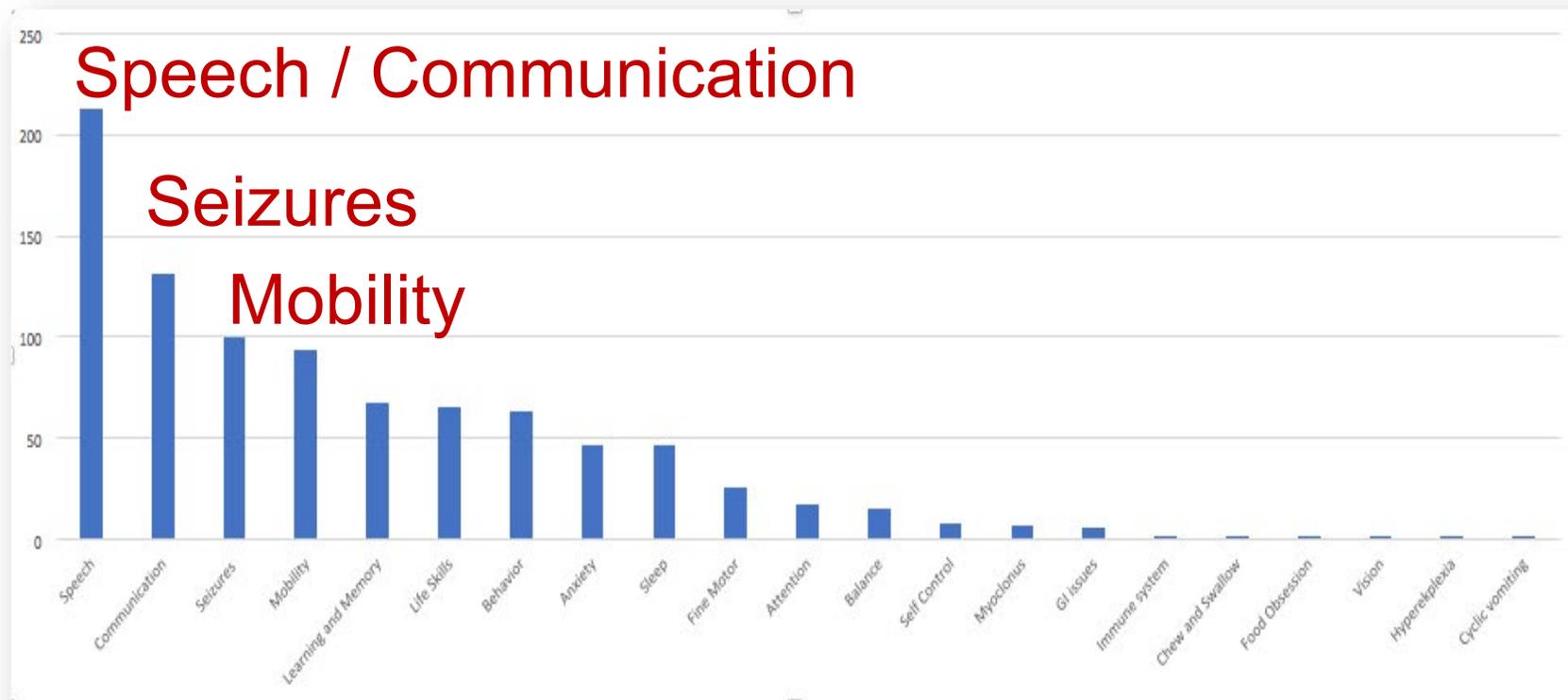
Duke University School of Medicine

Center  
for | **Health  
Measurement**

person centered • evidence driven

# What outcomes to measure as an indicator of an effective therapeutic for your child?

- 332 Parents/Caregivers responses to January 2018 FAST Survey
  - Ages of child: 0.5 – 44 years (median 9 years)



# Is there an existing **communication** measure that would meet the qualifications of the FDA?



- Limitations of existing measures:

1. Most measures require an SLP to complete. Children perform differently in a clinic/laboratory setting and cannot demonstrate their range of 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 Angelman Syndrome are 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 Angelman Syndrome.



## Overall Goal

Integrate a caregiver-reported measure of communication ability in forthcoming clinical trials to evaluate benefit of therapeutics for AS.

## Aims

1. Design caregiver-reported measure of communication ability of individuals with AS.
2. Provide evidence for the reliability and validity of the measure.

# Team

## **FAST Representatives**

- Paula Evans
- Allyson Berent
- Jennifer Panagoulas

## **Advisors**

- Emily Quinn
- Samuel Sennott

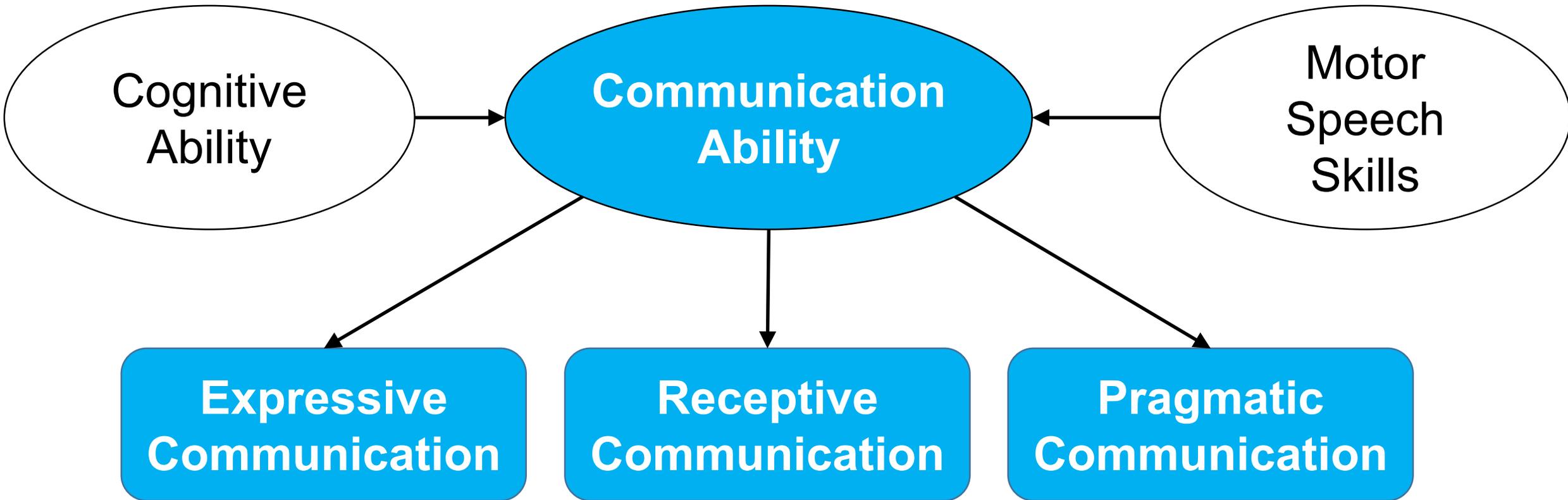
## **Duke University**

- Kelly Gordon
- Harrison Jones
- Li Lin
- Nicole Lucas
- Molly McFatrach
- Bryce Reeve
- Christy Zigler

# 7+ Step Process to Design and Evaluate the ORCA

1. Formative Research
2. Concept Elicitation
3. Draft Survey
4. Cognitive Testing
5. Translatability Review
6. Psychometric Testing
7. Dissemination and Implementation

# 1. Formative Research: Determine what is the concept we want to measure.



2. Concept Elicitation: Collect qualitative data from experts to better understand the concept of interest.



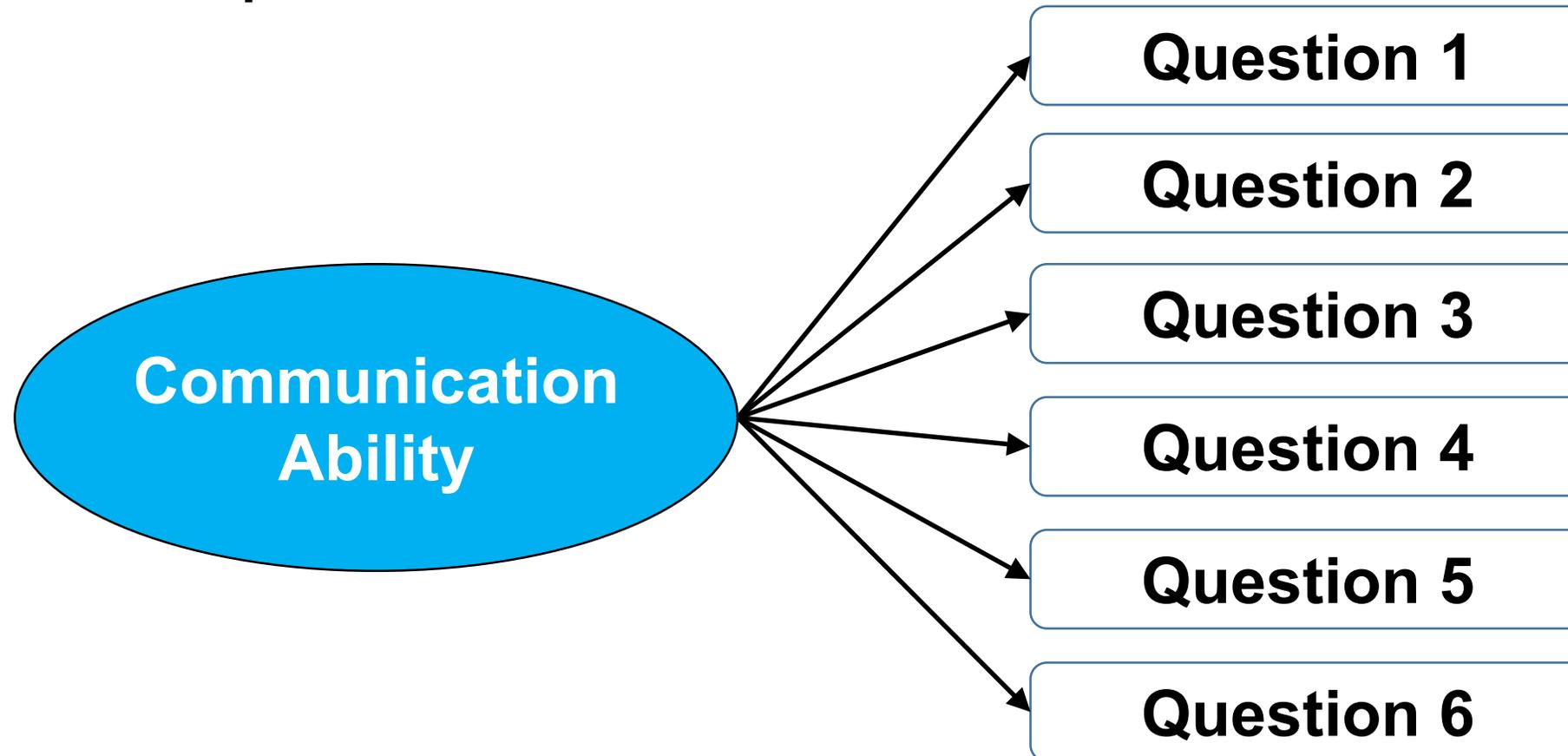
*On a typical day, what kinds of things does your child communicate about?*

*What would a meaningful change in communication look like for your child?*

# The 22 Communication Concepts currently included in the ORCA Measure

<b>Expressive Communication</b>	<b>Receptive Communication</b>	<b>Pragmatic Communication</b>
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	
Communicate Understanding	Respond to New Directions	
Asking Questions	Answer Questions	
Communicate with Others		
Telling About the Past*		

### 3. Draft Survey: Write questions we think will capture the concept.

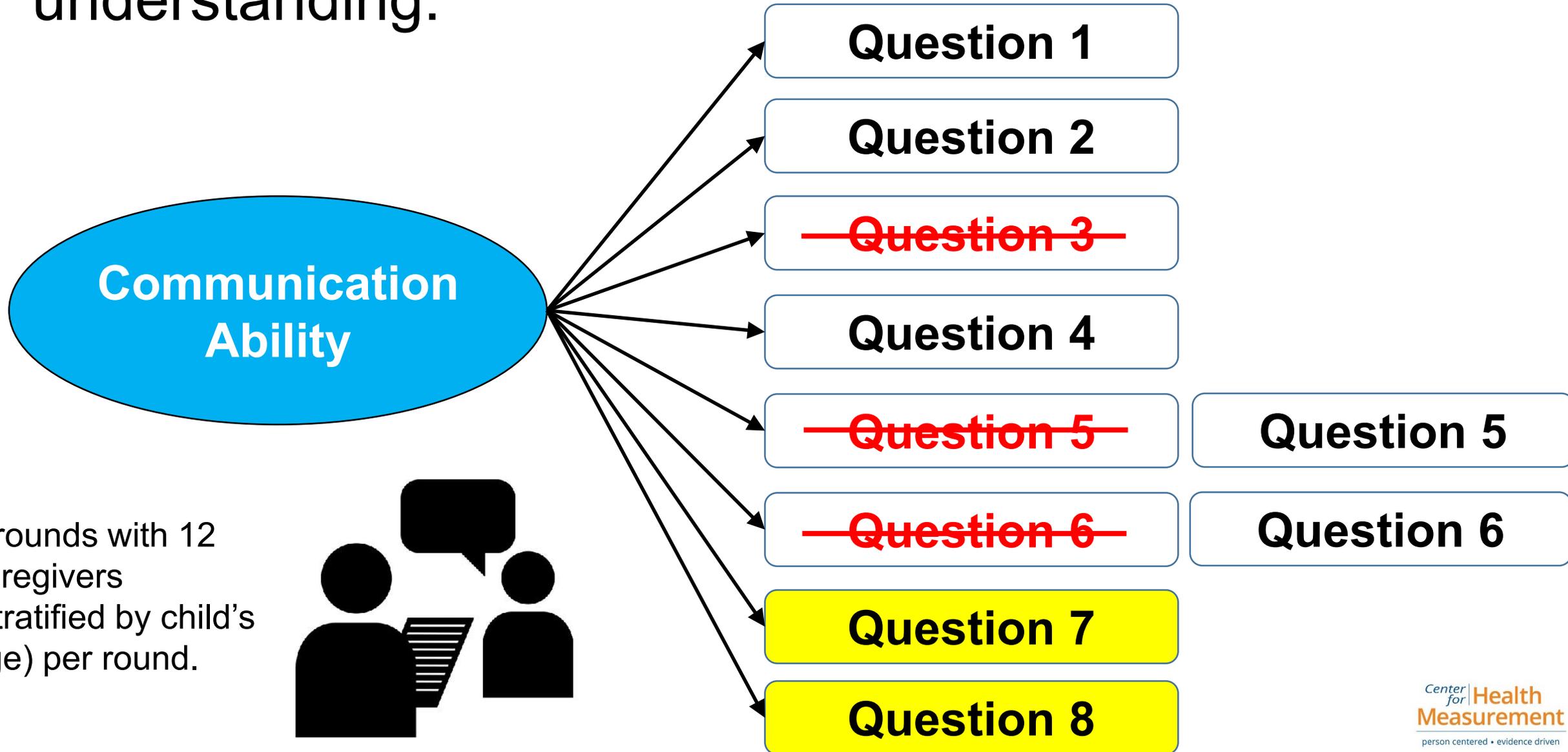


# Example of Draft ORCA Questions

2. Please tell us how your child seeks attention from you or someone else in their life.

		No	Yes, but not consistently	Yes, consistently
2a.	Does your child <u>cry or fuss</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2b.	Does your child <u>look at someone</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2c.	Does your child <u>grab, tap, or pull on someone</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2d.	Does your child <u>make a specific noise</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2e.	Does your child <u>use a specific gesture/sign</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2f.	Does your child <u>use a word or word approximation</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2g.	Does your child <u>use a device</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

# 4. Cognitive Testing: Evaluate questions for understanding.

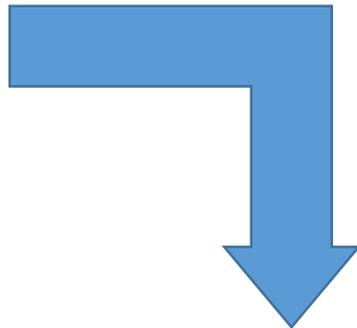


2 rounds with 12 caregivers (stratified by child's age) per round.



**2. Please tell us how your child seeks attention from you or someone else in their life.**

		No	Yes, but not consistently	Yes, consistently
2a.	Does your child <u>cry or fuss</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2b.	Does your child <u>look at someone</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2c.	Does your child <u>grab, tap, or pull on someone</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2d.	Does your child <u>make a specific noise</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2e.	Does your child <u>use a specific gesture/sign</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2f.	Does your child <u>use a word or word approximation</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2g.	Does your child <u>use a device</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

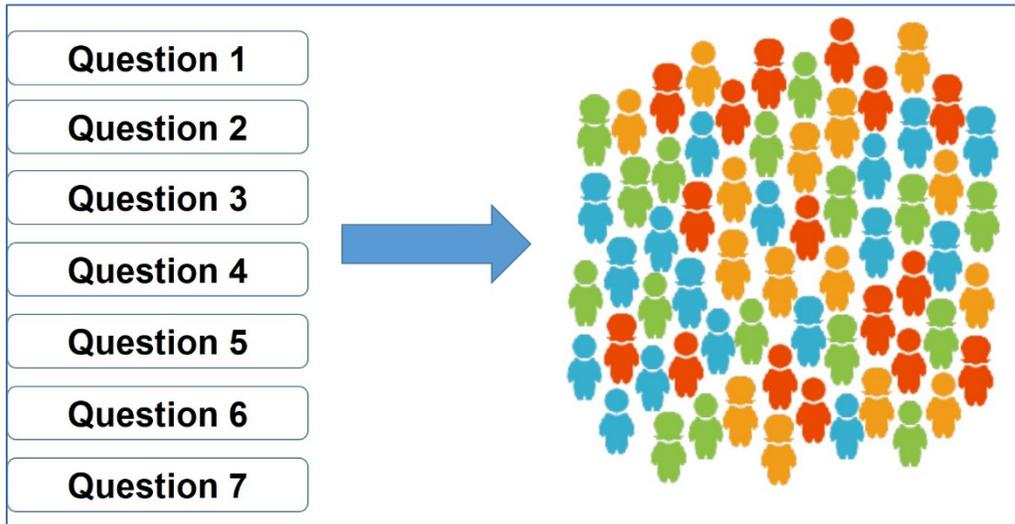


**3. Please tell us how your child sought attention from you or someone else.**

	<b>In the past 30 days,</b>	No or only once	Sometimes	Yes, almost all the time
3a.	Did your child <u>cry or fuss</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3b.	Did your child <u>look at someone</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3c.	Did your child <u>grab, tap, or pull on someone</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3d.	Did your child <u>make a specific sound</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3e.	Did your child <u>use a word/word approximation</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3f.	Did your child <u>use a specific symbol on a device</u> to seek attention?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>



## 6. Psychometric Testing: Evaluate reliability and validity of new measure in representative sample.



### Validity:

Measures what it's supposed to measure.

### Reliability:

Measures with little error.

# Psychometric Study Design

- Survey of  $\geq 250$  Parents of children with AS  $\geq 2$  years &  $\leq 40$  years
- Parents identified through FAST organization's Facebook page and other distribution lists.
- Test-Retest assessment only of ORCA measure 5 to 12 days later.

# Survey components

## 1. Demographic questions

- Parent and Child

## 2. Angelman syndrome and health

- Mutation type
- Seizures
- Sickness & hospitalizations

## 3. ORCA\*

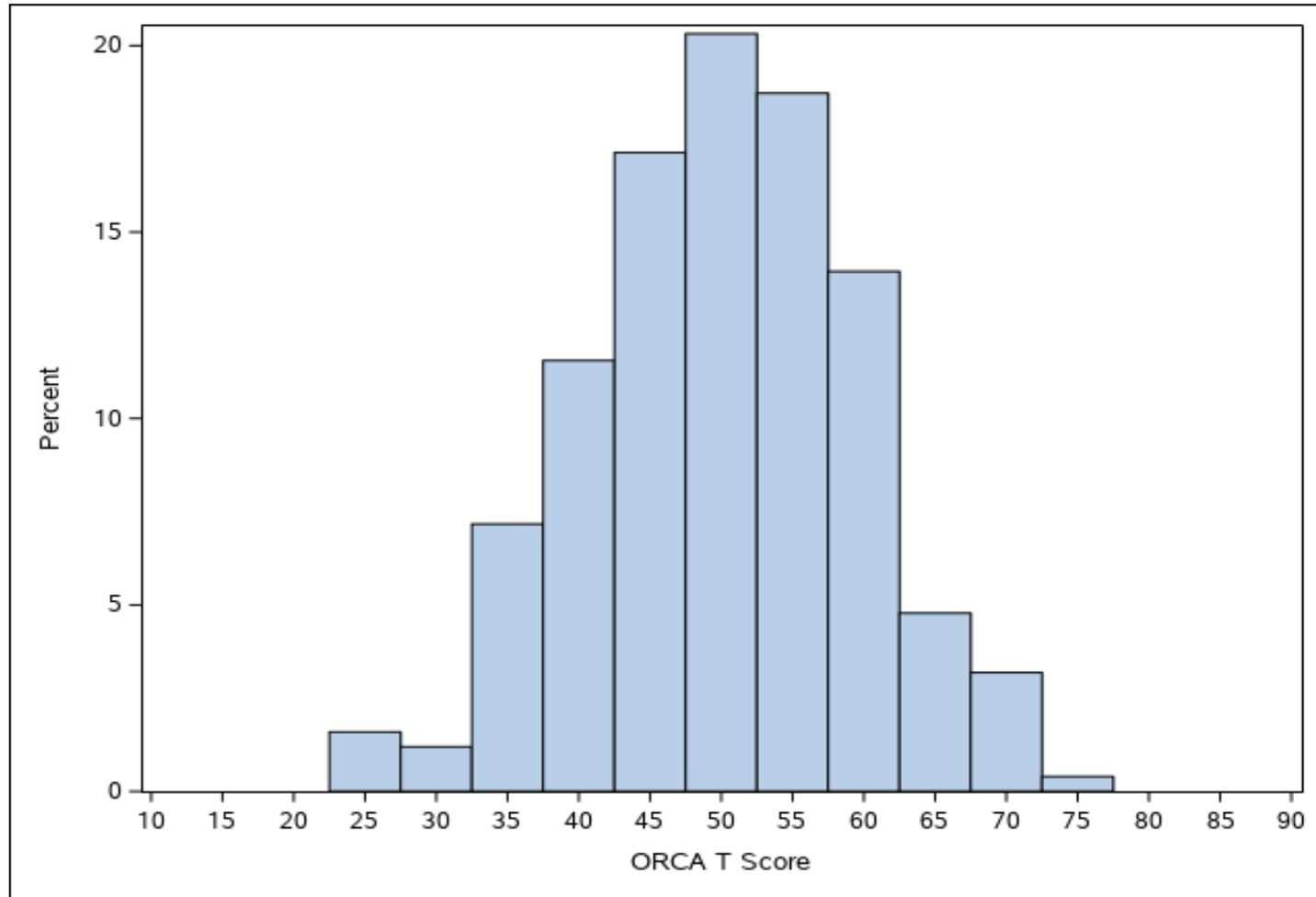
## 4. Communication and Symbolic Behavior Scale (CSBS)

## 5. PROMIS Proxy **Mobility** Short Form

Note one of the limitations of existing communication measures was:

- Many children with Angelman Syndrome are unable to be differentiated on the score metric (i.e., basement effect).

# Distribution of ORCA Scores (n = 295)

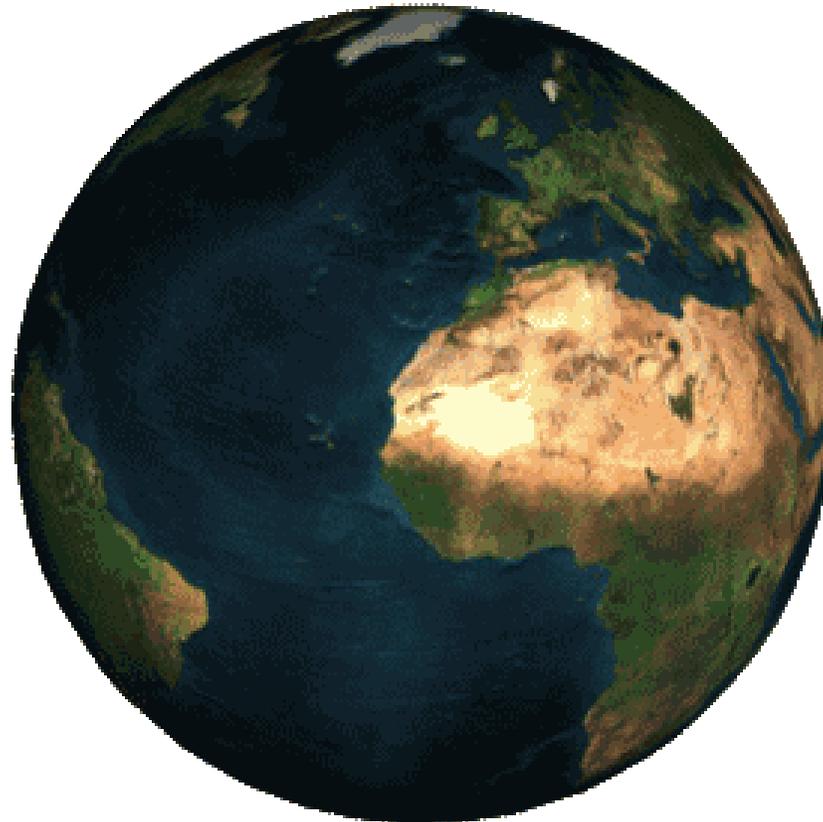


ORCA score range is 26.16 to 83.24

# Empirical Evidence for Validity & Reliability of ORCA measure

- Correlation with
  - Communication and Symbolic Behaviors Scale (CSBS):  $r = 0.82$
  - PROMIS Mobility:  $r = 0.53$
- Distinguished among
  - AS genotype
  - Seizures past year
- Reliability:
  - Internal consistency:  $\alpha = 0.89$
  - Test-retest: ICC = 0.90

7. Dissemination and Implementation: Distribute measure. Adapt questionnaire for other populations, cultures, and languages as needed. Build body of evidence of reliability and validity of measure.



# Activities to date

- FAST was able to include ORCA in GeneTx Biotherapeutics trial.
- Actively writing up our findings for peer-reviewed scientific journals
- Working with a translation company to translate ORCA into multiple languages
- Broad interest in the ORCA measure among rare neurodevelopmental disorders (NDDs):

	In AS	Other NDDs
Integrated or soon use ORCA	3	3
Extreme interest in using ORCA	3	16+

# Conclusions

- There is strong evidence for the validity and reliability in AS.
  - Continue to find opportunities through collaborations to collect more evidence.
- ORCA measure has important key features:
  - Assesses a broad range of communication concepts.
  - Allows multiple communication modalities.
  - Caregiver completes independently (15-20 minutes).
  - Designed for use in clinical trials.
- **Thank you, thank you, thank you to FAST and the AS community**

