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## Living with Fragile X Syndrome: Occupations as an Outcome Measure in a Clinical Trial

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## BACKGROUND

**Fragile X syndrome (FXS)** is the most common inherited cause of intellectual and developmental disability & single gene cause of autism spectrum disorder (National Fragile X Foundation, 2017)

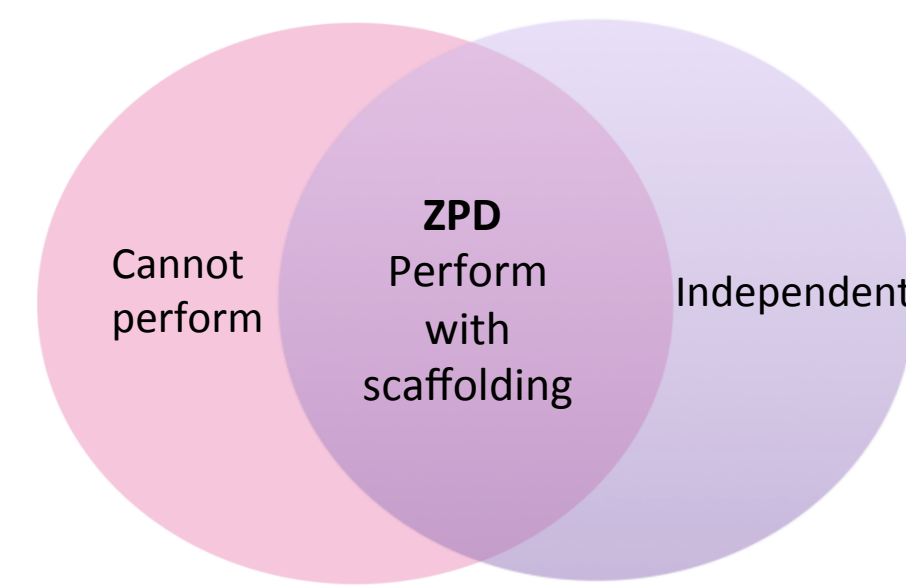
- Previous studies used quantitative methods (parent checklists and standardized assessments)
  - Outcome measures often do not reflect daily occupations and lack sensitivity for FXS (Berry-Kravis et al., 2012)

**Occupations** are activities that provide meaning and a sense of purpose in daily life

**Sociocultural theory** - Learning is not constrained by biology. Learning is social and cultural. **Occupations** happen in sociocultural contexts

### ➢ Zone of proximal development (ZPD)

= the optimal level for a child to explore and learn, with additional outside reinforcement & social support (Vygotsky, 1930)



## RESEARCH QUESTION

Can semi-structured parent interviews be used to determine difference in improved daily functioning between placebo and treatment groups when studying children with FXS during a clinical medication trial?

## METHODOLOGY

**Qualitative research - interview methods to provide voice and context of lived experiences of families & children with FXS**

**Participants: Families of children with full-mutation FXS, ages 2-6**

- Original study evaluated the effects of low-dose sertraline (Zoloft®) on language development, anxiety, and additional behavioral symptoms (Hess et al., 2016)

**Data: 25 audio interviews of FXS families** obtained from the U.C. Davis MIND Institute. Interviews were conducted at 6 months post testing.

- Interviews transcribed verbatim using Express Scribe software

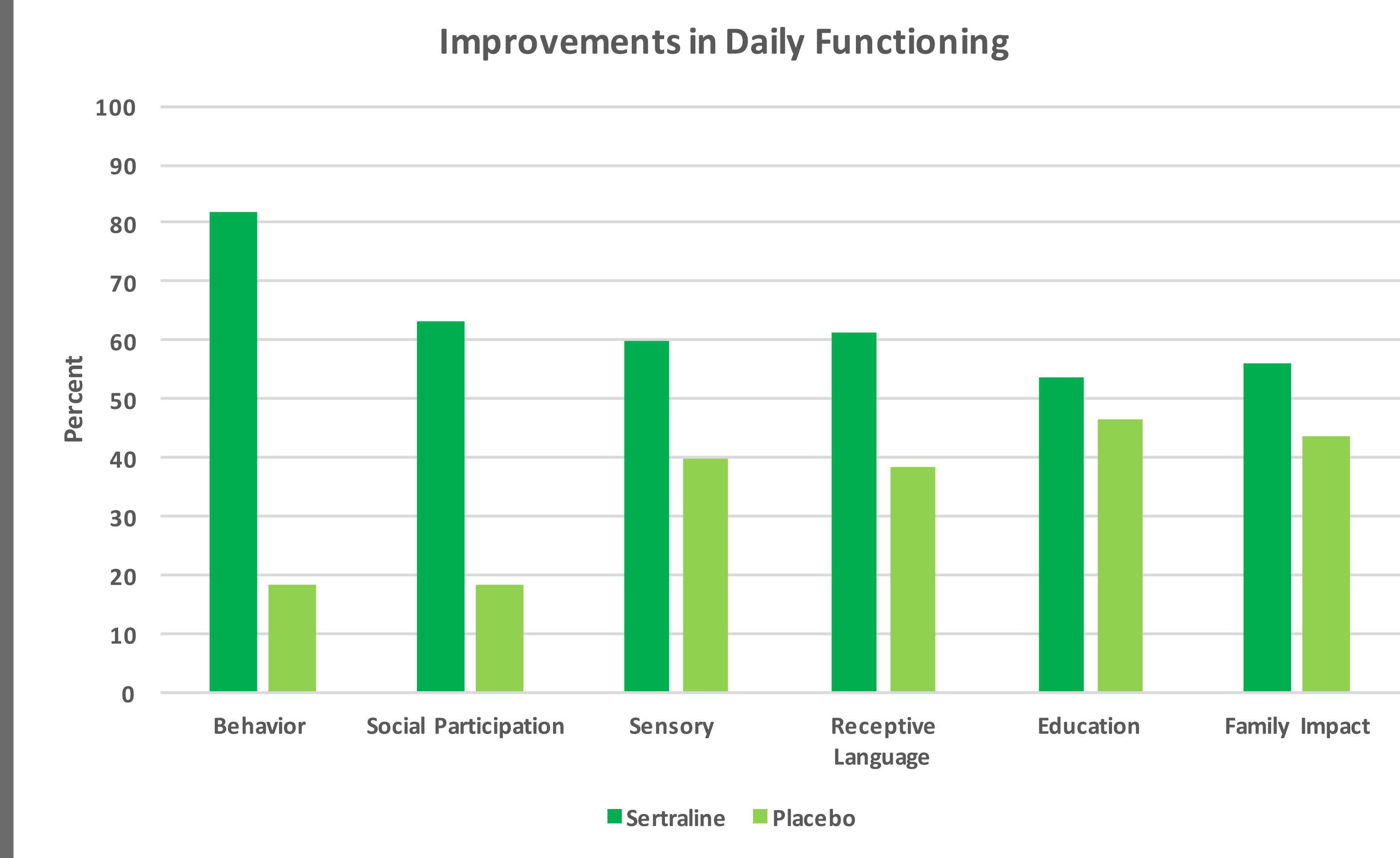
**Analysis:** Interviews coded (researchers blind to group assignment). Data analyzed using Constant Comparison Method with Dedoose software

- 3 interviews coded with 100% consensus by 4 researchers
- 17 interviews coded with 100% consensus by 2 researchers

- Categories & Codes (improved, not improved or no change)

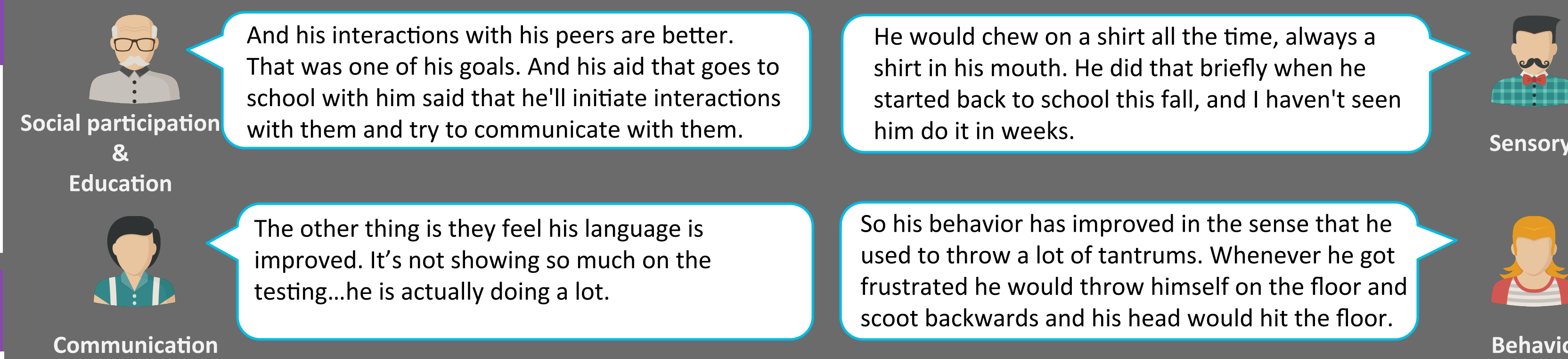
➢ *Behaviors, anxiety, sensory processing, communication, strategies, play, functional/ADLs, social participation*

## RESULTS



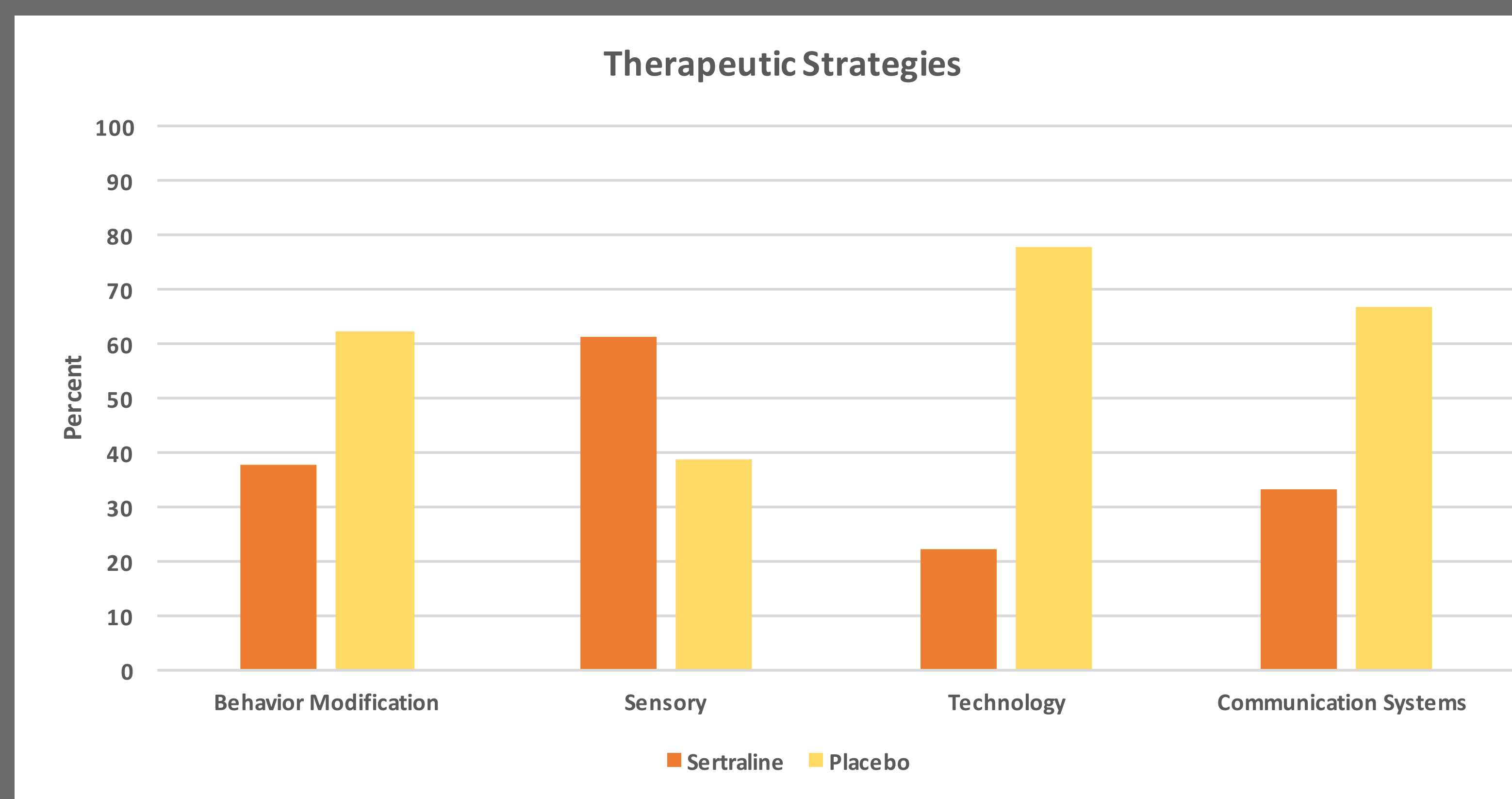
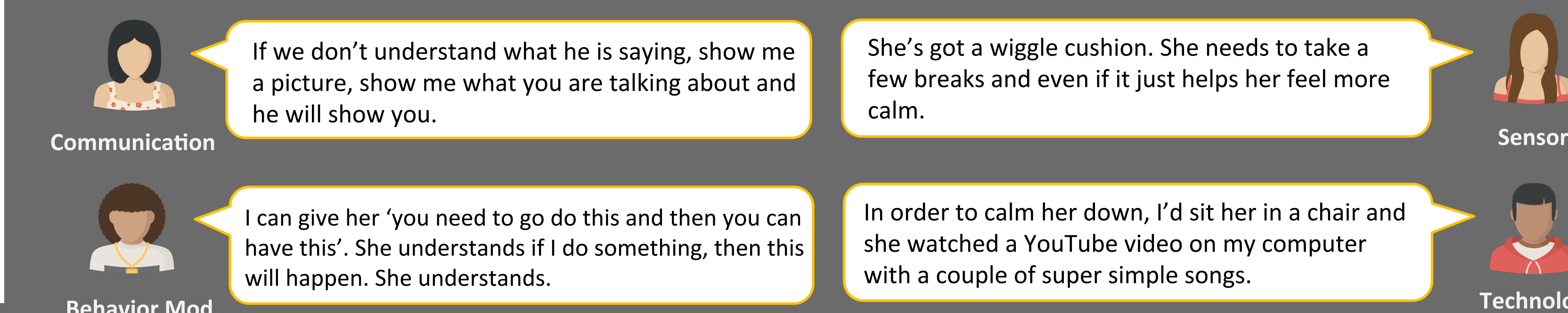
### Sample Interview Questions and Responses

- Have you noticed any changes related to anxiety, communication, behavior, sensory?
- How has this impacted you as a family?



A section titled 'Sample Interview Questions and Responses' featuring a researcher's questions and four participant responses. The researcher asks: 'Have you noticed any changes related to anxiety, communication, behavior, sensory?' and 'How has this impacted you as a family?'. The responses are:
 

- Social participation & Education:** 'And his interactions with his peers are better. That was one of his goals. And his aid that goes to school with him said that he'll initiate interactions with them and try to communicate with them.'
- Sensory:** 'He would chew on a shirt all the time, always a shirt in his mouth. He did that briefly when he started back to school this fall, and I haven't seen him do it in weeks.'
- Communication:** 'The other thing is they feel his language is improved. It's not showing so much on the testing...he is actually doing a lot.'
- Behavior:** 'So his behavior has improved in the sense that he used to throw a lot of tantrums. Whenever he got frustrated he would throw himself on the floor and scoot backwards and his head would hit the floor.'

A section titled 'Sample Interview Responses' featuring two participant responses. The first response is from a parent: 'If we don't understand what he is saying, show me a picture, show me what you are talking about and he will show you.' The second response is from a parent: 'She's got a wiggle cushion. She needs to take a few breaks and even if it just helps her feel more calm.'

Below these are two more responses:
 

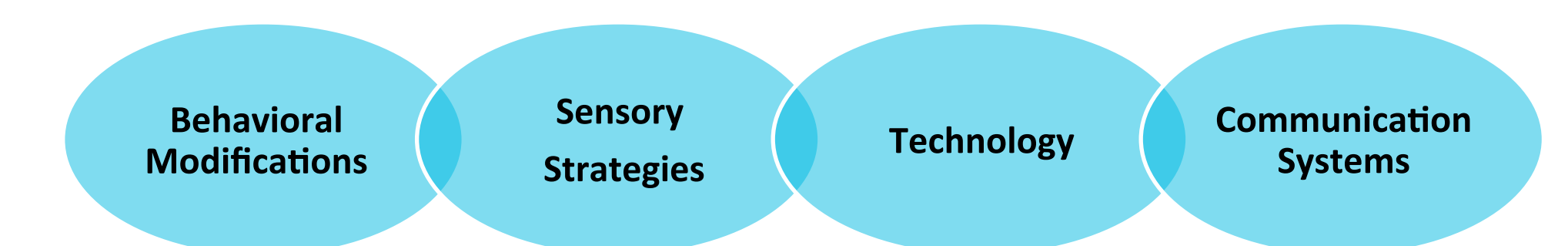
- Communication:** 'I can give her 'you need to go do this and then you can have this'. She understands if I do something, then this will happen. She understands.'
- Technology:** 'In order to calm her down, I'd sit her in a chair and she watched a YouTube video on my computer with a couple of super simple songs.'

## DISCUSSION & OT IMPLICATIONS

- **Children in the sertraline group showed greater improvements than the placebo group in 6 areas of daily functioning:**



- **Therapeutic strategies** were used by families in both groups, but **more prominently in the placebo group** (with the exception of sensory)



- **Analysis of semi-structured interviews revealed differences** between the placebo and sertraline groups in occupational performance outcomes
  - Interviews allowed for family voice, context and rich description of daily experiences of families with FXS.

- Outcome measures in clinical trials inclusive of **sociocultural and occupational factors** may gather a broader scope of daily life improvements vs. what can be measured on quantitative instruments (e.g. mostly performance skills) alone.

- **OTs have a role to play in clinical trials** → provide **holistic approach & occupation-based lens** to better **understand how interventions impact occupational engagement for a child with FXS**

### Future Directions:

- Investigate how various therapeutic strategies can help support children when taking medications
- **Incorporate interviews as outcome measures with families to add depth, context & additional sensitivity** for the FXS population

## ACKNOWLEDGEMENTS

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