Rethinking Clinical Trials in Neurodevelopmental Disabilities: Contributions from Language Assessment and Intervention

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In the past decade, dozens of medications targeting core and comorbid symptoms of fragile X syndrome, Down syndrome, autism spectrum disorder, and other neurodevelopmental disabilities have been developed and tested. Despite promising findings in preclinical studies involving multiple species, these medications have largely failed to show benefit in human studies. A number of reasons have been proposed for these “failures of translation.” In our research, we have focused on addressing two points of failure: (1) the use of psychometrically inadequate and poorly motivated outcome measures and (2) reliance on a clinical trials paradigm that is poorly suited to detecting changes in learning and behavior in individuals with neurodevelopmental disabilities. We have proposed that it would be useful to target language as an outcome measure in treatment studies because changes in language would be functional and meaningful for the individual. We have also proposed that medications are more likely to lead to detectable change, especially in short-term studies, if paired with behavioral or educational therapies that optimize the learning environment. In this presentation, I will present findings from two lines of research, one focused on evaluating the psychometric properties of outcome measures derived from expressive language sampling and the other focused on the development of telehealth-delivered, parent-implemented language interventions that can be useful complements to medication. The data to be presented are derived from studies of fragile X syndrome, Down syndrome, and autism spectrum disorder. This research has been supported by the National Institutes of Health through grants R01HD074346, R01HD024356, U54HD079125, P50HD103526, and U01NS096767, with additional funding provided by the Simons Foundation and the National Fragile X Foundation.