Adequately measuring treatment responses in children with Angelman syndrome requires outcome measures that are sensitive to developmental change and can be administered with continuity across a variety of ages and abilities. A number of passive experimental methods – such as eye movement analyses and psychophysiological methods – show promise in rare syndromes such as Angelman syndrome due to high resolution for detecting individual differences and minimal task demands on participants. However, many of these methods can only be administered in the laboratory or clinic, either due to technical limitations of commercially-available telehealth software or the practical challenges of collecting data without an examiner physically present. Many technical methods also require complex post-processing pipelines that are cumbersome for clinical research teams. Thus, these methods may be difficult to “scale up” for use in clinical trials for children with Angelman syndrome.

To address this challenge, our team has begun developing and optimizing a series of remotely administered assays for use in children with Angelman syndrome and other rare disorders. Here, we present preliminary data from 30 children with Angelman syndrome, 20 children with Down syndrome, and 20 low-risk community controls enrolled in two interrelated projects. First, we will present initial feasibility and implementation fidelity data from PANDABox (Parent-Administered NeuroDevelopmental Assessment), a novel parent-facilitated telehealth protocol for assessing early developmental skills and psychiatric risks in neurogenetic syndromes. Second, we will introduce a novel processing pipeline we have developed to extract acoustic and vocal maturity data from home-based audio recordings collected using the LENA device. Our preliminary findings suggest that with further refinement, these measures may provide naturalistic, low-cost, high-resolution options for characterizing developmental changes in young children with Angelman syndrome, including over the course of clinical trials.