Using wearable devices and ecological momentary assessment to define clinical severity in RTT

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Scientific Abstract:

Characterizing longitudinal clinical severity remains a challenge in RTT because clinician assessments are infrequent and existing assessment tools, while well-validated, remain subjective to a degree. Furthermore, symptom reporting can be inconsistent as patients cannot self-report and parent reporting of symptoms is subject to emotional and recall bias. Existing assessment strategies are thus problematic for reliably measuring developmental changes over time and response to interventions. Thus, it is essential to consider novel assessment strategies to overcome existing weaknesses. Recent advances in wearable technology permit real-time, objective measurements of physiological parameters commonly associated with disease severity in RTT including autonomic function and repetitive hand movements; and, additionally, the ubiquity of smartphones enables implementation of ecological momentary assessment (EMA) techniques to remotely survey parents within the child's home environment thus minimizing recall bias and maximizing ecological validity. We propose that data from EMA and wearables can initially be integrated with existing clinician ratings and parent reports from the natural history study to create a more granular picture of clinical trajectory, help identify clinically relevant outcome measures, and highlight discordance between assessment strategies. In this pilot study we will use the Empatica E4 wearable device to objectively measure autonomic function and hand movements for comparison with the existing standardized clinician ratings of disease severity and RTT- related behaviors; and conduct caregiver EMA surveys measuring seizure frequency, respiratory dysregulation, temperature dysregulation, hand movements, and mood for determination of: a) degree of concordance between EMA reports and prior parental reports of the same symptoms from the natural history study, and b) correlation of EMA data with parental reports of the same symptoms at the next natural history study visit. We will also examine the degree of concordance between EMA reports and objective measurements from the Empatica E4 device. We anticipate that the completion of these aims could have exceptionally high impact for RTT and related disorders in terms of establishing the most accurate method(s) of measuring symptoms and gauging clinical severity and change/progress. It is essential to determine what caregivers are best at reporting retrospectively and prospectively, and where the use of technology is essential for the management of RTT. The knowledge gained through this study would not only impact which

outcomes (and the data collection format) are utilized in future treatment trials and within natural history studies, but would also be crucial for the day-to-day clinical management of those with RTT and related disorders.