

**Production of a valid, useful tool for cross-sectional and longitudinal FXTAS studies: FXTAS Rating Scale**

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The goal of the project is to develop a reliable and valid measure of Fragile X Tremor and Ataxia Syndrome (FXTAS) symptoms. This scale, the FXTAS Rating Scale, will provide investigators and clinicians a common method to assess the severity of FXTAS and promote a shared research and treatment development. The need to have a reliable and valid measure of FXTAS severity is great. A reliable measure means that the scale will measure the level of impairment in a standard way across disease severity so that all investigators and clinicians will conduct the same examination. A valid measure is one that provides an accurate assessment of the signs and symptoms of FXTAS and shows sensitivity to different levels of impairment and to changes in disease status. The process of developing a reliable and valid scale are complex. First the items of the scale have to be developed in such a way that the scale captures all important aspects of the disease. We have already completed this step. Next, the scale must be administered to many patients with FXTAS, across the severity range, using many different raters. This allows us to see if the scale is administered in the same way by different raters and for different patients. The final step in developing a valid and reliable scale is to see if the scale assesses all important aspects of the disease, if it is sensitive to differences in severity, if it provides a stable measure of disease severity over the short term, and if it is responsive to changes in disease status. When the process is complete, we will have an instrument that can be utilized in standardized protocol design for collaborative studies by centers within and interacting with the FXS Clinic Consortium. This will allow researchers from multiple institutions to work together in FXTAS research and will allow meaningful comparisons of data produced from different regions of the world.