

Monitoring of Scale Administration and Videography Improves Quality in a Study of a Rare Genetic Neuromuscular Disease

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THE METHODOLOGICAL QUESTION BEING ADDRESSED:

Does Quality Monitoring of Scale Administration and Videography Help Improve Quality and Reduce Error Rate in a Study of a Rare Genetic Neuromuscular Disease?

INTRODUCTION

In clinical trials using centralized scoring of motor symptoms, optimal scale administration and high quality videography of subject assessments are essential to an accurate evaluation of subjects' initial status and progression. Recording subject assessments in movement disorder studies poses a challenge due to symptoms being rated based on observation of different parts of the subject's body in static or dynamic positions. High quality videography and adherence to scale administration guidelines are critical to capturing the frequency and severity of subtle motor signs, or small body parts, so that central raters and reviewers can accurately assess motor signs remotely.

METHODS

In this rare-disease, international, multi-center, randomized, double-blind, placebo-controlled clinical trial, selected site-based raters and videographers were certified based on their experience with the subject population. Raters (N=55) subsequently underwent a rigorous training program on scale administration and scoring. Videographers (N=62) were trained on the technical and clinical aspects of videography. Throughout the study, the sites provided video recordings of their scale administration for quality review. When the quality review was completed, a small group of external central raters scored the assessments. There were 175 participants at 22 sites in 12 countries enrolled in this clinical trial and they were evaluated in a total of 6 visits over the course of 12 months.

The quality of scale administration and videography was flagged for errors and feedback. Retraining was provided to scale administrators and videographers when it was found that the quality was suboptimal. In addition, there was a face-to-face retraining conducted with site-based raters to provide additional guidance on optimal scale administration and videography at around the midpoint of the study. The error rates over time and pre and post the face-to-face meeting were evaluated statistically to determine whether quality changed.

RESULTS

Error rate in scale administration and videography decreased from Screening at 39.18% to end of study at 20.56% ($p < 0.0001$, Fisher's exact test over categorical values). The error rate pre (57.49%) and post (14.01%) face-to-face training was also significant ($p < 0.0001$ Fisher's exact test over categorical values).

FIGURE 1. % ERROR RATE OVER TIME

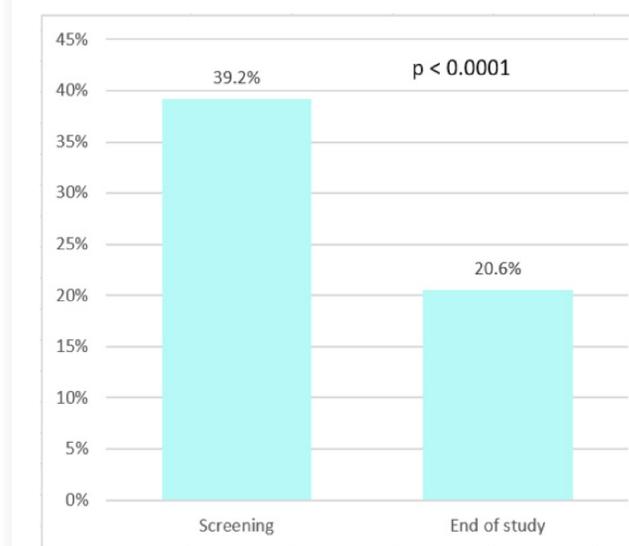
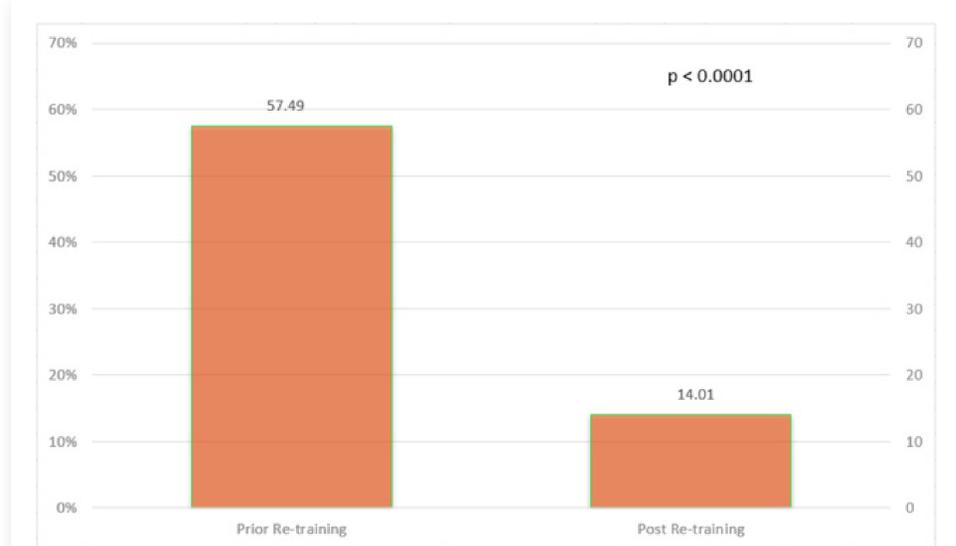


FIGURE 2. % ERROR RATE PRE AND POST-TRAINING



CONCLUSIONS

Centralized scoring and review of assessments in rare-disease movement disorder studies help to ensure accurate, consistent and high quality data. Many rare disease indications use novel instruments that are particularly suited for use by a small group of calibrated central scorers. It is important to develop and implement standardized, vigorous rater and videographer training and monitoring methodology to ensure that scale administration and video recordings are of optimal quality to allow accurate remote central scoring. Continued monitoring and re-training allow for a reduction in errors and improved quality of scale administration and videography.