



Background

- Fully remote or hybrid administration of clinical outcome measures in rare neurodevelopmental disorders trials is increasing due to the ongoing pandemic and recognition that remote assessments reduce burden on families.
- Many assessments in rare neurodevelopmental disorders trials are complex.
- Remote/hybrid trials readily allow for the use of centralized raters to administer/score scales.
- Use of centralized raters have many benefits, including reducing site burden, but specific impact on data quality have not yet been determined.

Objective

The current study has two aims:

- Evaluate differences in data quality between administration of a standardized clinical interview, the Vineland Adaptive Behavior Scales (Vineland-3), completed by centralized raters compared to those completed by site raters
- Evaluate improvement in accuracy of scoring a standardized developmental assessment, the Bayley Scales of Infant and Toddler Development (Bayley-3) when scored centrally compared to when scored by site raters.

Method

For aim 1, the Vineland-3, a widely used measure of adaptive functioning, was administered by site raters (n= 52) participating in one of four rare disease trials. The measure was also administered as part of two additional trials that utilized central raters (n=7).

- Each rater completed a comprehensive training program on the assessment.
- Following completion of the training, each rater completed a Vineland-3 with a mock caregiver. Administrations were recorded and reviewed by a neuropsychologist for administration and scoring accuracy.
- Raters were able to certify for the trials after demonstrating an accurate administration of the scale.
- For site raters, 25% of each rater's in-study administrations were reviewed by a neuropsychologist for accuracy of administration and scoring. For central raters, the first two administrations and every 10th administration were reviewed.

Method, Cont.

Aim 2 evaluated the added benefit of centralized scoring on accuracy of scoring of the Bayley-3, a comprehensive developmental assessment widely used in rare neurodevelopmental disorders trials.

- Bayley-3 administrations across four rare disease trials were centrally scored.
- For all administrations, the site rater who administered the Bayley-3 scored the scale.
- A centralized rater reviewed the video recordings of the administrations and also scored the scales to confirm accuracy.

Results

Aim 1

- 53 site raters were required for 138 assessments.
- Only 7 central raters were required for 150 assessments.



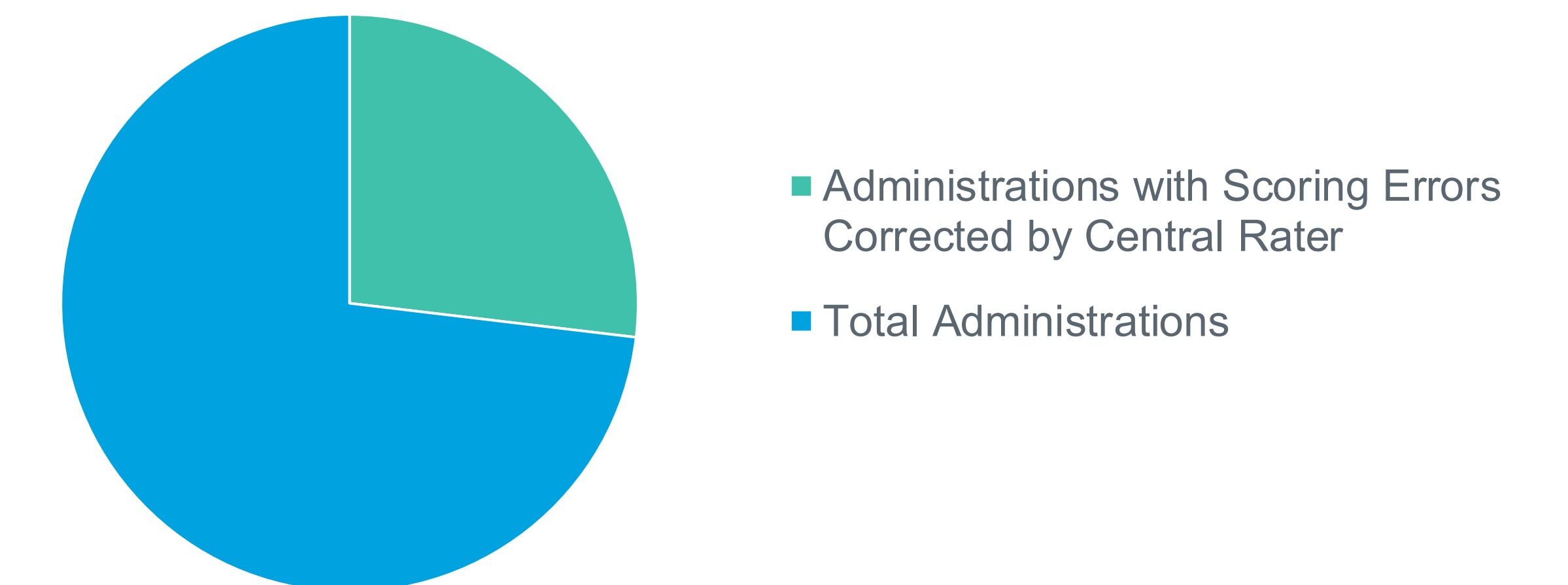
- Site raters completed 138 Vineland-3 administrations.
 - 53 administrations were reviewed by a neuropsychologist.
 - Four of the administrations had errors that compromised the validity of the assessment.
- Central raters completed 150 Vineland-3 administrations.
 - 38 administrations were reviewed.
 - None had significant errors.

Aim 2

- 68 administrations of the Bayley-3 were reviewed and scored by both a site rater and a centralized rater.
- Of these administrations, 25 administrations had scoring errors that were corrected by the central rater.

Results, Cont.

Administrations with Scoring Errors Corrected by Central Rater



Conclusions

In rare neurodevelopmental disorders trials, data quality is critical.

- The use of central raters for administration:
 - Decreases site burden
 - Decreases rater variance
 - Improves accuracy of administrations
- The use of central raters to score assessments dramatically improves accuracy of the data.

References

Bayley, N. (2005). Bayley Scales of Infant and Toddler Development, Third Edition. Pearson.
 Sparrow, S., Cicchetti, D., and Saulnier, S. (2016). Vineland Adaptive Behavior Scales, Third Edition. Pearson.

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