



Response Evaluation In Neurofibromatosis Schwannomatosis INTERNATIONAL COLLABORATION

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Decentralizing Neurocognitive Trials

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Pros of Decentralized Neurocognitive Research

- Increased geographic reach
- Ability to recruit more representative samples
- Less patient and family burden (travel, time, missing work/school)
- Possibly improve patient retention in research
- There is more information and ability to do some remote assessment since COVID-19
 - Payne et al. in children with NF1: WISC, WIAT, NEPSY (some tasks), CELF
 - Can we do a prospective study of virtual assessment to examine reliability?
 - PEARSON developed tools to support virtual assessment; NIH Toolbox
- Questionnaire-based data is very feasible (synchronous is still going to be important to consider to support response rate)

Remote Assessment: Pearson

- Guidance documents on best practices in virtual assessment (developed in response to COVID-19)

Example of some tools available for virtual assessment:

- Bayley-4
- VMI
- CELF
- CVLT
- D-KEFS
- EVT
- KTEA/WIAT/WRAT
- NEPSY
- PPVT
- WISC/WAIS/WASI
- WMS



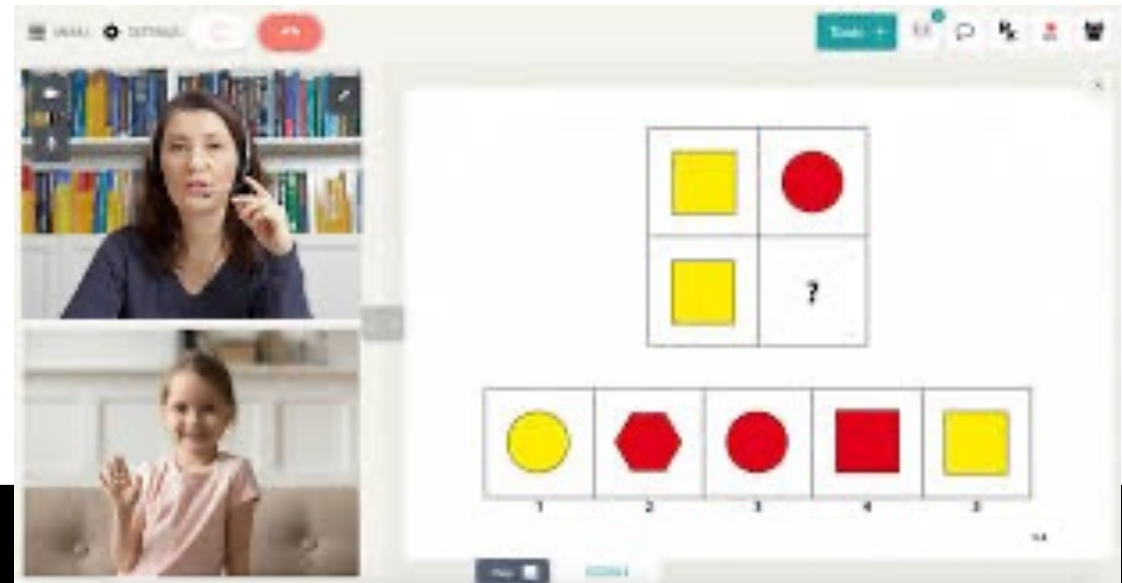
Remote Assessment: NIH Toolbox

“To assist in helping research studies and assessments continue, we provided guidance on which of the NIH Toolbox tests can be administered remotely via screen sharing. We have also revised two of our Cognition tests, Picture Vocabulary and Picture Sequence Memory, to make remote assessment easier.”



Virtual Neurocognitive Assessment Considerations

- Virtual assessment requires significant training and pre-assessment set up
 - Pre-assessment appointment(s) to discuss assessment, ensure environment is appropriate for assessment, engage parents to report testing behavioral observations
 - Assessment – managing the session



Concerns/Limitations

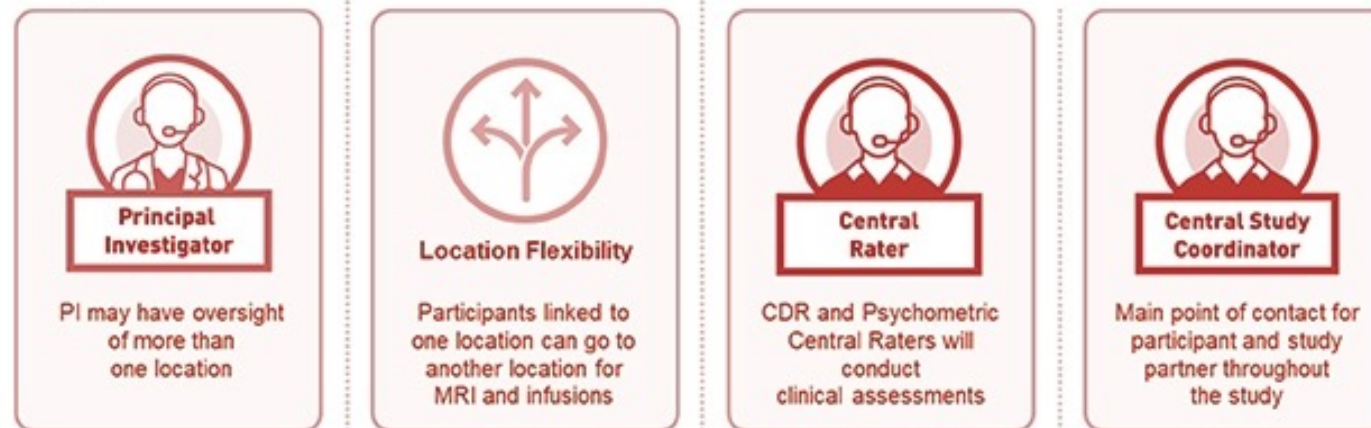
- In some regions there may not be neuropsychologists/psychologists
- Limits what tools can be administered/selected as outcome measures (performance based)
- Access to research-based instruments could be limited in the community
- Limitations of what can be evaluated virtually
- Potential difficulty achieving standardization study-wide (vital for cognitive assessment)
- Restricts age range of participants (not likely feasible in the pre-school cohort)
- For cognitive research/virtual assessment, this may require more work on the site/psychologist (training, pre-assessment session w/family) – this will require more funding
- Cost/time involved in training evaluators, coordination, admin
- GCP training is required – community sites are unlikely to have that – does that matter?

Possible ways forward

- Establish core experts for centralized virtual study evaluation
- Conduct prospective reliability study in pediatric NF1 (in person and virtual correlation) – start with REiNS approved measures?
- Hybrid trials using centralized and decentralized trials

Considerations for Neurocognitive Trials

- Centralized vs. decentralized assessment
 - Decentralized – local psychologists with assessment experience needed (in person or virtual)
 - “e-Centralized” – single group of evaluators, virtual (patients assessed in their home)



Conclusions

- It is important to consider novel ways of engaging patients in clinical trials
- There are steps that can be taken to do so with scientific rigor
 - Including determining the feasibility and validity of alternate approaches as well as setting up the home or other environment to support valid data

Questions/Discussion



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