

Validating Inherited Retinal Disease-Specific Patient-Reported Outcome Measures in Adolescent Patients

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Background

- Technical assessments of inherited retinal diseases (IRD) do not reflect the impact of the condition on patients' daily lives.
- Patient-reported outcome measures (PROMs) are questionnaires that can assess condition impact but should be disease-specific.
- There are no tailored PROMs for pediatric patients with IRDs.
- The Michigan Retinal Degeneration Questionnaire (MRDQ) and Michigan Vision-Related Questionnaire (MVAQ) are the only PROMs tailored to adults with IRDs.
- This study aims to validate the MRDQ and MVAQ in adolescents with IRDs.

Methods

- Inclusion criteria: adolescents (aged 13 to <18 years) with IRDs
- Exclusion criteria: inability to understand questions due to reasons other than vision
- Tools administered: MRDQ, MVAQ, and Patient Health Questionnaire-4 (PHQ-4)
- Data analysis: skewed distribution (*i.e.*, floor/ceiling effects), test-retest reliability, question and domain correlations to participant traits, and comparing to adult participant responses from the original validation studies

A. Pilot Test Questionnaires (n=15)

B. Wide Scale Testing of Questionnaires (n=76)

C. Psychometrically Analyze Questionnaires (n=91)

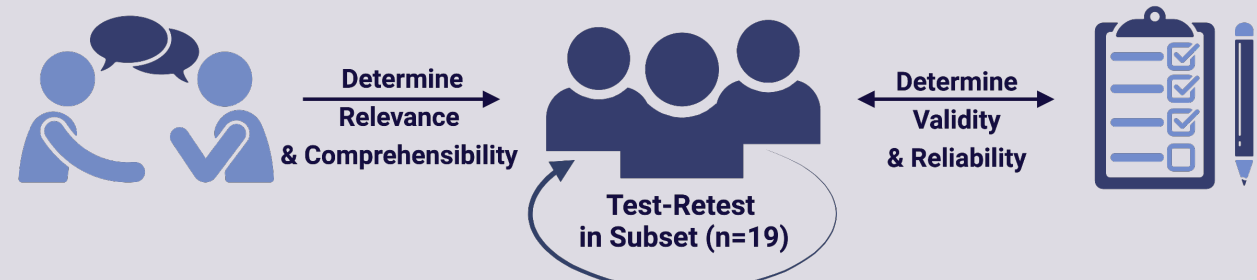


Figure 1. Patients recruited from SickKids Hospital (n=70) and Kellogg Eye Center (n=21) responded to and critiqued the tools to validate them in their population.

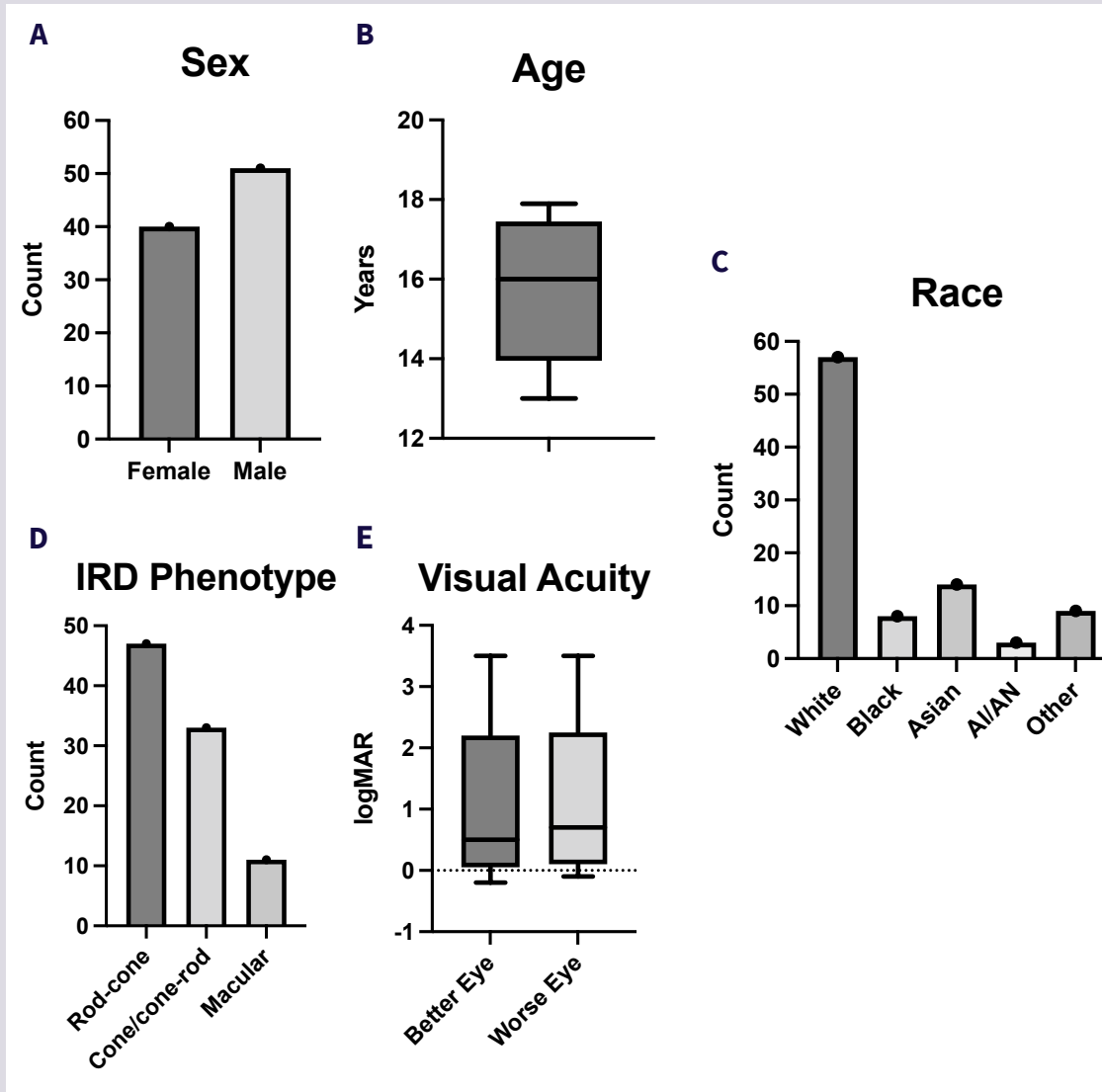


Figure 2. Participants' demographics and characteristics (n=91). A) Participant count by sex. B) Frequency of participants by age. C) Participant count by race (AI/AN = American Indian and Alaskan Native; Other = interracial and unknown). D) Participant count by IRD phenotype (IRD = inherited retinal disease). E) Frequency of participants by corrected visual acuity in better and worse eyes in logMAR.

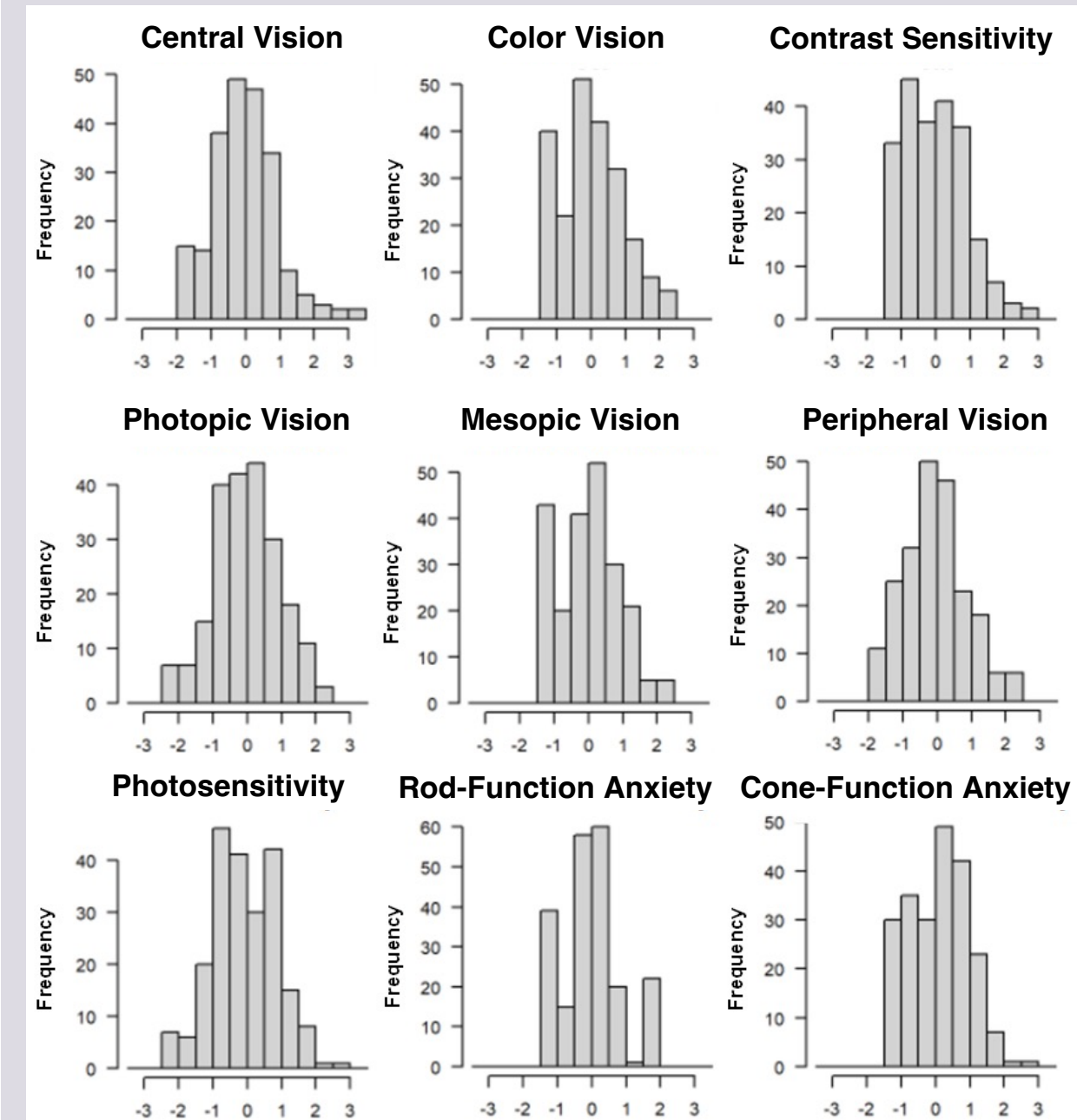


Figure 3. Domain (θ) scores in adolescents patients with inherited retinal diseases does not show a skewed distribution.

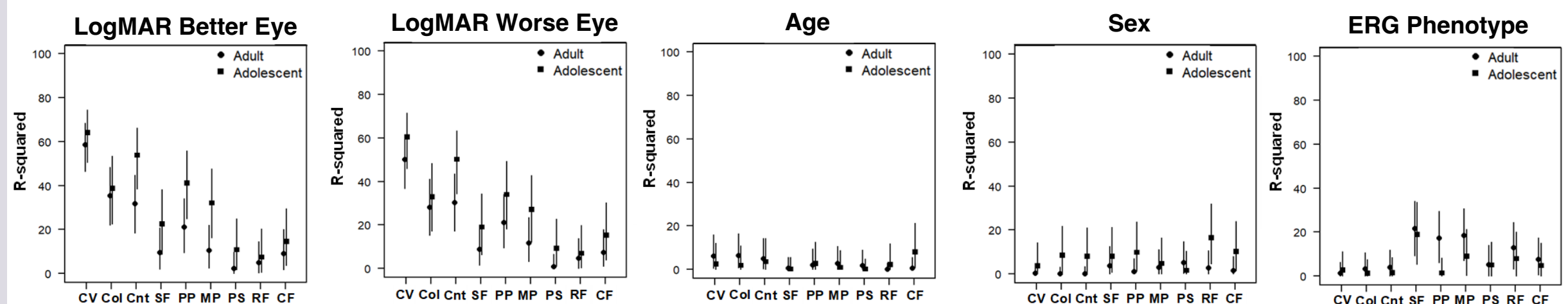


Figure 4. No significant differences were observed in associations of domain scores (θ) and participant traits between adolescents and adults*. *Responses from original validation studies. LogMAR=logarithm of the minimal angle of resolution; CV=Central Vision; Col=Color Vision; Cnt=Contrast Sensitivity; SF=Scotopic Function; PP=Photopic Peripheral vision; MP=Mesopic Peripheral Vision; PS=Photosensitivity; RF=Rod Function Anxiety; CF=Cone.

Summary

MRDQ and MVAQ are the first tailored PROMs suitable for adolescents with IRDs for routine assessment and clinical trials.

- No floor/ceiling effects observed (Fig. 3)
- Acceptable test-retest reliability ($r = 0.73-0.86$)
- No items excluded due to unexplained trait associations
- Scoring for adolescents was similar to that of adults (Fig. 4)

