Measuring Within-Child Change in Treatment Studies of Low-Functioning Children

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SUBMISSION DETAILS

What is the Methodological Question Being Addressed? Measuring outcomes in children with rare disease is methodologically challenging. How do researchers best measure within-child change over time? The rationale for these questions was formed from expert opinion that documenting within-child change over time requires better metrics than those in use now (age equivalent scores), and out of level testing often requires the use of multiple measures. The approach was a review of test capabilities shown through the standardization norms compared to a small sample of patients enrolled in a natural history study.

Introduction Out of level testing is complex, often requiring the use of multiple measures when patients “age-out” of younger batteries. Standard Scores (SS) are affected by population change and can be misleading when measuring change that is slower than typical. Age Equivalents (AE) are often used to track change in studies of neurodegenerative diseases of childhood, but not ideal metric because they lack SEMs to determine MCIDs and are inaccurate at the extremes. For long-term monitoring of change, scores that function across a wide range of developmental ages (DA) and chronological ages (CA) are desirable, to minimize the need to switch to harder or easier tests.

Methods The authors analyzed tests commonly used in clinical practice to measure cognition. Sources for analysis were the norms for Bayley 4 Cognitive subtest, and the DAS-II Picture Similarities and Matrices subtests. Performance of proposed metrics in the standardization samples were compared against natural history data from two large industry studies (Allievex: NCT02493998 and NCT03227042).

Results Normative scores are limited to the Chronological Age (CA) range of the norms, and to Developmental Age (DA) within about 2 SDs of average for the CA. Age Equivalents are limited to DAs within the range of CAs of the norms; there is no upper limit to the CA at which they can be used. Raw scores and GSVs have the widest range of DAs, from 2 SDs below the average of the youngest age of the norms, to 2 SDs above average of the highest age; like AEs, there is no upper limit to CA.

The two sets of charts illustrate this: first, the set of 3 charts comparing SS, AE, and RS/GSV; and second, the chart showing that Bayley Cognitive is accurate up to a DA of about 55 months (4:7), a year higher than the top of the age range of the norms. AE and SS are limited to this age range, but raw scores (RS) and GSVs measure accurately outside this age range. For example, the Bayley-4 Cognitive subtest was normed from birth to 42 months; therefore, the highest AE is 42 months. However, the subtest measures 42-month-olds who are up to 2.33 standard deviations above the mean. According to norms for similar scales (WPPSI-4 NVI, DAS-2 Nonverbal, KABC-II Nonverbal), this is an AE of about 54 months.

Conclusion As shown in this analysis, the Bayley-4 Cognitive RS and GSVs are useful for children with developmental ages up to about 54 months. This data shows the utility of using such metrics when studying children with low-occurring conditions which significantly affect cognitive development. This shift in assessment metric choice may improve methodological challenges experienced when out-of-level testing is necessary.

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Pearson develops and publishes assessments frequently used in clinical trials, including the Bayley-4, WPPSI-4, DAS-2 , and KABC-II, which are mentioned as examples in this poster.

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