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## FRI0571 MEASUREMENT PERFORMANCE OF REDUCED VERSIONS OF MUSCLE STRENGTH TOOLS IN JUVENILE DERMATOMYOSITIS

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**Background:** Assessment of muscle strength is a fundamental component of the clinical evaluation of children with juvenile dermatomyositis (JDM). Regular measurement of muscle strength in daily care requires the availability of simple and quick muscle assessment tools.
**Objectives:** To investigate whether reduced versions of the MMT8 and CMAS are equally reliable as the original tools.
**Methods:** The following 4 reduced instruments were devised: 1) MMT4 (score 0-40), including 4 items of MMT8 (neck flexors, deltoid middle, gluteus maximus, and gluteus medius); 2) MMT6 (score 0-60), composed of the same items of MMT4 plus biceps brachii and quadriceps; 3) head lift time of CMAS (0-120 seconds or 0-5 points); 4) sum of CMAS head lift time in points and the 6 sit-ups maneuvers of CMAS (score 0-11). Validation was conducted according to OMERACT filter on 213 patients followed in standard clinical care at 13 international pediatric rheumatology centers and evaluated at baseline and after a median of 5.9 months.
**Results:** All reduced instruments revealed strong correlations ($r > 0.7$) with muscle activity VAS and total DAS, moderate correlations ($r = 0.4$-$0.7$) with physician's global VAS, muscle DAS, skin activity VAS, pain VAS, parent's overall wellbeing VAS, and CK. Correlations with skin DAS and fatigue VAS were low ($r < 0.4$). Cronbach's alpha was excellent (0.92-0.95) for all reduced tools for which this property could be assessed. SRM was good-to-moderate (0.60-0.91) for all reduced instruments in patients judged as improved by the physician. All reduced tools discriminated strongly between patients classified in different disease activity states by the physician ($p < 0.0001$), and between patients whose parents were satisfied or not satisfied with their children's disease status ($p < 0.0001$). Overall, the metrologic performance of the reduced instruments was comparable to that of MMT8 and CMAS.
**Conclusion:** We found that reduced versions of the MMT8 and CMAS have good metrologic properties and perform similarly to the original tools in a population of patients followed in standard clinical care. Our results suggest that these simplified and shortened instruments could serve as surrogate for the complete measures in routine practice, particularly in a busy clinical setting.

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## FRI0572 DISABILITY AND HEALTH-RELATED QUALITY OF LIFE OUTCOMES IN PATIENTS WITH SYSTEMIC JUVENILE IDIOPATHIC ARTHRITIS TREATED WITH TOCILIZUMAB IN A PHASE 3 RANDOMIZED CONTROLLED TRIAL

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**Background:** Tocilizumab (TCZ) intravenous (IV) formulation was approved for the treatment of patients with systemic juvenile idiopathic arthritis (sJIA) based on the results of a large phase 3 clinical trial. Physical function, measured by the Childhood Health Assessment Questionnaire–Disability Index (CHAQ-DI), and health-related quality of life (HRQOL), measured by the Child Health Questionnaire (CHQ), were evaluated.
**Objectives:** To examine measures of disability and HRQOL in patients with sJIA treated with TCZ IV for up to 2 years in post hoc analysis of data from the phase 3 trial of TCZ.
**Methods:** Changes within 3 months of treatment initiation with TCZ (baseline) were compared between TCZ- and placebo (PBO)–treated patients using CHAQ-DI, pain global assessment (Pain-GA), physician global assessment, and patient global assessment (Pt-GA) using analysis of variance adjusted for treatment group. Changes in CHAQ-DI overall and domain scores and changes in CHQ domain and summary scores from baseline to 2 years were compared for patients treated with TCZ using the unpaired *t* test.
**Results:** Patients with sJIA experienced clinically relevant improvement in physical function (CHAQ-DI) and reduction of pain (Pain-GA). Mean (SD) CHAQ-DI scores for patients treated with PBO and TCZ were 1.7 (0.8) for both groups at baseline and 1.2 (1.0) and 0.9 (0.8), respectively, at week 12 (week 12 mean difference, –0.2; median [range] change from baseline to week 12 difference, –0.3 [–0.6 to 0.0]). These patients also had significantly improved CHQ socialization, behavior, mental health, and psychosocial summary scores after 3 months compared with those receiving PBO (Figure 1). Improvement in all CHAQ-DI domains over 2 years was observed with TCZ treatment (Figure 2); improvement rates in patient well-being (Pt-GA) were 87.7%. Similar improvements in physical function were observed in patients with polyarticular JIA in the phase 3 trial that led to approval of TCZ IV in these patients (not shown). There was also significant improvement ($p<0.05$) in most domains of HRQOL (CHQ domain scores) in patients with sJIA; of note, patients experienced marked improvement in mean scores from baseline to week 104 for pain/discomfort (31.7 to 75.3), self-esteem (61.0 to 76.0), mental health (62.1 to 76.8), and social limitation-emotional (52.6 to 86.2).
**Conclusion:** Two years of TCZ treatment resulted in statistically significant and clinically relevant improvements in function and HRQOL in patients with sJIA.

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