Longitudinal Assessment of Strength, Functional Capacity, Oropharyngeal Function, and Quality of Life in Oculopharyngeal Muscular Dystrophy

Rosemarie H.M.J.M. Kroon, MA, Johanna G. Kalf, PhD, Bert J.M. de Swart, PhD, et al.

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Study Question
Which clinical outcome measures capture disease progression in patients with oculopharyngeal muscular dystrophy (OPMD)?

What Is Known and What This Paper Adds
Patients with OPMD experience slow disease progression, and there is little available information concerning the natural history of OPMD. This investigation’s results show that several outcome measures can capture disease progression over 20 months and may serve as trial outcomes, with deltoid muscle strength as measured with fixed dynamometry exhibiting the greatest decline.

Methods
For this cohort study, the investigators recruited 43 patients with genetically confirmed OPMD (mean age 60.2 years; 49% women, mean age at onset 50.3 years). At baseline and around 20-month follow-up timepoints, a single investigator examined the patients with fixed dynamometry, Medical Research Council (MRC) grading, maximum bite force and isometric tongue strength assessments, the Motor Function Measure (MFM), the 10-step stair test, quality of life evaluations, and maximum swallowing, chewing, and speech tasks. The present study’s primary outcomes were from-baseline changes in these measures over 20 months.

Results and Study Limitations
Of the 18 distinct measures included in the assessments, 8 captured disease progression over 20 months. The greatest observed deterioration was that of dynamometry-measured strength of the right deltoid muscle, which exhibited a mean decline of 26.8% over 20 months (95% confidence interval [CI], 16.7%–36.9%). The mean decrease in dynamometry-measured left deltoid strength was 23.5% (95% CI, 16.8%–30.2%). The investigators also observed deterioration of left quadriceps strength (mean, 14.3%; 95% CI, 6.0%–22.6%), right quadriceps strength (mean, 12.7%; 95% CI, 3.7%–21.7%), right iliopsoas strength (mean, 12.2%; 95% CI, 5.5%–18.9%), maximum tongue strength (mean, 9.9%; 95% CI, 5.6%–14.9%), and MRC sum scores (mean, 2.5%; 95% CI, 0.9%–4.1%). They did not observe deterioration of left iliopsoas strength. There were also from-baseline increases in 10-step stair test times (mean, 12.5%; 95% CI, 5.4%–19.6%), from-baseline decreases in MFM part D1 scores (mean, 7.1%; 95% CI, 2.4%–11.7%), and from-baseline decreases in the maximum repetition rates for the /PA/sound (mean, 5.3%; 95% CI, 1.1%–8.8%). A limitation of the present study is that the investigators did not include some potentially relevant outcomes measures, for example, vital force capacity.

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This study was funded by AFM Télétion. The authors report no competing interests. Go to Neurology.org/N for full disclosures.

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