Mark Gorman, Mike Michaelis - *Inaugural Findings on OMSLife Registry*

Abstract:

Background: Most prior studies of opsoclonus-myoclonus-ataxia syndrome (OMAS) are limited in size due to its rarity. Patient-reported registries may overcome this challenge.

Methods: Retrospective analysis of data entered by parents of patients with OMAS into nine online surveys created by OMSLife Foundation and NORD assessing demographics, symptoms at onset, triggers, time of diagnosis, treatment, and additional therapies.

Results: 194 patients were enrolled. There was a female predominance (54%) and high rate of parental autoimmunity (31%). Age at onset peaked between 12 and 18 months overall. Initial misdiagnosis occurred in nearly 50% and tumor discovery was delayed in 18 patients, but overall median time to correct diagnosis was 25 days. Most patients (56%) received combination immunomodulatory therapies, and nearly all underwent supportive therapies.

Conclusions: Patient- and parent-powered research is feasible in OMAS and created the second largest published cohort of pediatric patients with OMAS. Results were similar to other large cohorts and also validated findings from prior case reports and smaller case series.