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Costs and health resource use in patients with X-linked myotubular myopathy: insights from US commercial claims

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Abstract

BACKGROUND: In X-linked myotubular myopathy (XLMTM), mutations in the *MTM1* gene result in absence or dysfunction of myotubularin, a protein required for normal development, maintenance, and function of skeletal muscle. Extreme muscle weakness results in severe respiratory failure that is fatal for approximately half of XLMTM-affected children by age 18 months. Most surviving patients require invasive mechanical ventilation, feeding tubes, and wheelchairs for mobility, due to profoundly impaired motor function. Little is known about the costs of care for this rare disease. Currently, there are no approved therapies for XLMTM. **OBJECTIVE:** To quantify the direct medical costs and health care resource utilization (HRU) incurred by XLMTM patients and paid by commercial insurers. **METHODS:** A retrospective, longitudinal study was conducted using the IQVIA PharMetrics Plus commercial database of adjudicated claims for more than 140 million individuals with commercial insurance coverage in the United States. An algorithm based on demographic information, diagnosis and procedure codes, and medications was used to identify XLMTM patients younger than aged 2 years during the study period from January 1, 2006, through September 30, 2018. All-cause direct medical costs and HRU during each month were calculated. Costs were grouped as inpatient hospital admissions (including the intensive care unit or neonatal intensive care unit [NICU]); emergency department visits; outpatient services (outpatient hospital visits, office visits, physician/provider office visits, ambulatory surgeries and procedures, laboratory tests, and imaging tests); and prescription medications. Monthly costs and HRU over time were stratified by age and use of mechanical ventilation. **RESULTS:** 49 patients met the study criteria. All had at least 1 inpatient hospital admission, and 36 (73%) had at least 1 NICU stay. All patients received ventilation at some time during the study period, including 40 (82%) treated with invasive ventilation. Mean monthly per patient direct medical costs were highest in the first year of life ($74,831), including costs for inpatient admissions ($69,025), outpatient services ($5,266), and prescription medication ($540). Mean monthly costs were lower in the second, third, and fourth

years of life ($23,207, $13,044, and $9,440, respectively). When annualized, these all-cause monthly medical costs totaled $897,978 per patient in the first year of life and nearly $1.5 million total for patients who survived the first 4 years of life. Costs were consistently highest when patients were receiving invasive ventilation and lowest when they were not receiving ventilation (i.e., before they started on ventilator support). **CONCLUSIONS:** This direct health care cost and HRU analysis demonstrates the substantial economic burden associated with XLMTM. Costs are highest in the first year of life and are particularly significant for patients receiving invasive ventilation. **DISCLOSURES:** This study was funded by Audentes Therapeutics, an Astellas Company, and was conducted by PRECISIONheor with funding from Audentes Therapeutics, an Astellas Company. Slocomb is an employee of Audentes Therapeutics, an Astellas Company; James was an employee at the time of the study. Sacks, Healey, and Cyr are employees of PRECISIONheor. Graham participated in the medical/scientific advisory board for Audentes as part of a clinical trial design for XLMTM but declares no vested interest or holdings that would represent a conflict of interest. Beggs received consulting fees from Audentes Therapeutics, for work on this study, and has received grants from Alexion Pharmaceuticals, Audentes Therapeutics, Dynacure SAS, Pfizer Pharmaceuticals, along with personal fees from Asklepios Biopharmaceutical, Inc., Ballard Biologics, Biogen, F. Hoffmann-La Roche AG, GLG, Guidepoint Global, and Kate Therapeutics, unrelated to this study. In addition, Beggs has a patent (Patent number: 10736945) for systemic gene replacement therapy for treatment of X-linked myotubular myopathy (XLMTM) licensed to Audentes Therapeutics.

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