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Medicaid Cost Analysis in Patients with X-Linked Myotubular Myopathy: A Retrospective Longitudinal Study

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Introduction

- X-linked myotubular myopathy (XLMTM) is a rare, life-threatening congenital myopathy characterized by profound muscle weakness leading to impairment of respiratory and neuromuscular function[1,2]
- XLMTM is associated with high rates of healthcare utilization, hospitalization, and surgical intervention[3–5]
- Most children with XLMTM have significant dependence on invasive mechanical ventilation (>16 hours/day)[4], feeding tubes, wheelchairs, and home health services[6]
- In the US, Medicaid provides coverage for XLMTM; however, the costs paid by Medicaid for XLMTM patients have not been estimated

Objective

- This study aimed to quantify the economic impact of patients with XLMTM paid by Medicaid by estimating costs and healthcare and resource utilization (HRU) per patient per month (PPPM), by age

Methods

Data Source

- IBM MarketScan multi-state Medicaid claims database from January 1, 2009 to December 31, 2018

Selection Criteria

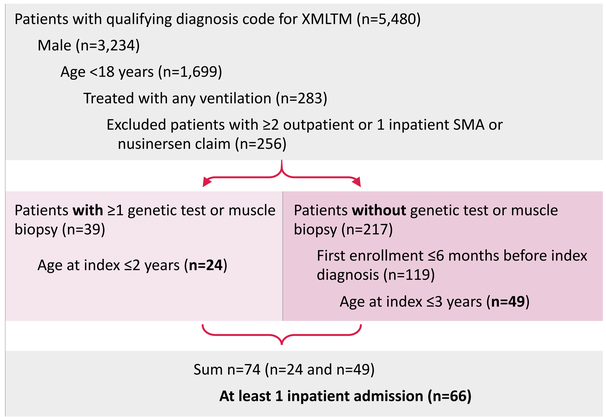
An algorithm based on demographic information, diagnosis and procedure codes, and medications was used to identify probable XLMTM patients (**Figure 1**). This included patients who had at least one claim with a qualifying diagnosis code for XLMTM:

- ICD-9-CM: 359.0; congenital hereditary muscular dystrophy
- ICD-10-CM: G71.2; congenital myopathies

Endpoints

- HRU and all-cause direct medical costs paid by Medicaid PPPM stratified by patient age

Figure 1. XLMTM Medicaid Patient Counts Following Inclusion/Exclusion Criteria



Results

Summary Statistics

- Among 66 patients meeting study criteria, mean age (± SD) at first observable diagnosis was 0.7 ± 0.9 years (**Table 1**)
- Following diagnosis, patients were observable for an average (± SD) of 35.7 ± 27.1 months
- Hospitalizations were frequent (4.3 ± 3.1 over all observable months) and lengthy (mean length of stay [LOS]: 88.6 ± 83.7 days)
- Nearly all (92%) had at least 1 intensive care unit stay

Table 1. Baseline Characteristics

Characteristic	Mean or %	SD
Index age (years)	0.7	0.9
Months enrolled pre index	2.6	3.6
Months enrolled post index	35.7	27.1
Patients with at least 1 inpatient visit	100%	
Inpatient admissions per patient	4.3	3.1
Inpatient admission LOS (days)	112	101.2
Patients with at least 1 NICU or ICU admission	92%	
NICU or ICU LOS (days)	88.6	83.7

Costs and Healthcare and Resource Utilization (HRU)

- Costs were highest in the first year of life, averaging $37,250 PPPM (**Table 2**), largely reflecting inpatient admissions ($31,538 ± 58,331 PPPM), which were most frequent in the first year (**Table 3**)
- After the first year, inpatient admissions decreased while home health service visits increased and remained at a high frequency throughout the observation period (**Table 3**)
- Costs paid by Medicaid averaged $1.156 million per patient over the first 4 years of life, with annualized inpatient costs decreasing and in-home care increasing proportionally (**Figure 2**)
  - Age 1–2 years: total costs $23,074 PPPM, including inpatient admissions $11,034 ± 9,041 and in-home care $8,980 ± 2,383
  - Age 2–3 years: total costs $17,588 PPPM, including inpatient admissions $4,126 ± 2,786 and in-home care $9,919 ± 2,123
  - Age 3–4 years: total costs $18,459 PPPM, including inpatient admissions $7,102 ± 3,782 and in-home care $8,286 ± 1,825

Table 3. HRU: Average Visits/Encounters/Prescriptions PPPM by Age and Setting, mean ± SD

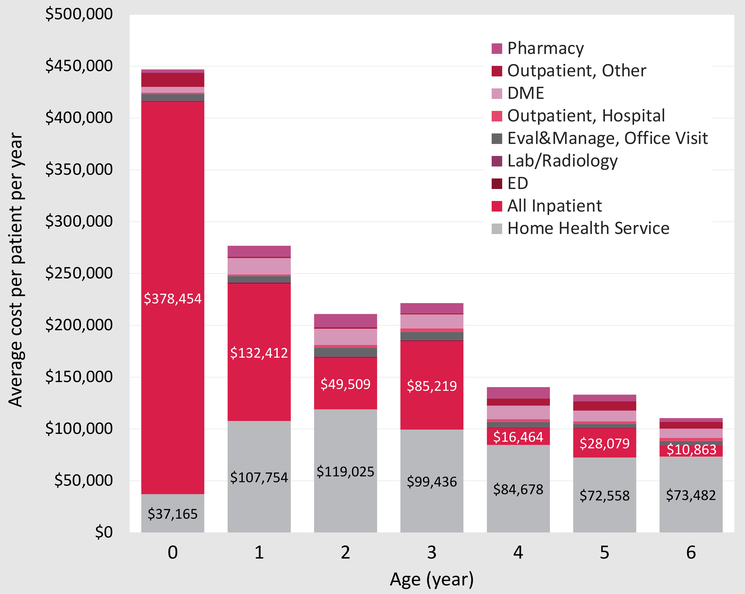
Age	All Inpatient	ED	Lab/Radiology	Eval & Manage, Office visit	Outpatient, Hospital	DME	Home Health Service	Outpatient, Other	Pharmacy
0	0.43 ± 0.27	0.17 ± 0.09	0.74 ± 0.62	2.87 ± 1.36	0.33 ± 0.14	0.64 ± 0.19	4.73 ± 1.88	0.33 ± 0.16	1.58 ± 0.41
1	0.17 ± 0.06	0.15 ± 0.06	0.62 ± 0.24	3.93 ± 1.01	0.31 ± 0.08	1.14 ± 0.25	16.03 ± 3.16	0.47 ± 0.22	3.06 ± 0.74
2	0.07 ± 0.03	0.11 ± 0.03	0.31 ± 0.08	5.01 ± 1.31	0.30 ± 0.08	1.29 ± 0.29	19.08 ± 3.06	0.58 ± 0.31	2.89 ± 0.69
3	0.06 ± 0.03	0.11 ± 0.04	0.34 ± 0.09	4.83 ± 1.27	0.36 ± 0.11	1.21 ± 0.26	17.37 ± 3.27	0.82 ± 0.38	3.71 ± 0.93
4	0.03 ± 0.01	0.06 ± 0.02	0.44 ± 0.18	3.17 ± 0.88	0.28 ± 0.08	1.10 ± 0.27	16.39 ± 3.21	2.81 ± 1.68	4.61 ± 1.41
5	0.05 ± 0.03	0.06 ± 0.04	0.23 ± 0.06	3.06 ± 0.72	0.26 ± 0.08	1.04 ± 0.25	13.80 ± 3.17	3.01 ± 1.62	3.94 ± 1.20
6	0.04 ± 0.01	0.07 ± 0.03	0.20 ± 0.04	3.30 ± 0.76	0.73 ± 0.64	1.19 ± 0.24	14.48 ± 3.17	2.56 ± 1.21	3.31 ± 1.32

Table 2. All-cause Healthcare Costs Per Patient Per month (PPPM) by Age

Age Year	Mean all-cause healthcare cost by age PPPM
0	$37,250
1	$23,074
2	$17,588
3	$18,459
4	$11,700
5	$11,099
6	$9,209

- It is common for XLMTM patients to have dual commercial and Medicaid insurance coverage
  - Therefore, it is likely that these costs are under-estimates of the total amounts paid to providers
  - The dual coverage is a likely explanation for the decrease in average cost per patient per year
- Most inpatient admission costs will be covered first by commercial insurance
  - Therefore, if patients have both Medicaid and commercial coverage, the Medicaid claims will not capture the total costs when those patients require hospitalization

Figure 2. Annualized Healthcare Encounter Cost Per Patient Per Year by Place of Service



Conclusions

- There is substantial economic burden to Medicaid imposed by XLMTM, with considerable inpatient admissions and home health service costs
- Patients diagnosed and stabilized early in life may require intensive, long-term home health care

ABBREVIATIONS DME, durable medical equipment; ED, emergency department; Eval & Manage, evaluation and management; HRU, healthcare and resource utilization; ICD-9-CM, International Classification of Disease Ninth Revision, Clinical Modification; ICD-10-CM, International Classification of Disease Tenth Revision, Clinical Modification; ICU, intensive care unit; LOS, length of stay; NICU, neonatal intensive care unit; PPPM, per patient per month; SD, standard deviation; SMA, spinal muscular atrophy; XLMTM, X-linked myotubular myopathy.

REFERENCES **1.** Beggs AH, et al. *Muscle Nerve* 2018;57:550–60; **2.** Jungbluth H, et al. *Orphanet J Rare Dis* 2008;3:26; **3.** Annoussamy M, et al. *Neurology* 2019;92: e1852–67; **4.** Graham RJ, et al. *Arch Dis Child* 2020;105:332–38; **5.** Amburgey K, et al. *Neurology* 2017;89:1355–64; **6.** Sacks NC, et al. *J Manag Care Spec Pharm* 2021:1–8.

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