The cost of care among infantile and child spinal muscular atrophy patients: RWE from a large claims database

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CHRONOS

Background: Spinal muscular atrophy (SMA) is an autosomal recessive genetic disorder that affects the spinal cord and nervous system causing severe muscle wasting and weakness. Patients with SMA Type 1 and Type 2 are diagnosed between the ages of 0-12 months and 12-36 months, respectively. Available treatments include *nusinersen* (approved in December 2016 in the US) and *onasemnogene abeparvovec-xioi* (approved in May 2019 in the US).

Objectives

- Describe the cost of care for infantile and child SMA patients in the US by setting of care
- Understand healthcare coverage by payer channel in the US

Methods: This was a retrospective descriptive analysis conducted using CHRONOS, a hybrid data ecosystem of linked closed and open claims data. Patients newly diagnosed with SMA (ICD-10-CM: G12.0, G12.1, G12.8, G12.9) were identified in the closed claims data between January 1, 2017, and December 31, 2021. Patients were followed from the date of the first claim for SMA (index) until the end of enrollment or 365 days after index, whichever occurred first. Patients diagnosed with SMA between 0-12 months and between 13-36 months of age were defined as infantile SMA (iSMA) and child SMA (cSMA), respectively. Patients with the first claim for an infantile SMA diagnosis (ICD-10-CM code G12.0) after 12 months of age and juvenile SMA were excluded **(Figure 1).**

Calculation of the cost of care included all allowed costs on professional, institutional, and pharmacy claims in the closed claims data. The cost per patient per month (PPPM) was calculated from the index date through the end of follow-up and adjusted to reflect 2021 USD. In addition, because gene therapy became available partway through the study period, costs were calculated with and without gene therapy. For patients with a link to the open claims data in CHRONOS, the distribution of payer for all claims submitted over follow-up was examined.

At the time of the analysis, Forian's CHRONOS data ecosystem included healthcare claims from over 300 million deidentified patients receiving care in the United States between 2017 and 2022. This study utilizes only deidentified patient-level data and was therefore exempt from Institutional Review Board approval.

Results: Patients with iSMA(n= 119) and cSMA(n=63) were 7- and 25-months old at index and 53% and 59% male, respectively. *Onasemnogene abeparvovec-xioi,* a gene therapy, was received by 35% and 24% of iSMA and cSMA patients.

The cost PPPM in the closed claims data was $139,115 (Median: $98,008; IQR: $2,193 - $187,340) among iSMA patients and $51,073 (Median: $4,033; IQR: $1,553 - $52,483) among cSMA patients. After excluding costs related to gene therapy, the cost PPPM was $41,892 (Median: $12,289; IQR: $2,302 - $34,193) among iSMA patients and $25,886 (Median: $4,033; IQR: $1,675 - $30,456) among cSMA patients.

The cost PPPM was highest in intermediate care settings. Excluding costs related to gene therapy substantially decreased costs in these settings as 94% of all gene therapy was administered in skilled nursing facilities and outpatient hospitals **(Figure 2).**

Of the patients defined in the closed claims, 87% and 94% of iSMA and cSMA have a link to open claims data in CHRONOS. All care recorded in the closed claims data reflects coverage by a commercial payer, but at least 13% of iSMA and 9% of cSMA related care was also covered by Medicaid **(Figure 3).** All gene therapy was covered by a commercial payer.

Figure 2. Cost PPPM by setting of care

Abbreviations: IM, intermediate care; ER, emergency room; PPPM, per patient per month

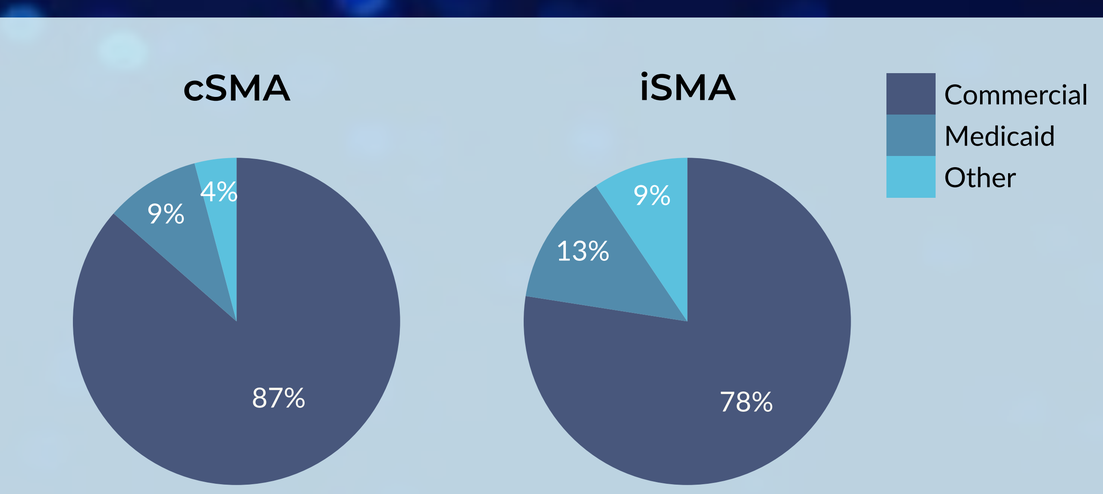

Figure 3. Payer channel for submitted claims by SMA Type

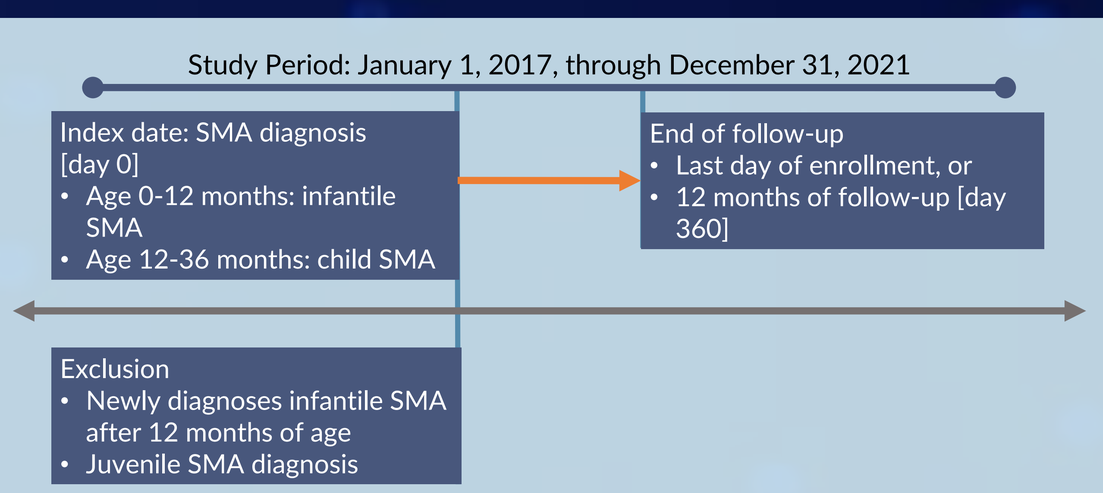

Figure 1. Study diagram

Conclusions: Gene therapy contributes substantially to the cost of care for iSMA and cSMA patients and is primarily administered in skilled nursing facilities and outpatient hospital settings. The link between closed and open claims data in CHRONOS provides insight into the proportion of care covered by Medicaid. Further research will examine the effectiveness of novel therapies and the differences between SMA patients covered by commercial and public payers.

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