

Evaluation of Inpatient Healthcare Resource Utilization and Costs Pre- and Post-Nusinersen for the Treatment of Spinal Muscular Atrophy (SMA) Using US Claims



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Objectives

- To evaluate real-world HRU and cost patterns associated with nusinersen use through US claims databases.

Conclusions

- This study demonstrates that nusinersen treatment was associated with reductions in number and costs of inpatient admissions across pediatric and adult patients over a period of 12 months post-treatment initiation compared with the pre-treatment period using US claims databases.
- This study also suggests that nusinersen was associated with an additional benefit of reduced time in the hospital, which is relevant to patients and their families, providers, and payers.

Introduction

- Nusinersen is indicated for the treatment of 5q SMA across all ages, administered intrathecally at a dose of 12 mg.
- The clinical efficacy and effectiveness of nusinersen has been demonstrated in a number of clinical trials and real-world studies.¹⁻⁶
- A few studies to date have demonstrated that nusinersen is associated with reductions in economic outcomes, such as hospital admissions between the first and second year of treatment.^{7,8}
- As such, limited evidence exists on real-world HRU and costs among patients taking nusinersen, especially in the United States.

Methods

- Number and costs (\$US 2020) of inpatient hospitalizations, not relating to nusinersen administration or observational care, were evaluated over 12 months pre- and post-nusinersen initiation.
- Using pre/post-study design, patients who met the inclusion criteria served as their own controls.
- Results were stratified by age: pediatric (< 18 years) and adult (≥ 18 years).
- Descriptive analysis was presented for HRU and costs due to limited sample size and that the majority of patients did not have an inpatient hospitalization event during the study period.
- Due to the pre/post-study design, infants aged 0–1 years and/or those identified via newborn screening, who were likely to have severe symptoms, were excluded from the analysis.
- Patients who did not have complete information on the date of nusinersen initiation were excluded so as to capture treatment use starting from loading dose phase.

^aNusinersen treatment codes included HCPCS codes J2326 and C9489 and NDC codes 64406-0058-01 and 64406-058-01; SMA ICD-10 diagnosis codes included G12.0, G12.1, G12.8, and G12.9.
^bPatients who received any of the first 4 recorded nusinersen doses in 120-day or greater intervals (which would indicate maintenance doses and not loading doses per US label) were excluded. Patients with ≤ 4 doses were retained as long as the inter-dose intervals for each of the first 4 recorded doses were within 120 days, respectively.
^cEntire study period included data from 01 January 2016 to 30 June 2020 (Commercial) and 01 January 2016 to 31 December 2019 (Medicaid) to allow for 12 months pre- and 12 months post-evaluation of nusinersen use; nusinersen index dates were limited to 01 January 2017 to 30 June 2019 (Commercial) and 01 January 2017 to 31 December 2018 (Medicaid).

Results

Table 1. Baseline Characteristics

Baseline Characteristics	Total n = 103	Pediatric (< 18 y) n = 59	Adults (≥ 18 y) n = 44
Percentage of total study population	100%	57%	43%
Mean age (range) at index date of nusinersen, y	18 (1–63)	9 (1–17)	30 (18–63)
Female, n (%)	44 (42.7)	30 (50.9)	14 (31.8)
Region, n (%) ^a			
Northeast	19 (18.5)	11 (18.6)	8 (18.2)
North central	13 (12.6)	10 (17.0)	3 (6.87)
South	14 (13.6)	4 (6.8)	10 (22.7)
West	13 (12.6)	7 (11.9)	6 (13.6)
Other/unknown	44 (42.7)	27 (45.8)	17 (38.6)
Race/ethnic group, n (%) ^a			
White	31 (30.1)	19 (32.2)	12 (27.3)
Black	4 (3.9)	3 (5.1)	1 (2.3)
Hispanic	3 (2.9)	2 (3.4)	1 (2.3)
Other/unknown	65 (63.1)	35 (59.3)	30 (68.2)
Database, n (%)			
Commercial	59 (57.3)	32 (54.2)	27 (61.4)
Medicaid	44 (42.7)	27 (45.8)	17 (38.6)

^aData for region were only available for patients identified in Commercial claims, and data for race were only available for those identified in Medicaid claims.

References 1. De Vivo DC, et al. *Neuromuscul Disord*. 2019;29(11):842-856. 2. Darras BT, et al. *Neurology*. 2019;92(21):e2492-e2506. 3. Mercuri E, et al. *N Engl J Med*. 2018;378(7):625-635. 4. Finkel RS, et al. *N Engl J Med*. 2017;377(18):1723-1732. 5. Hagenacker T, et al. *Lancet Neurol*. 2020;19(4):317-325. 6. Maggi L, et al. *J Neurol Neurosurg Psychiatry*. 2020;91(11):1166-1174. 7. Menard J, et al. *Pediatr Pulmonol*. 2022;57(6):1505-1512. 8. Chen KA, et al. *Paediatr Respir Rev*. 2021;39:54-60. 9. Youn B, et al. *Adv Ther*. 2023;40(3):1129-1140. 10. Johnson NB, et al. *J Neuromuscul Dis*. 2021;8(4):569-578. **Disclosures** CZ, BY, ADP, SR, BAN, and NB: employees of and hold stock/stock options in Biogen; CZ: scientific advisory or data safety monitoring board member for Biogen and Sarepta; research support from Biogen. The institution of Dr. Zaidman has received research support from Novartis. **Acknowledgments** This study was sponsored by Biogen (Cambridge, MA, USA). Writing and editorial support for the preparation of this presentation was provided by Excel Scientific Solutions (Fairfield, CT, USA); funding was provided by Biogen.

Figure 1. Cohort Diagram Using Merative MarketScan® Data

Data from 01 January 2017 to 30 June 2020 for Commercial and 01 January 2017 to 31 December 2019 for Medicaid claims^a

Patients with ≥1 code for nusinersen treatment and SMA ICD-10 diagnosis codes during the study period^a
n = 482

Patients who were likely to have complete information on the date of nusinersen initiation^b
n = 291

Patients with 12 mo continuous enrollment pre- and post-index^c (first date of nusinersen)
n = 103

Figure 2. Total Number of Inpatient Admissions and Days Spent in Hospital Decreased Post-Nusinersen Treatment Across Age Cohorts

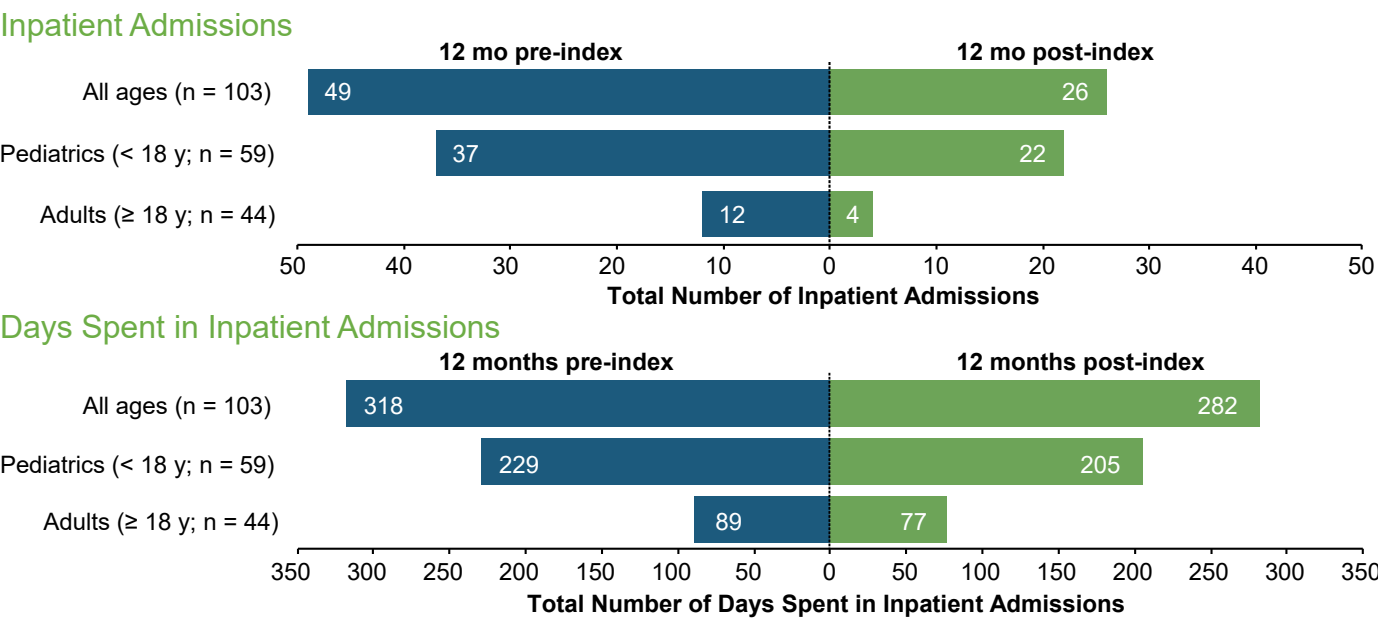
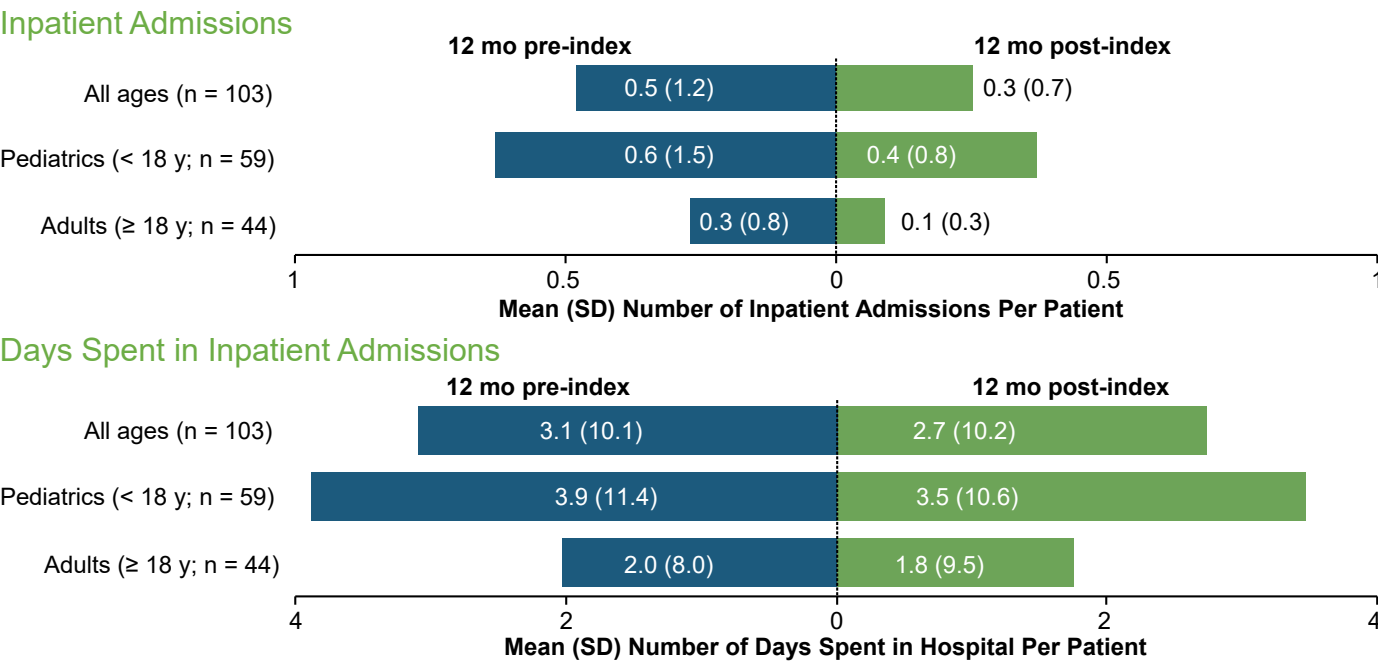


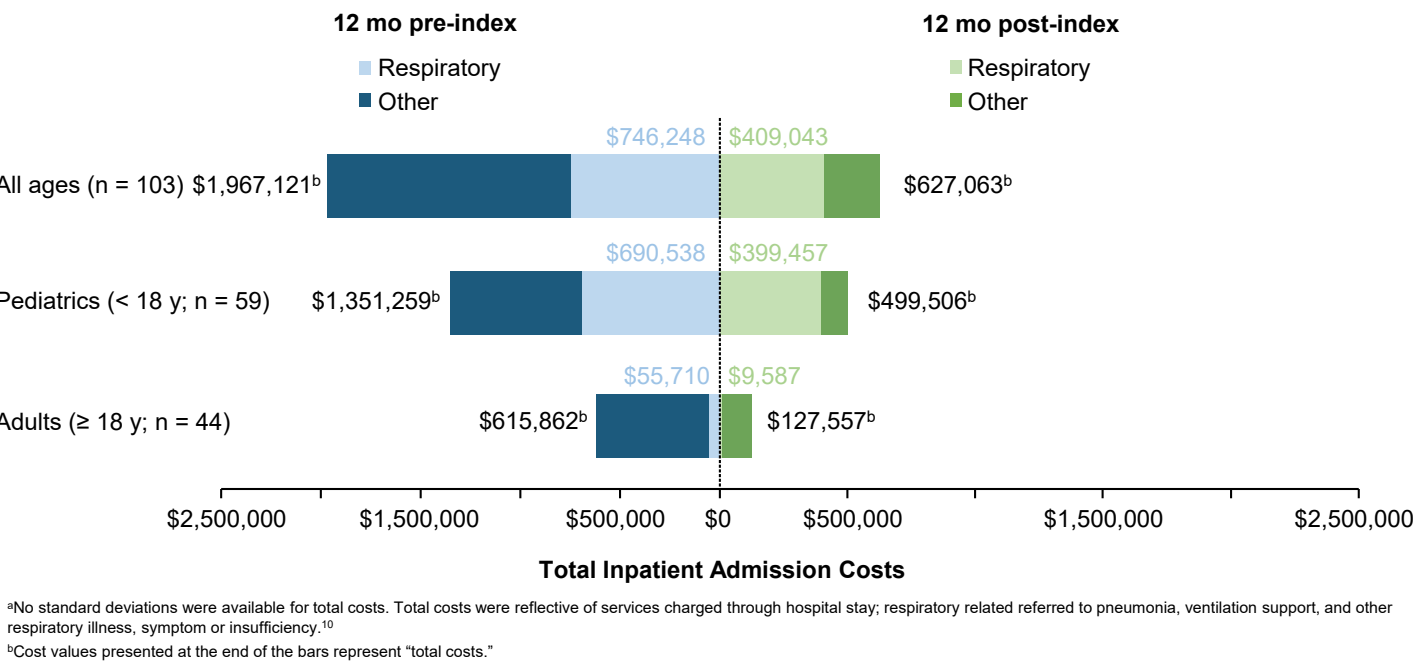
Figure 3. Average Inpatient Admissions and Days Spent in Hospital Decreased Post-Nusinersen Treatment Per Patient Across Age Cohorts



Cohort	Mean (SD) Difference Per Patient in Inpatient Admissions	Cohort	Mean (SD) Difference Per Patient in Days Spent in Inpatient Admissions
All ages (n = 103)	-0.22 (1.07)	All ages (n = 103)	-0.35 (10.63)
Pediatrics (< 18 y; n = 59)	-0.25 (1.21)	Pediatrics (< 18 y; n = 59)	-0.41 (8.92)
Adults (≥ 18 y; n = 44)	-0.18 (0.87)	Adults (≥ 18 y; n = 44)	-0.27 (12.68)

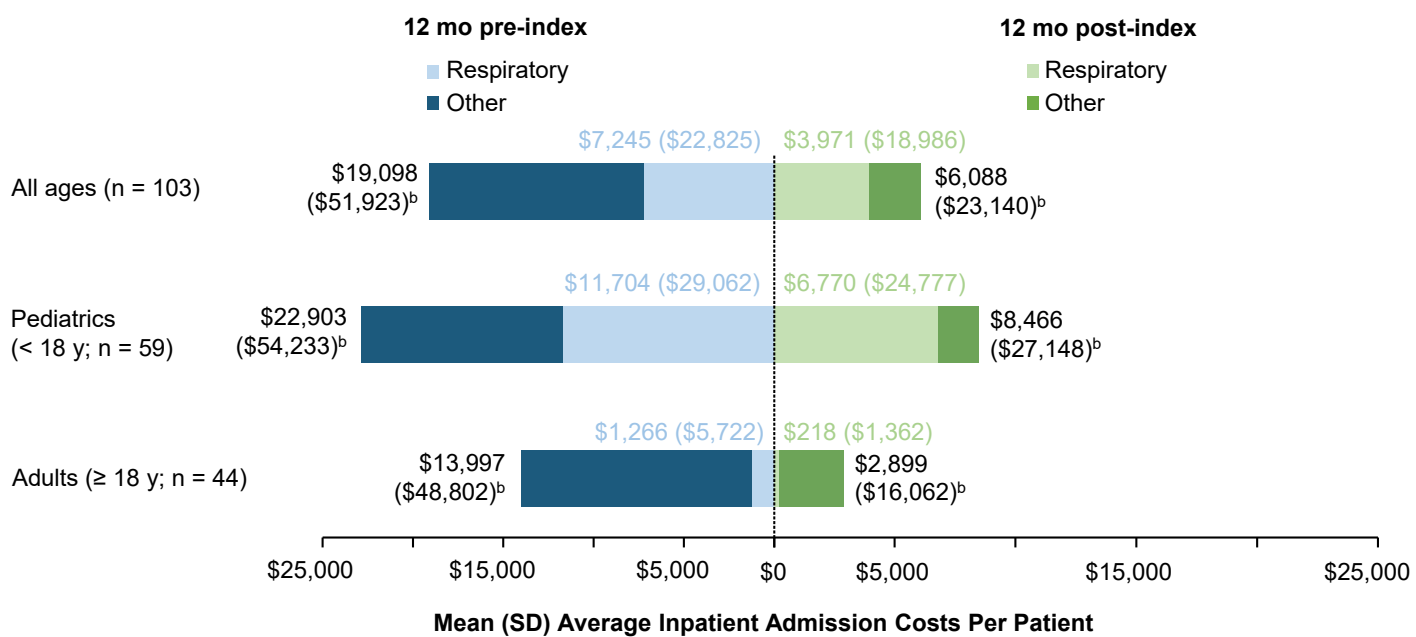
Acronyms: ED = emergency department; HCPCS = Healthcare Common Procedure Coding System; HRU = healthcare resource utilization; ICD-10 = International Classification of Diseases, 10th revision; NDC = national drug code; SMA = spinal muscular atrophy

Figure 4. Total Costs^a of Inpatient Admission Decreased Post Nusinersen Treatment Across Age Cohorts



^aNo standard deviations were available for total costs. Total costs were reflective of services charged through hospital stay; respiratory related referred to pneumonia, ventilation support, and other respiratory illness, symptom or insufficiency.¹⁰
^bCost values presented at the end of the bars represent "total costs."

Figure 5. Average Costs^a of Inpatient Admission Decreased Post Nusinersen Treatment Per Patient Across Age Cohorts



^aTotal costs were reflective of services charged through hospital stay; respiratory related referred to pneumonia, ventilation support, and other respiratory illness, symptom or insufficiency.¹⁰
^bCost values presented at the end of the bars represent "total costs."

- For ED visits, similar trends were observed for the pediatric cohort (data not shown).
 - Average ED costs per patient decreased from 12 months pre-index to post-index period for the pediatric cohort: \$1,707 versus \$757.