Spinal Muscular Atrophy (SMA) Disease-Related Complications Decreased Over Time After Start of Nusinersen Treatment

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Objective

- To describe common SMA disease-related complications over time in patients treated with nusinersen using real-world data from an administrative database including a large network of hospitals in the United States.

Conclusion

- SMA-related complications decreased over time with nusinersen treatment in both pediatric and adult cohorts, suggesting potential improvement of healthcare resource utilization associated with treatment.

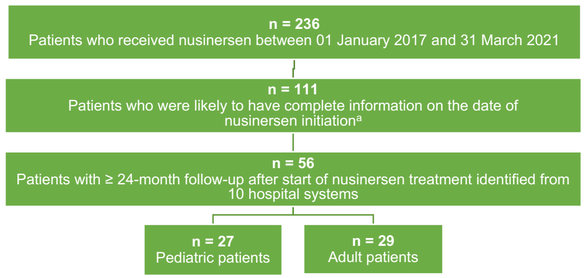
Introduction

- Nusinersen has demonstrated efficacy and effectiveness in patients with spinal muscular atrophy (SMA).[1] More research is needed to understand the real-world benefits of nusinersen treatment with regards to symptoms and complications.[2]
- The objective of this study was to describe common SMA-related complications over time in patients treated with nusinersen using the PINC AI™ Healthcare Database (PHD), an administrative database from a large network of US-based hospitals.

Methods

- Patients who likely received their first nusinersen dose[3] (index admission) between 01 January 2017 and 31 March 2021 were included. Patients were followed from index date to their last discharge date up to 30 September 2021.
- Nine SMA-related complications were selected from previous research using US clinician input and assessed for patients with ≥ 24-month follow-up over 3 time periods: index to 6 months, 6 to 12 months, and 12 to 24 months (normalized to 6-month values).
- The complications, defined using diagnosis and procedure codes, were pneumonia, ventilation support including tracheostomy, gastrointestinal complications including gastrostomy tube, musculoskeletal problems, support/therapy in the form of occupational/speech therapy and wheelchairs, orthopedic surgery, and other respiratory illnesses.[2]
- Unadjusted descriptive analyses were conducted, and results were stratified by age: pediatric (< 18 years) and adult (≥ 18 years).

Figure 1. Cohort Diagram



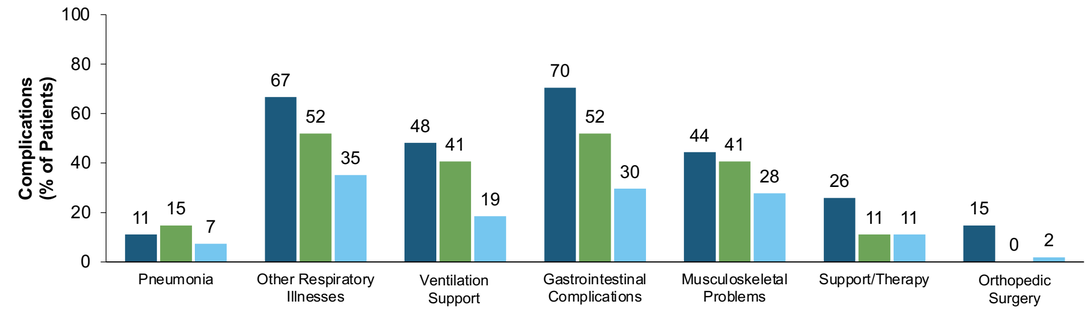
[a]Patients who received any of the first 4 recorded nusinersen doses in 120-day or greater intervals (which would indicate maintenance doses not loading doses per US label) were excluded. Patients with ≤ 4 doses were retained as long as the interdose intervals for each of the first 4 recorded doses were within 120 days, respectively.

Table 1. Patient Characteristics at Index

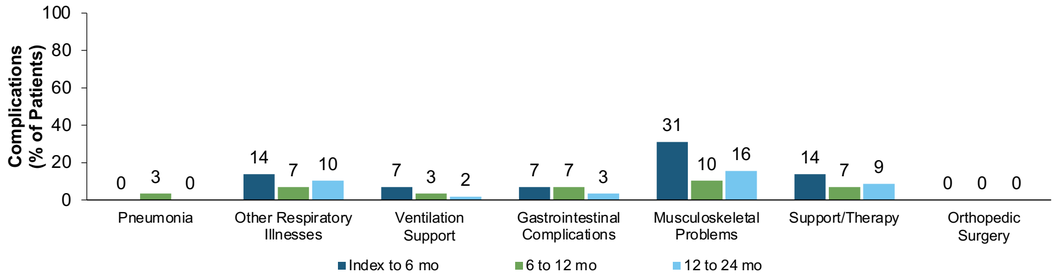
Patient Characteristic	Total	Pediatric (< 18 y)	Adult (≥ 18 y)
Number of unique patients	56	27	29
Age, y, mean (SD)	22 (18)	6.3 (4.7)	36 (13)
Sex, n (%)			
Female	28 (50)	16 (59)	12 (41)
Male	28 (50)	11 (41)	17 (59)
Race, n (%)			
White	42 (75)	17 (63)	25 (86)
Black	5 (8.9)	4 (15)	1 (3)
Other/unknown	9 (16)	6 (22)	3 (10)
Primary payer at index, n (%)			
Medicare	10 (17.9)	0 (0)	10 (34.5)
Medicaid	24 (42.9)	19 (70.4)	5 (17.2)
Private insurance	21 (37.5)	7 (25.9)	14 (48.3)
Other/unknown	1 (1.8)	1 (3.7)	0 (0)
Index treatment setting, n (%)			
Inpatient	4 (7.1)	3 (11.1)	1 (3.4)
Outpatient	52 (92.9)	24 (88.9)	28 (96.6)
Provider region at index, n (%)			
Northeast	0 (0)	0 (0)	0 (0)
Midwest	12 (21.4)	10 (37)	2 (6.9)
South	41 (73.2)	14 (51.9)	27 (93.1)
West	3 (5.4)	3 (11.1)	0 (0)
Number of nusinersen doses administered			
Mean (SD)	8.5 (4.1)	8.3 (4.6)	8.7 (3.6)

Figure 2. SMA-Related Complication Rates Decreased Over Time With Nusinersen Treatment

(A) Pediatric Patients, n = 27



(B) Adult Patients, n = 29



SMA = spinal muscular atrophy

Results and Limitations

- Percentage of patients with SMA-related complications decreased over time among pediatric and adult patients.
- We could not ascertain disease severity in the PHD.
- We could not follow patient across multiple hospitals.

Spinal Muscular Atrophy (SMA)

Nusinersen (Spinraza®)

PINC AI™ Healthcare Database

References 1. SPINRAZA (nusinersen) injection, for intrathecal use [full prescribing information]. Cambridge, MA: Biogen; 2016. 2. Johnson NB, et al. *J Neuromuscul Dis.* 2021;8(4):569-578. 3. Youn B, et al. *Adv Ther.* 2023;40(3):1129-1140. Disclosures NBJ, BY, CZ, SR, and ADP: employees of and hold stock/stock options in Biogen. VA, ZC, and GSL: employees of Premier at the time of the study, which received consulting fees from Biogen for conducting the study.
Acknowledgments This study was sponsored by Biogen (Cambridge, MA, USA). Editorial support for the preparation of this presentation was provided by Excel Scientific Solutions (Fairfield, CT, USA): funding was provided by Biogen.