Clinical and Economic Burden of Spinal Muscular Atrophy in France

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Introduction

Spinal muscular atrophy (SMA) is a rare, progressive genetic disease affecting the nervous system causing muscle weakness and atrophy. Historical data on the clinical and economic burden of SMA in France is limited and therefore this study aimed to address this by assessing health care resource use (HCRU) and associated costs of patients with SMA, using the national French health insurance database.

Method

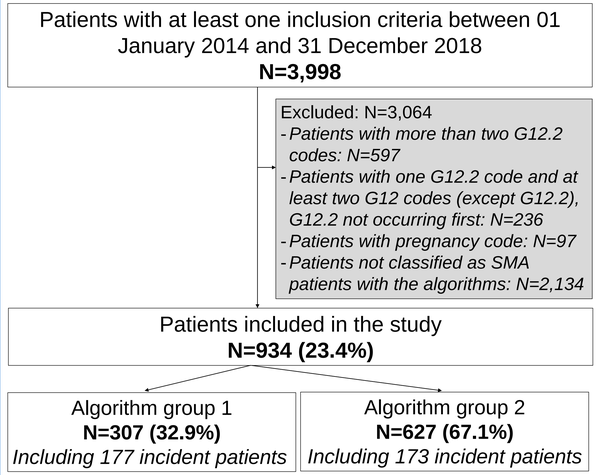
A retrospective cohort study between 2014 and 2018 using data from the national French health insurance database (SNDS) was conducted. As the ICD-10 classification does not include codes sufficiently specific to distinguish the different types of SMA, an exploratory algorithm was deployed to try and classify SMA type 1 ("algorithm group 1") and SMA Type 2-4 ("algorithm group 2"). The exploratory algorithm was based on ICD-10 codes, Nusinersen use, presence of g-tube codes and patient age.

The index date was defined as the first occurrence of an inclusion criterion among the first SMA diagnosis of hospital stay (ICD-10 code G12), the beginning of the Long Term Disease (LTD) status or the first dispensing of Nusinersen recorded over the study period, whichever comes first.

HCRU and associated costs from a global perspective were described per month, by algorithm group and by age. End-of-life costs were also described.

Results

Study population



170 patients initiated Nusinersen from its Marketing Authorization in 2017 to end of study period, including 57 patients in algorithm group 1 and 113 patients in algorithm group 2.

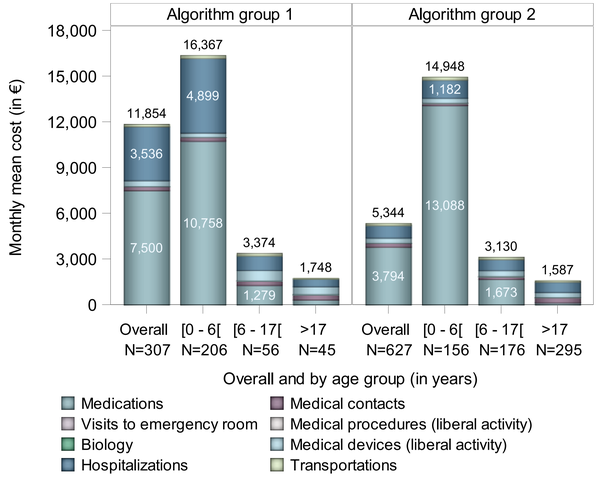
Patient's characteristics at index date

- In patients in algorithm group 1 and group 2, 54% and 57% were male and the median age was 8 months and 17 years, respectively.
- Algorithm group 1 included 101 patients (33%) aged 6 years and over, demonstrating imprecision of the algorithm to classify by SMA type.
- Median follow up time in algorithm group 1 and group 2 was 16 months and 59 months, respectively.
- During the follow-up, 44% and 7% of patients in algorithm group 1 and group 2 died, respectively.

Healthcare Resource Use

- 96% of algorithm group 1 and 91% of algorithm group 2 patients with at least one hospitalization (mean number: 0.8 and 0.3 hospitalization/month, respectively).
- Mean number of medical contacts : 8/month (of which around 60% were visits to physiotherapists).
- 61% and 36% of patients in algorithm group 1 and group 2 with at least one ventilation procedure, respectively, and ~60% with at least one breathing support.
- Mean number of medication reimbursements: 5/month

Economic burden of patients



- Overall monthly costs excluding medication related costs (€4,226 [group 1] and €1,572 [group 2]), were mainly driven by hospitalizations and medical devices costs. These costs decreased with age in patients in algorithm group 1.
- When excluding medication costs, end-of-life costs were higher than the other costs in both subgroups (€6,665 [group 1] and €4,331 [group 2] per month), mainly driven by hospitalization costs (92% and 80% respectively).

Discussion/Conclusion

This study highlights the high economic burden of SMA. The costs were higher in patients in algorithm group 1 (compared to patients in algorithm group 2), especially in younger patients, and in the last months before death. The per month per patient unit reporting is challenging for the drug costs given that not all patients are exposed to all drugs with the same frequency over the whole study period. For example, Nusinersen was launched in France during the study period (2017) and the number of injections are more frequent shortly after treatment initiation. Moreover, the true cost of medicines after confidential rebates is not reflected in this analysis. The clinical benefit of disease modifying therapy was out of scope of this analysis.

Additionally, the misclassification of some older patients into algorithm group 1 showed that further work is also needed to refine an algorithm to accurately classify SMA

Reference

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Acknowledgments

We thank the French National Health Service (Caisse National de l'Assurance Maladie) for providing data.

This work was funded by F. Hoffman-La Roche Ltd.

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