Healthcare pathways and therapeutic outcomes of patients with spinal muscular atrophy

Results from the 12-year real-world study EPI-SMA based on the French National Healthcare Database (SNDS)

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Background

Spinal Muscular Atrophy (SMA) is a genetic disease affecting the central and peripheral nervous system, and voluntary muscle movement.

In the past years, 3 innovative therapies have been launched in France, improving the management of patients with SMA: nusinersen with an access via nominative early access program since mid-2016, onasemnogene abeparvovec since 2019 and risdiplam since 2020.

Objective

This study was designed to provide real-world data regarding the management of therapeutic options, the healthcare pathways/patient journeys, and costs from 2011 up to 2022.

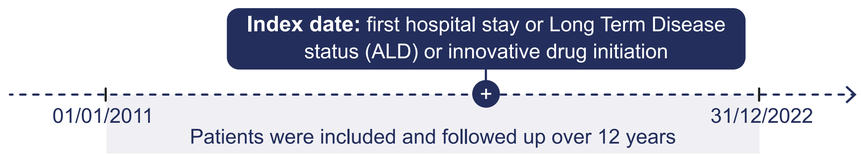
Methodology

Data sources

This study was conducted on the French National Health Insurance database (SNDS), which contains socio-demographic data and information on all health care expenses. The SNDS covers about 99% of the population living in France.

Although diagnoses, purpose of outpatient visits, and results of laboratory tests are not available, the SNDS uses the International Classification of Diseases 10th revision (ICD-10), which provides medical information related to hospital stays.

Study period



Study population

Patients with at least one diagnosis code of SMA (ICD-10 codes G12.0, G12.1, G12.8 and G12.9) in hospital stay **OR** LTD (long term disease) **OR** one delivery of innovative therapies between January 1, 2011 and December 31, 2022 were included.

Identification of subgroup

SMA1	SMA2	SMA3
(G12.0 or G12.1) ***OR*** (G12.8 or G12.9 with at least one administration of one of the 3 innovative therapies) ***AND*** Age < 12 months at index date	(G12.0 or G12.1) ***OR*** (G12.8 or G12.9 with at least one administration of one of the 3 innovative therapies) ***AND*** 12 ≤ age < 27 months at index date	With at least one administration of one of the 3 innovative therapies ***AND*** not treated by riluzole (used only for amyotrophic lateral sclerosis) ***OR*** G12.0 ***AND*** ≥ 24 years old at index date

G12.0 Infantile spinal muscular atrophy, type I; G12.1 Other inherited spinal muscular atrophy; G12.8 Other spinal muscular atrophies and related syndromes; G12.9 Spinal muscular atrophy, unspecified

Statistical analysis

Treated patients all had at least one delivery of nusinersen, risdiplam and/or onasemnogene abeparvovec during the study period.

Analyses were performed on incident patients (i.e. patients with an ICD10 code of SMA identified before January 2011 were excluded).

Both descriptive analyses and process mining methods were used to describe healthcare pathways.

Care consumption was valued according to the National Health Insurance perspective in € 2023 according to the years of follow-up.

Conclusion

This study provides a visualization of SMA healthcare pathways by subtype SMA1, SMA2 and SMA3, even if differentiating these patients with identification criteria in the database proved to be difficult as there is a continuum in the disease progression.

This vizualisation shows that there is a high diversity of healthcare pathways especially for SMA1 treated patients.

Despite a strong improvement of the management and survival of these patients following the arrival of the 3 specific SMA therapies, the burden of this disease remains high.

The majority of treated patients in this study received chronically administered therapies. Treated patients tend to be followed up more frequently.

Regulatory statement

This SNDS study was registered with the HDH under the reference T94955362022081, was approved by CESREES on 22 September 2022 and authorised by the CNIL on 30/11/2022 (DR-2022-258 (request 922250))) - CNAM agreement signed on 12/09/2023.

Acknowledment

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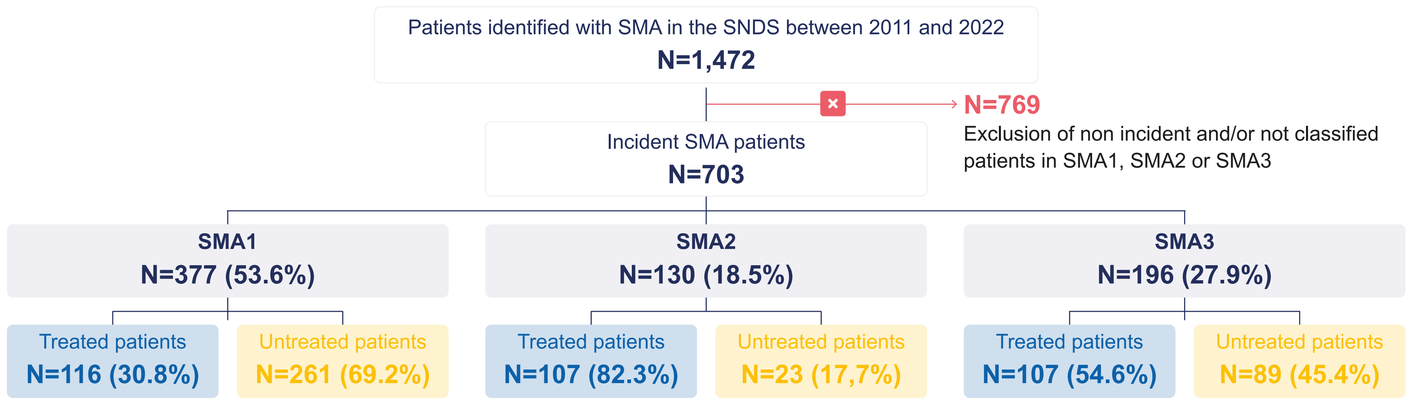
Results

Study population



Baseline characteristics

SMA1	SMA2	SMA3
Median age (Q1; Q3) **4.0 months** (2.0; 7.0)	Median age (Q1; Q3) **18.0 months** (14.0; 22.0)	Median age (Q1; Q3) **188.5 months** (107.0; 372.0)
Female **46.0%**	Female **53.7%**	Female **47.7%**
Median follow-up time* (Q1; Q3) **0.3 years** (0.1; 2.1)	Median follow-up time* (Q1; Q3) **4.8 years** (2.1; 7.7)	Median follow-up time* (Q1; Q3) **3.3 years** (1.3; 8.5)

*Time from index date up to the end of study period (31/12/2022), or death, or last healthcare claim before 2 years without any healthcare claim, whichever occured first.

Healthcare pathways

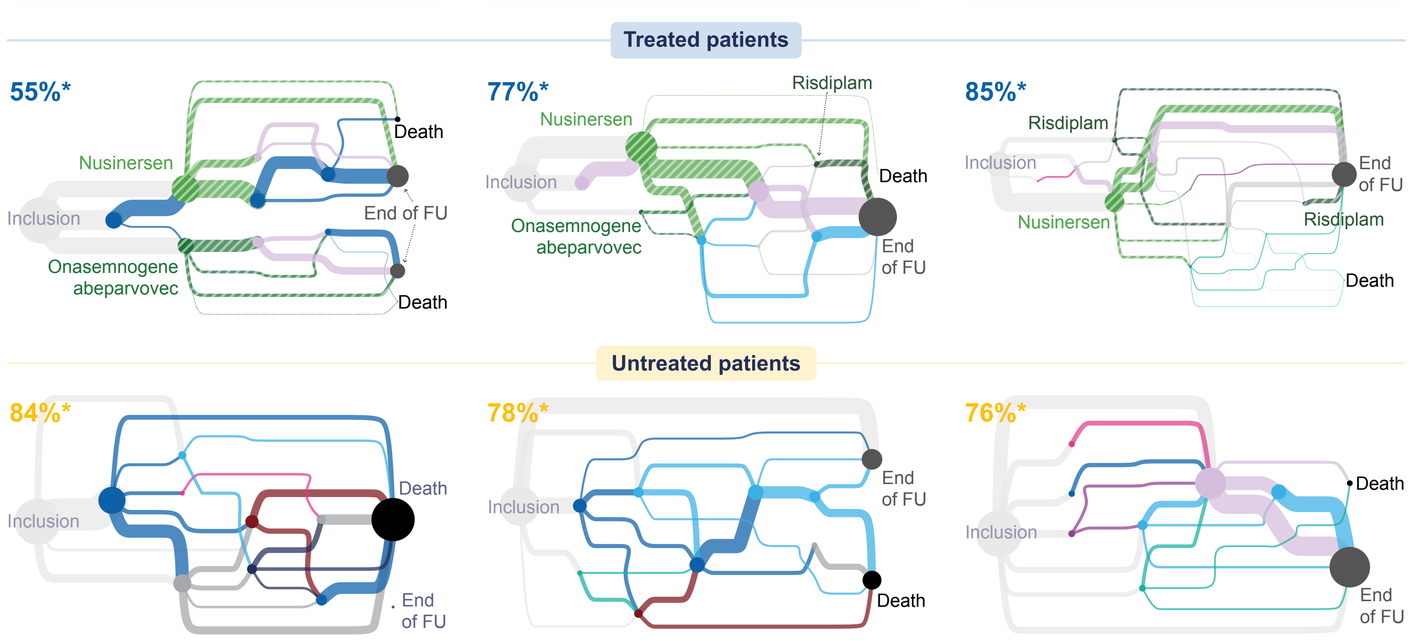
How to read the graph

The size of each node and arc is proportional to the number of patients involved. The graph represent the order in which the event occurred. Duration between each event is not displayed on the graph but is available as descriptive information complementary to the graph.

How to interpret the graph (exemple for the 116 SMA1 treated patients)

Most of patients (60%, or 70 patients) received nusinersen, either as the first treatment or after respiratory complications. Only 14 patients died following treatment. Respiratory complications occurred in 38 patients, with 37 experiencing a second one. Additionally, 17 patients required mobility assistance after nusinersen.

Among the 40 patients (34%) treated with onasemnogene abeparvovec, most had no prior respiratory complications. Their journey differed, with 26 patients (22%) needing mobility support and 16 developing respiratory complications.



*Percentage of patients' healthcare pathways represented by each graph (replayability score)

Legend: Main healthcare events identified during the follow-up

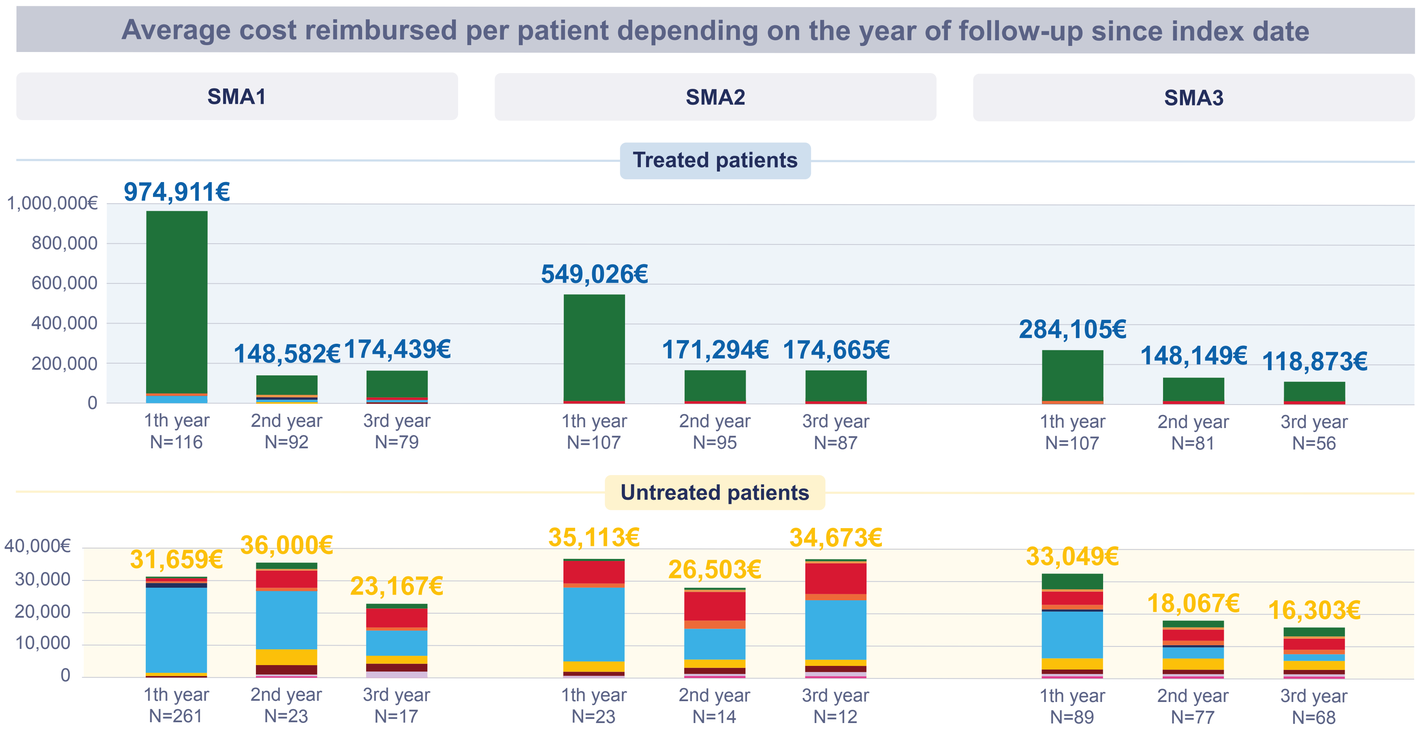
Treatment initiation Risdiplam · Onasemnogene abeparvovec · Nusinersen

Hospitalizations ICU · Hospitalizations > 7 days · Respiratory complication · Hospitalization at home

Support implementation Orthopaedic/spinal surgery · Mobility support · Respiratory support · Nutritional support

Inclusion · Treatment discontinuation · Palliative care · End of FU (follow-up) · Death

Average cost reimbursed per patient depending on the year of follow-up since index date



Legend : Main type of costs

Medications · Hospitalization (excluding expensive medications reimbursed in hospital *("liste en sus")* and drugs in early access programs *(ATU/post ATU)*)

Hospitalization at home · Mobility support · Respiratory support · Nutritional support

Nursing and paramedical care · Other type of costs · Transports · Medical devices (other than support/nutrition/respiratory support)