

Spinal muscular atrophy treatments in France: what are the organizational, economic, and environmental impact of oral and intrathecal administrations ?



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Context

- Spinal Muscular Atrophy (SMA) is a rare genetic neuromuscular disease characterized by progressive muscle weakness and atrophy, resulting from the degeneration of motor neurons responsible for movement.
- **In France, the national prevalence is estimated at approximately 750 adults' patients¹.**
- Currently, **two treatments are available** : an **intrathecal therapy** administered in a hospital setting, and an **oral therapy** dispensed through community pharmacies.
- Hospital-based intrathecal administration is associated with significant time and logistical burdens for patients with motor impairments and their caregivers.

Objectives

The aim of this study is to compare the organizational, economic, and environmental impacts of intrathecal and oral administrations in the management of SMA from health insurance, patient, caregiver, and health care professionals' perspectives.

Methods

- A **multidimensional impact model** was developed to quantify the time spent by health care professionals, patients and caregivers, as well as public payer costs, and carbon emissions. It **simulates the care pathway of adult patients** receiving either intrathecal or oral treatment over the first year period.
- Impacts on healthcare professionals, health insurance and the environment are evaluated at the population level (based on prevalence), while the consequences for patients and caregivers are assessed per patient.
- **This study focuses only on treatment administration.** It did not include the time needed for hospital-based functional assessments, even though these are performed for both administration methods.

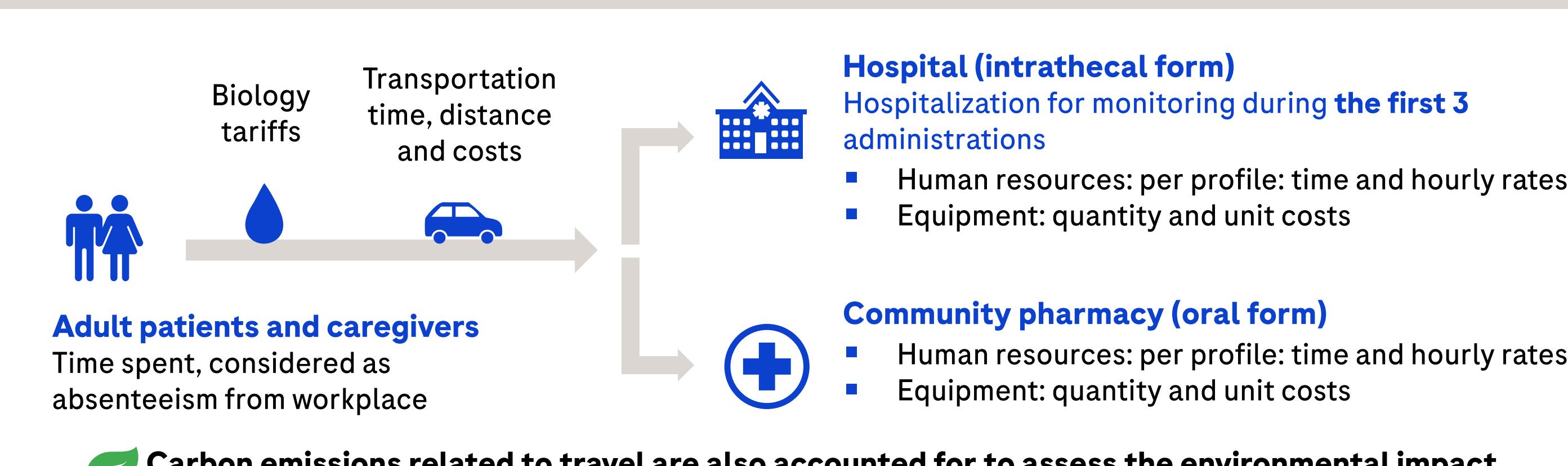


Figure 1. Patients' pathway

Hospital and pharmacy	<ul style="list-style-type: none">• <u>Profil, equipment, time:</u> expert opinions• <u>Valuation:</u> pay grids²
National health insurance	Published tariffs
Patients	<ul style="list-style-type: none">• <u>Time:</u> expert opinion• <u>Transportation:</u> PASO DOBLE study³, FINAUT report⁴• <u>Absenteeism:</u> national statistics^{5,6}

Table 1. Data source per perspective

Results per perspective

Health care professional perspective

For a cohort of 750 patients over the first year:

- **Intrathecal administration** mobilizes a neurologist, nurse, carer, radiologist and hospital porter, requiring a total of **11,606 hours**.
- The **oral form** requires a pharmacist and a pharmacy technician for a total of **6,500 hours**.

Transferring care to oral administration in community pharmacies **would free up 11,606 hospital healthcare hours**, which could be reallocated to other clinical needs.

Patient & caregiver perspective

On average, per patient and caregiver, over the first year:

- **The intrathecal treatment requires a total of 72 hours**, with the patient committing 66 hours and the caregiver 6 hours. Whereas, the **oral form** is much less time-intensive, requiring **only 1 hour from the caregiver**.
- The intrathecal form has an economic valuation of 812 €, while the oral form is valued at 12 €.

This model highlights that the oral form **saves 66 hours** for the patient and **reduces costs**.

National insurance perspective

For a cohort of 750 patients over the first year:

- Intrathecal administration generates **€2,794,669** to health insurance. This includes transportation, anesthesia, laboratory tests, and drug administration.
- For the oral form, costs are primarily associated with biology, then drug manufacturing and dispensing, totaling **133,215 €**.

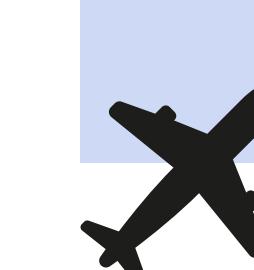
This represents a potential saving of **€2,6 million for health insurance**, due to fewer hospitalizations and transportations.

Environmental perspective

In the first year, the carbon footprint for treating 750 patients is estimated as follows:

- **51,660 tones of CO₂** for the intrathecal administration

This represents **14 round-trip flights from Paris to Tokyo** for one person!



- **10,969 tones of CO₂** for the oral form

The carbon footprint for the intrathecal administration is nearly five times greater than that of the oral form.

Conclusion

The findings demonstrate to multiple stakeholders the advantages of shifting from intrathecal to oral administration in the management of SMA, promoting a more sustainable healthcare approach with optimization of resources, reducing caregiver burden and improving patients' quality of life.

¹ Registre français des patients avec une amyotrophie spinale. Newsletter de Février 2025

² Grilles indiciaires hospitalières available on www.emploi-collectivites.fr

³ Cabon M. Eur J Public Health. 2024 Oct 1;34(5):866-871.

⁴ Le coût des déplacements pour les voyageurs. FINAUT, #281. Mars-avril 2020

⁵ Le handicap en chiffres. Rapport de la DRESS, édition 2023

⁶ Aider un proche, quel lien avec l'activité professionnelle. DARES analyses #081. Décembre 2017