

COST-SAVING IMPACT OF USING NUSINERSEN BY CLINICAL TRIALS FOR SPINAL MUSCULAR ATROPHY

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BACKGROUND AND IMPORTANCE

Nusinersen is an antisense oligonucleotide that increases the production of full-length functional survival of motor neuron protein.

- It was the first disease modifying therapy approved for **Spinal Muscular Atrophy 5q (SMA)**.
- **SMA** is a progressive neuromuscular rare disease, however the cost of available treatments implies a high economic burden for the sanitary system.

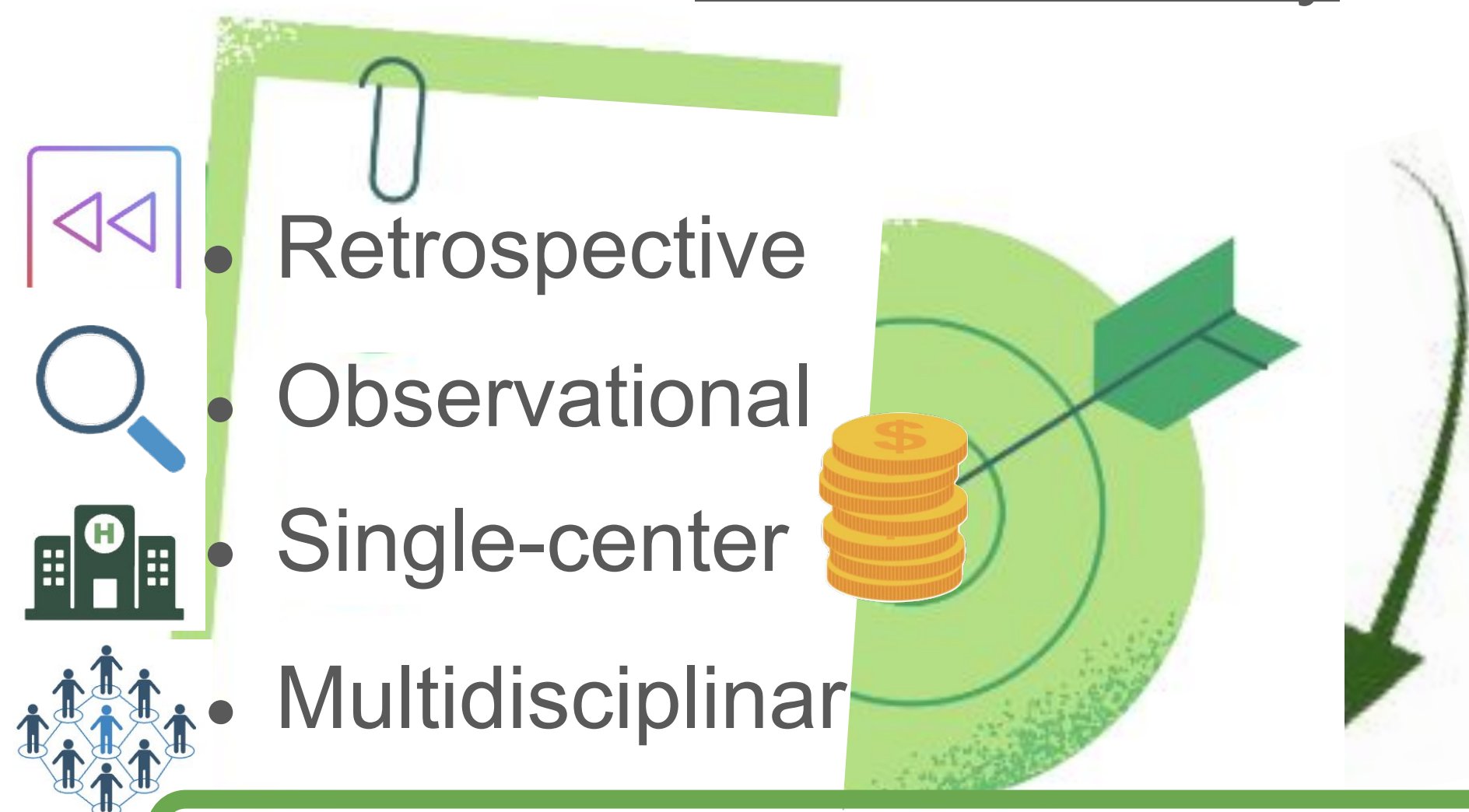
AIM AND OBJECTIVES

To analyze the **economic advantage** of treating **SMA** in clinical trials (CT) with **nusinersen** provided by the sponsor.



MATERIAL AND METHODS

Economic study



- Data: **FarmisOncofarm**[®] and **pKensayos**[®] (clinical data), **OrionLogis**[®] (economic data)
- **Variables** analyzed: age, anthropometric data, diagnosis, pharmacotherapeutic data (cycle received and administrations), and consumption data (preparations and avoided costs)
- **Results expression**: percentage, median [interquartile range (IQR)]

Cost-saving impact of the use of intrathecal nusinersen in CT between February 2021 and September 2023.

RESULTS

2 CT: Phase II/III + Phase III extension

	Pediatrics	Adults	mg/patient (mg)	cost/patient (€)
N	5 (71.4%)	2 (28.6%)	240.0	886,389.2
Age (years, median, [IQR])	2.8 [2.5-7.4]	34.8 (31.4; 38.1)	296.0	1,093,213.3
Weight (kg, median, [IQR])	10.0 [6.5-23.0]	78.5 (48.8; 109.0)	162.0	598,312.7
			134.0	3,827.3
			206.0	760,817.4
			178.0	3,871.3
			178.0	3,871.3
Total			1394.0	3,350,302.4
Median [IQR]			178.0	598,312.7
			[162.0-240.0]	[3,871.3- 886,389.2]

Table 1. Patient characteristics

Table 2. Preparations (mg and cost)

	Nusinersen 12 mg
vial cost (€)	44,319.5
cost/mg (€)	3,693.3

Table 3. Cost per vial and cost per mg

Total mg consumption: **1394,0 mg**

↓ 3.693,3 €/ mg

5,148,443.7 €

Global cost saving

2,067,648.1 €

Annual economic impact

Median treatment cost avoided:

per CT: **2,574,221.9 €**

per patient: **598.312,7 €**

	CT 1	CT 2
consumption/CT (mg)	654.0	740.0
cost/CT (€)	2,415,410.5	2,733,033.5
cost avoided/ CT (€, median)	2,574,221.9	

Table 4. Cost per CT

CONCLUSION AND RELEVANCE

SMA is considered one world's most expensive treatment disease, and **nusinersen** is the promotion to participate in SMA CT allows **access to innovative treatments** for patients: aim of **reducing the large underlying budgetary burden**.



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