Budget Impact Analysis of Risdiplam for the Treatment of Spinal Muscular Atrophy in Colombia

Vásquez E[1], Lasalvia P[2], Suárez F[3], Quitian D[4]

[1]Neuroeconomix, Bogota, Colombia, [2]NeuroEconomix, Bogota, Colombia, [3]Pontificia Universidad Javeriana, Bogota, Colombia, [4]Productos Roche S.A., Bogota, Colombia

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INTRODUCTION

- Spinal muscular atrophy (SMA) is a rare, hereditary neurodegenerative disease leading to loss of motor function and reduced life expectancy, which is the result of a survival motor neuron (SMN) protein deficiency [1, 2]. Risdiplam, a survival of motor neuron 2-directed (SMN2) splicing modifier is the only oral drug approved to treat SMA [3].

OBJECTIVES

- To estimate the budgetary impact of risdiplam for SMA treatment from the Colombian health system perspective.

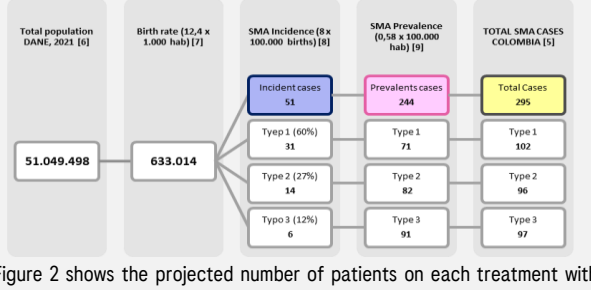
METHODS

- A global budget impact model was adapted to the Colombian perspective. Incident and prevalent patients with type 1-3 SMA were estimated using Colombian public databases and a published literature review.
- Two market scenarios were assessed over a 5-year time horizon: without risdiplam and with risdiplam. In the first scenario the market was constituted by nusinersen (12% to 24% of the market share from year 1 to 5) and the best supportive care (BSC); in the second scenario risdisplam increased its market share from 4% at year 1 until reaching 20% at year 5.
- Public databases and published articles were used to estimate target population (Figure 1).
- Drug acquisition, administration, follow-up and adverse events costs were considered (table 1-3). Cost-generating events were identified using a base case validated with clinical experts. Costs were estimated using public rate manuals for the January 2022 period and were expressed in USD - (COP 4.011,08) [4]. None assumptions regarding adherence, treatment discontinuation, stopping rules were considered to calculate costs
- Two different prices were considered for risdiplam: i) base case scenario and ii) upside price according international prices.
- Budget impact is calculated as difference between scenario with risdiplam – scenario without risdiplam.

RESULTS

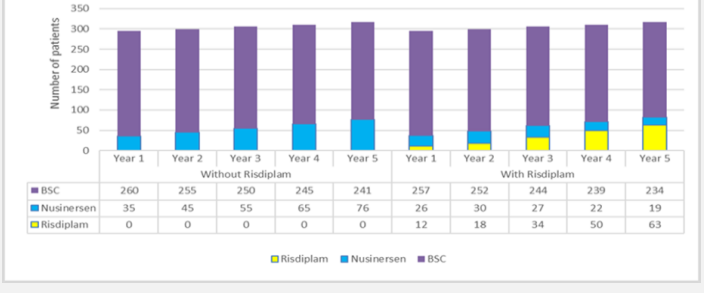
- A total of 51 incident cases of SMA were estimated in Colombia for the year 2021 and correspond to 60%, 28% and 12% for type 1, 2 and 3, respectively. The prevalent cases were associated with the SIVIGILA [5] report, where the population objective was 295 for the first year (Figure 1).

Figure 1. Population algorithm for Budget impact



- Figure 2 shows the projected number of patients on each treatment without risdiplam and with risdiplam, and their increase after the first year.

Figure 2. Number of patients on each treatment with and without Risdiplam



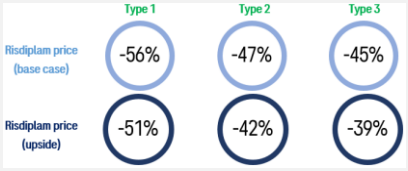
- Considering only cost of treatment, Risdiplam generates savings per patient during the first year compared to nusinersen in both price scenarios (Table 1 and Figure 3).

Table 1. Technologies Cost per patient in Colombia

Technology	mg Total	List Price	Year 1 Cost (USD)	Year 2 Cost (USD)	Source
Risdiplam (base case)	60 (per bottle)	USD 6.899	Type 1: 163.963 Type 2: 195.320 Type 3: 205.571		Roche, Colombia
Risdiplam (upside)	60 (per bottle)	USD 7.611	Type 1: 180.889 Type 2: 215.483 Type 3: 226.791		
Nusinersen	12 (per vial)	USD 61.932	371.591	185.795	SISMED [10]

Risdiplam: Dose is mg per Kg [11]. Calculated according to weight by type of SMA patient (ENSIN [12] and clinical experts validation)
Nusinersen: Loading dose: 4 doses in days 0, 14, 28 y 63 and then every 4 months (SISMED Cost, 2021)

Figure 3. Cost reduction per patient with risdiplam in the first year



- Under the market scenarios considered and the base case price, the progressive adoption of risdisplam impacts the budget by USD 439.862 and USD 969.311 at years 1-5, respectively.
- The impact is stable over the time horizon with an average annual budget impact of 6,46% (min: 5,08% - max: 8,85%) and is mainly explained by more patients accessing to risdiplam instead of BSC.
- With the upside price, risdiplam impacts the budget by USD 695.643,82 and USD 2.342.502,37 at years 1-5 with an average annual budget of 12,23% (min:8,76% - max: 14,84%).

Table 2. Follow-up Cost

Treatment	Total Cost (USD)	Source
Risdiplam	29,91	Calculated by authors – Bottom up methodology Experts validation
Nusinersen	65,46	
BSC	569,36	

- Costs to treat adverse events are USD 1.156,54
- In both cases, cost overrun were mainly related to acquisition´s cost of technologies. There were savings in the cost of drug administration, concomitant medications and general disease management costs with risdiplam use.

CONCLUSIONS

- Risdiplam generates savings per patient in the medications cost during first year compared with nusinersen.
- Risdiplam offers a stable budget impact from the Colombian health system perspective, providing an alternative treatment option for Colombian SMA patients.

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