Budget impact analysis of risdiplam in the treatment of newborn spinal muscular atrophy patients in Poland

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Introduction

Spinal muscular atrophy (SMA) is a rare neurodegenerative genetic disorder characterized by the loss of motoneurons that leads to muscle atrophy and difficulties in breathing and walking, which may require artificial respiration assistance[1,2]. SMA is caused by mutations in the survival motor neuron 1 (SMN1) gene, but the variation in clinical presentation of the disease is largely due to the different number of copies of the SMN2, which is a SMN1 paralogous gene[2,3]. Over 90% of patients, regardless of phenotype, have no more than 4 copies of SMN2[4]. Risdiplam (RIS) is indicated for the treatment of SMA in patients with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with 1 to 4 SMN2 copies[5] and can therefore be used in the vast majority of disease cases.

Despite the availability of 3 therapies in Poland, nusinersen (NUS) since January 2019, risdiplam and onasemnogene abeparvovec (OA) since September 2022[6], there still remains a high unmet need for effective treatment. This is partly due to the very limited reimbursement indication for risdiplam, which is available from 2 months of age for patients with contraindications to nusinersen.

Objective

Given the limited scope of the reimbursement indication, RIS cannot be used in practice in infancy, when the implementation of therapy is most effective. From 2022, all newborns in Poland are covered by the government's SMA screening program[7], but risdiplam therapy is not available for disease cases identified in this way. The aim of this study is therefore to calculate the potential budget impact of extending the reimbursement indication for RIS to include the population of newborn SMA patients.

Method

The target population for the budget impact analysis covers newborn SMA patients with 1-4 SMN2 copies. A time horizon of 2 years was adopted using the public payer perspective. Two scenarios were considered: existing (RIS not reimbursed in the target population in the time horizon) and new (RIS financed in the target population). Market shares in both scenarios are presented in Figure 1.

Current market shares of NUS, OA and estimated uptake of RIS were estimated based on the local National Health Fund (NHF) data[8,9,10] and the opinions of clinical experts. Two cost categories were included: acquisition costs based on official list prices[6] and administration costs based on the current NHF tariff[11]. All cost estimates were made taking into account an exchange rate of EUR 1.00 = PLN 4.32[12]. Given the definition of the target population and the fact that OA is indicated for the treatment of patients with up to 3 copies of the SMN2 gene[13], calculations were performed separately for populations with 1-3 SMN2 copies and 4 SMN2 copies.

Table 1. Assumptions of budget impact analysis

AREA	ASSUMPTION
Target population	Newborn SMA patients with 1 to 4 SMN2 copies
Time horizon	2 years
Therapies	OA, NUS, RIS
Perspective	Public payer (NHF)
Cost categories	Acquisition costs, administration costs
Exchange rate	EUR 1.00 = PLN 4.32
Discount rate	No discounting applied

Key: OA, onasemnogene abeparvovec; NUS, nusinersen; RIS, risdiplam; NHF, National Health Fund; SMN2, survival motor neuron 2; SMA, Spinal muscular atrophy; PLN, Polish Zloty

Table 2. Cost and dose of therapies

COST CATEGORY	RIS	OA	NUS
Acquisition cost	Average EUR 8,200.71 per month (Figure 2) (recommended once daily dose is determined by age and body weight[5] calculated based on WHO Child Growth Standards[14])	EUR 2,088,000.00 per dose (single dose at the start of treatment[13])	EUR 75,750.00 per dose (on days 0, 14, 28 and 63 and every 4 months thereafter[15])
Administration cost	No cost (oral administration)	EUR 125.19 per administration	EUR 128.54 per administration

Key: WHO, World Health Organization; RIS, risdiplam; OA, onasemnogene abeparvovec; NUS, nusinersen

Results

The result of the budget impact analysis is the incremental cost, i.e. the difference between public payer expenditure in the new and existing scenarios (Figure 3).

The cost savings resulting from the extension of the reimbursement indication for risdiplam amount to EUR 5.03M in the 1st year and EUR 11.51M in the 2nd year of the time horizon for a total of EUR 16.54M (Table 3, Figure 4).

Table 3. Budget impact results (millions of EUR)

	1ST YEAR	2ND YEAR	TOTAL
Existing scenario	69.27	72.93	142.20
New scenario	64.24	61.42	125.66
Incremental	-5.03	-11.51	-16.54

Figure 1. Newborn SMA patients starting treatment – market shares in the 1st and 2nd year of the analysis

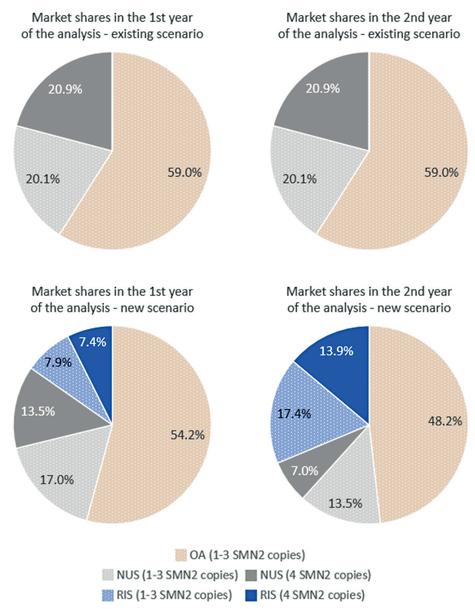


Figure 2. Cost and dose of RIS in the following months of treatment

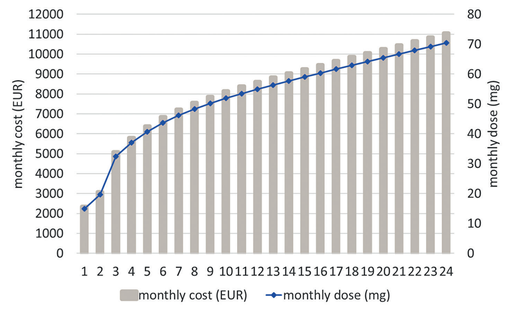
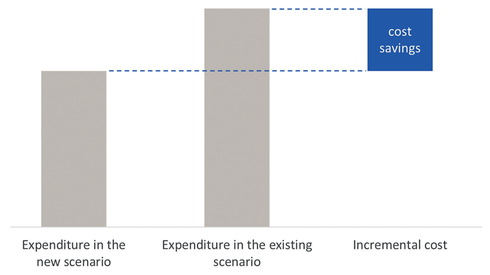
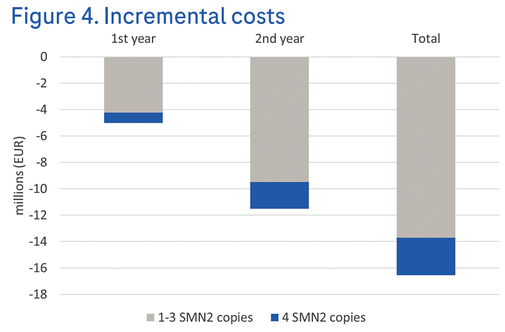


Figure 3. Method of estimating incremental cost



Market shares of individual drugs were determined separately in the newborn population starting SMA treatment in the 1st and 2nd year of the analysis. It was assumed that not all patients start therapy simultaneously. Qualification for treatment with individual therapies occurs smoothly throughout the time horizon according to the entry curve, which increases over time for risdiplam - hence the higher market shares observed in the 2nd year compared to the 1st year of the analysis in the new scenario.

Figure 4. Incremental costs



The results of the sensitivity/scenario analysis showed cost savings for all the variants tested, with results ranging from EUR 2.25M to EUR 7.76M in the 1st year and from EUR 6.09M to EUR 16.13M in the 2nd year of the time horizon with the total savings ranging from EUR 8.34M to EUR 23.89M (for alternative RIS market shares in the population starting treatment).

Conclusions

Starting treatment with risdiplam by 15.3% of the target population in the first year and 31.3% in the second year of the budget impact time horizon leads to total savings of approximately EUR 16.54M. The extension of risdiplam indication to newborn patients with 1-4 SMN2 copies results in cost savings to the Polish healthcare system, mainly due to reduced treatment acquisition costs.

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