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Cost-Effectiveness of Onasemnogene Abeparvovec in the Treatment of Presymptomatic Spinal Muscular Atrophy in Türkiye

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INTRODUCTION

Spinal Muscular Atrophy (SMA) is a rare, genetic, neuromuscular disease with an estimated prevalence of approximately 1–2 per 100,000 people[1]. It is one of the most common genetic causes of infant mortality and is associated with progressive, irreversible motor neuron loss that leads to muscle atrophy. The progressive muscle weakness and paralysis and impairment of swallowing and breathing, result in premature death in more severe forms of SMA. The disease is caused by a homozygous loss of function or absence of the Survival Motor Neuron gene 1 (*SMN1*), resulting in a lack of Survival Motor Neuron (*SMN*) protein[2].

SMA is more frequently seen in Türkiye compared to other countries due to the high frequency of consanguineous marriages. According to the Ministry of Health (MoH) figures, there are 3,000 patients with the disease[3]. Newborn screening enables diagnosis of SMA at the pre-symptomatic stage, providing an opportunity for early treatment intervention prior to the irreversible destruction of motor neurons[4]. SMA is included in both the pre-marriage screening program and newborn screening program of Türkiye.

Currently both nusinersen and risdiplam are reimbursed by the Social Security Institution (SSI) for treatment of SMA. Onasemnogene Abeparvovec IV is a gene therapy designed to introduce a functional copy of *SMN1* in the transduced cells to address the monogenic root cause of the disease.

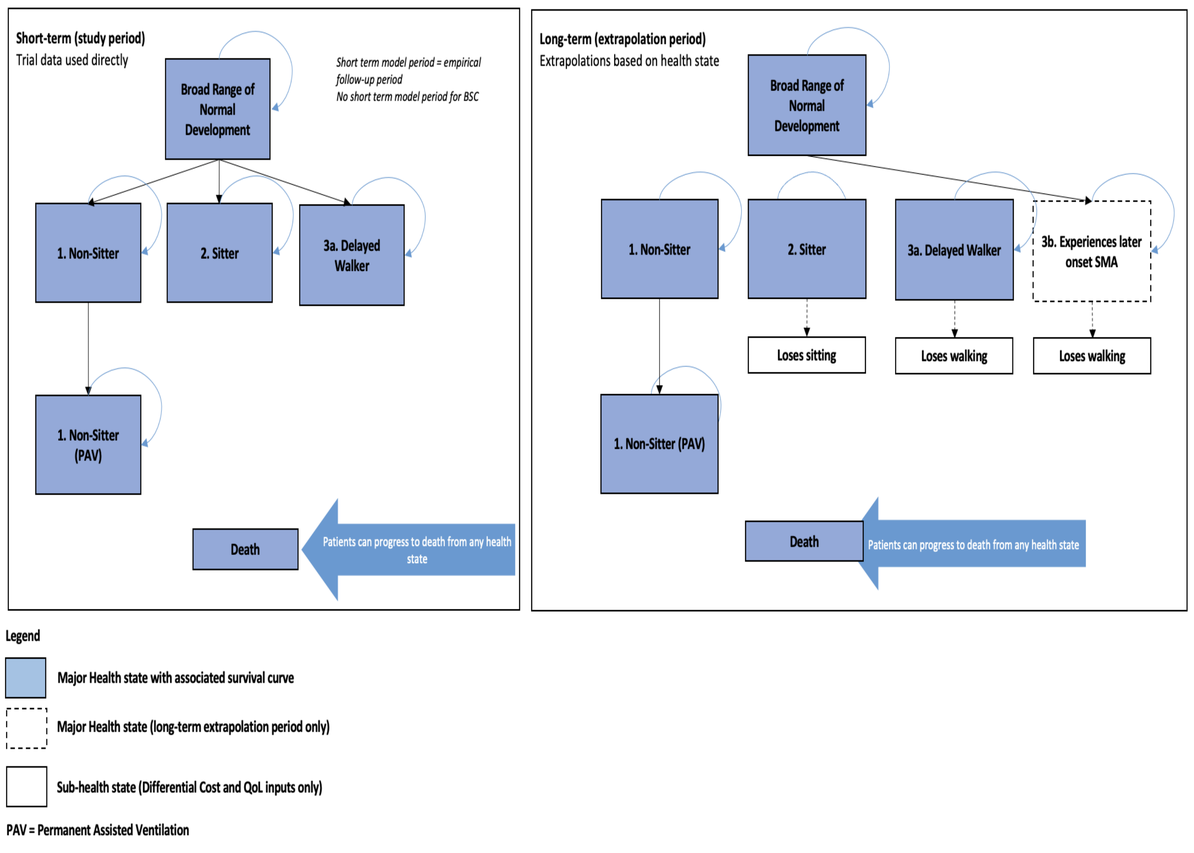
OBJECTIVES

The objective of this study is to estimate the cost-effectiveness of onasemnogene abeparvovec IV for the treatment of presymptomatic SMA compared to nusinersen and risdiplam in Türkiye.

METHODS

A Markov model was developed to estimate the lifetime health effects and costs of onasemnogene abeparvovec IV compared with nusinersen and risdiplam. (Figure 1). The patient group included in the cost-effectiveness analysis was a population of newborn infants with genetically confirmed, pre-symptomatic SMA with two or three copies of the *SMN2* gene who were aged ≤6 weeks (≤42 days) at the time of treatment. The health states in the model were broad ranges of normal development, delayed walker, experiences later onset SMA, sitter, non-sitter, non-sitter (permanent assisted ventilation), and death. The analysis was conducted from the Turkish payer perspective (SSI) with a lifetime horizon. Costs of treating SMA were calculated using the reimbursement prices of the SSI. All costs and outcomes were discounted at 3%.

Figure 1: Markov Model



The effectiveness data were obtained from the clinical studies of the three treatments (Table 1). The base case analysis used life years as the main health outcome in line with the guidelines of the SSI. QALY results were given as supplementary information. A health care resource utilization questionnaire was designed to estimate the costs of treating SMA Type 1,2 and 3 as these costs were used as proxies for the cost of different health states.

Table 1: Sources of Effectiveness Data

	Clinical Study	Source
Onasemnogene Abeparvovec IV	SPR1NT and LT-002	Novartis Data on file
Nusinersen	NURTURE	(Crawford et al, 2023; Finkel et ak 2020) [5,6]
Risdiplam	RAINBOWFISH	(Servais et al 2024)[7]

RESULTS

The costs of treating SMA Type 1, 2 and 3 were estimated as 90,369 TRY, 76,583 TRY and 12,798 TRY respectively. Tables 2 and 3 present the results of the analysis.

Table 2: Cost Effectiveness of Onasemnogene Abeparvovec IV Compared to Nusinersen

	Cost (TRY)	Life Years	QALYs	Incremental Cost (TRY)	Incremental Life Years	Incremental QALYs	ICER
OAV 101 IV	53,534,935	30.41	27.550	-66,091,051	0.03	0.421	Dominates
Nusinersen	119,625,986	30.38	27.130				

OAV 101 IV: Onasemnogene Abeparvovec IV

Table 3: Cost Effectiveness of Onasemnogene Abeparvovec IV Compared to Risdiplam

	Cost (TRY)	Life Years	QALYs	Incremental Cost (TRY)	Incremental Life Years	Incremental QALYs	ICER
OAV 101 IV	53,534,935	30.41	27.550	-115,853,651	0.46	3.935	Dominates
Risdiplam	169,388,586	29.95	23.615				

OAV 101 IV: Onasemnogene Abeparvovec IV

The cost-effectiveness analysis results for the combined cohort (2 and 3 gene-copied cohorts) comparing onasemnogene abeparvovec with nusinersen estimated that the lifetime cost of treating presymptomatic SMA was lower for onasemnogene abeparvovec IV (53,534,935 TRY vs 119,625,986 TRY) with higher life years (30.41 vs 30.38) and QALYs (27.55 vs 27.13). Similar results were found in comparison with risdiplam as well. The cost of treatment was lower for onasemnogene abeparvovec IV compared with risdiplam (53,534,935 TRY vs 169,388,586TRY) with higher life years (30.41 vs 29.95) and QALYs (27.55 vs 23.62). The results showed that onasemnogene abeparvovec IV has dominated both nusinersen and risdiplam. In the one way sensitivity analysis variables were changed by 20%. Both one-way and probabilistic sensitivity analysis results indicated that the findings were robust. Figures 2A and 2B present the cost effectiveness planes.

Figure 2A: Cost Effectiveness Plane: Onasemnogene Abeparvovec IV vs Nusinersen

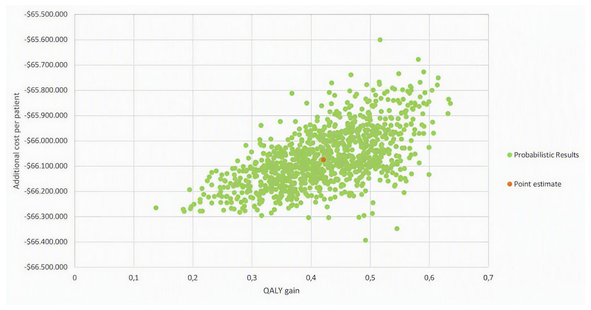
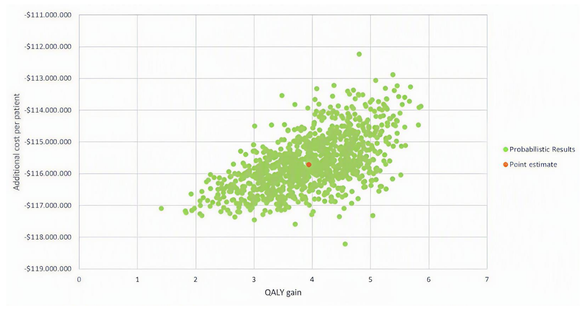


Figure 2B: Cost Effectiveness Plane: Onasemnogene Abeparvovec IV vs Risdiplam



CONCLUSION

Onasemnogene abeparvovec IV, in comparison to nusinersen and risdiplam, is a cost-effective option for the treatment of presymptomatic SMA in Türkiye.

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*This study was supported by Novartis Türkiye

Poster presented at ISPOR Europe 2026 Congress