Societal Costs of Spinal Muscular Atrophy Type 1 for Patients Treated with Onasemnogene Abeparvovec or Nusinersen in the United Kingdom

EE424

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Joris Kleintjens[1]; Anish Patel[2]; Marjolijn van Keep[3]; Krutika Srivastava[4]; Patrick McHale[5]; Sylvain Affinito[6]; Matthias Bischof[6]

[1]PRECISIONheor, London, UK; [2]Novartis Gene Therapies, Inc., Bannockburn, IL, USA; [3]Keep Consulting, Utrecht, The Netherlands; [4]PRECISIONheor, Boston, MA, USA; [5]Novartis Gene Therapies, Dublin, Ireland; [6]Novartis Gene Therapies GmbH, Rotkreuz, Switzerland

Introduction

- SMA is a rare genetic disease characterized by profound physical disability and muscular weakness due to degeneration of motor neurons[1]
- The prevalence of SMA type 1 is estimated to be approximately 1–2 per 100,000 people,[2] with approximately one new case diagnosed each year per 10,000 live births[3]
- SMA type 1 is the most severe and predominant form of SMA, comprising approximately 50–60% of cases, and has an early onset of symptoms during the first 6 months of life[3]
- If left untreated, this disease can lead to death or the need for permanent assisted ventilation before the age of 2 years[1]
- Neuron and motor function loss requires extensive supportive care that impacts family life significantly. Therefore, SMA type 1 is associated with substantial economic and health care responsibilities for patients, families, and caregivers.
- A recent real-world study estimated that 70% of the total costs for SMA in the United Kingdom were related to direct non–health care costs, which included transport and caregiver costs[4]
- In the United Kingdom, three disease-modifying treatments for SMA — onasemnogene abeparvovec (OA), nusinersen, and risdiplam — have received approval. These innovative therapies have led to rapid and significant improvements in motor function, enabling patients with SMA to gain or retain the ability to sit, stand, and walk.
- However, the societal costs associated with SMA and the effects of treatment on these expenses remain uncertain

Objective

- We sought to quantify the lifetime societal costs and life-years for patients with SMA type 1 who were treated with OA or nusinersen in the United Kingdom

Methods

- We conducted a targeted literature review to identify cost elements related to SMA type 1 from a societal perspective
  - In a cost-effectiveness analysis, the societal perspective should consider "everyone affected and counting all benefits and costs, regardless of who gains or loses." Hence, a societal perspective means that all resources — both health-related and not — associated with the intervention should be identified, measured, and valued.[5]
  - All elements we included in the costs of SMA from a societal perspective are:
    1) Direct health care costs (for the UK [NHS and PSS])
    2) Direct non–health care costs (e.g., expenses for paid caregiving, moving, home modification, purchasing or modifying motor vehicles, paid caregivers, and other miscellaneous items, including transportation)
    3) Indirect costs (e.g., family income loss and patient earnings)
- Following the identification of these cost elements, the lifetime costs of SMA type 1 were calculated using a published economic model[6]
  - The model used a Markov framework with patients transitioning between five health states (**Table 1**) and death from any health state
  - Clinical inputs of the model included mortality, need for permanent ventilatory support, and motor milestone achievement
  - The calculation of the societal costs was based on the disease trajectory of OA- or nusinersen-treated versus untreated patients

Table 1. SMA health states with corresponding annual health care costs[6]

Health states	SMA proxy applied	Annual direct health care costs
Within a broad range of normal development	—	£0
Walks independently	SMA type 3	£5,400
Sits independently	SMA type 2	£9,250
Cannot sit independently	SMA type 1	£25,918
Requires permanent assisted ventilation	SMA type 1	£25,918

SMA, spinal muscular atrophy.

Economic inputs

- The economic inputs were calculated on a per-patient basis and annualized in the model. The cost estimates were derived from published studies and NHS websites. Future costs and outcomes were discounted at 3.5% per year.
  - Direct health care costs
    - For each health state, health care resource use and costs included in the model were derived from the NHS website[6]
    - The costs included in the model were those associated with drugs, medical tests, visits, hospitalizations, physician visits, emergency visits, and public social services (**Table 1**)
  - Direct non–health care costs
    - The estimates for these costs were derived from a US-based survey.[7] The survey provided data on costs, classified by patients having either early onset SMA or late-onset SMA. For simplification, we applied the data for early onset SMA to the health state "Sitter," and for late-onset SMA to the state "Walker" in the model (**Figure 1**).
  - Indirect costs
    - **Lost family income:** The annualized loss of family income was estimated using published data categorized by level of care.[7] This study estimated the cost based on a regression analysis with type of disease and intensity of care as explaining variables while controlling for other variables (e.g., demographics) that may have contributed to family income loss. These estimates were then converted to UK costs using purchasing power parities (**Table 2**). Based on clinical expert opinion, the level of care was further categorized by health state and age band of the patient with SMA. These estimates were then used to determine lost family income.
    - **Patient income:** Average patient income per health state was estimated based on 1) the potential educational achievement for patients at working age, and 2) the income of the general population by education status. The data on educational achievement was derived from a caregiver survey for the health states "Walker" and "Sitter" and from national statistics for health state "Normal Development" (**Table 3**).[7,8] Unemployment rate for "Normal Development" was assumed to be equal to the general population; for "Sitters," it was assumed to be equal to the population of people with a disability, and for "Walkers," it was assumed to be the average between both.

Figure 1. Annual direct non-medical costs by health state

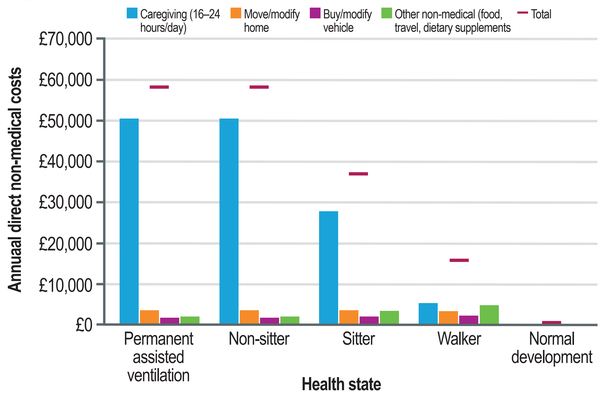


Table 2. Predicted annual lost family income[7]

Age band	Permanent assisted ventilation	Non-sitter	Sitter	Walker	Normal development
0–<24 months	£21,598 (16–24 hours/day)	£21,598 (16–24 hours/day)	£21,598 (16–24 hours/day)	£21,598 (16–24 hours/day)	£0 (no SMA-specific care needed)
24–<60 months					
5–17 years			£7,323 (8–15 hours/day)	£7,323 (8–15 hours/day)	
18+ years			£4,170 (1–8 hours/day)	£0 (no SMA-specific care needed)	

Note: Income loss is based on regression analysis, with the care intensity in hours per day as the explaining variable.

Table 3. General population income based on educational achievement

		Unemployment rate			Educational achievement by health state		
Educational level	Median annual earnings, £	Normal development, %	Walker, %	Sitter, %	Normal development, %	Walker, %	Sitter, %
Some high school	17,868	5.6	44.3	83.0	11.4	19.0	19.0
High school graduate	23,628	3.1	28.8	54.4	20.0	30.0	30.0
Some college/Associate's degree	29,469	3.1	28.8	54.4	28.6	28.0	28.0
College graduate	34,909	2.3	15.3	28.3	28.9	13.0	13.0
Post-graduate degree	40,527	2.3	15.3	28.3	7.3	6.0	6.0
Not available	—	—	—	—	3.7	4.0	4.0
			Weighted average income		**£28,427**	**£25,057**	**£19,141**

Results

Societal costs per life-year

- The model simulation for patients with SMA type 1 indicated that the discounted life-years for OA were greater than for nusinersen (12.8 years vs. 8.9 years, respectively) (**Table 4**)
- Consequently, the total discounted lifetime societal costs were greater for OA at £3,374,801 and lower for nusinersen at £3,306,714, but the difference was only £68,087
- When dividing the total societal costs over the number of life-years, the societal costs per year were £109,387 lower for OA than for nusinersen (£262,740 vs. £372,126, respectively; **Figure 2**)

Costs by category

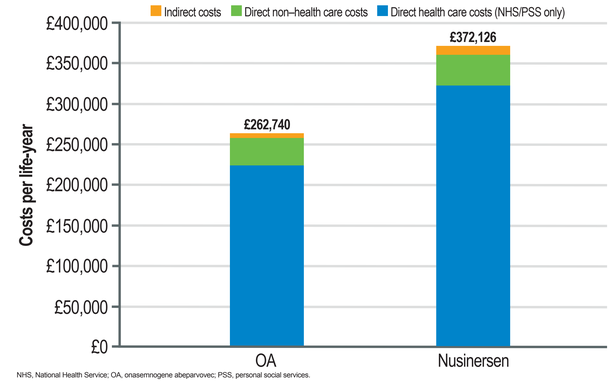
- Direct health care cost was the main cost category of the total lifetime societal costs, but comparable between treatments, with a difference of only £3,151
- Direct non–health care costs in both comparator groups contributed to 13% (£428,865) and 10% (£342,438) of the lifetime societal costs for OA and nusinersen, respectively
- Indirect costs
  - Family lost income over a lifetime was estimated at £116,582 for OA and £108,612 for nusinersen
  - Potential patient income gain was estimated at £41,846 for OA and £12,385 for nusinersen, which may offset a part of the family lost income

Table 4. Total discounted societal costs and life-years by treatment

	OA	Nusinersen
Societal costs	**£3,374,801**	**£3,306,714**
Direct health care costs (NHS/PSS only)	£2,871,199	£2,868,049
Direct non–health care costs	£428,865	£342,438
Indirect costs	£74,736	£96,227
Family lost income	£116,582	£108,612
Patient potential income gain	–£41,846	–£12,385
Life-years	12.8	8.9
Societal costs per life-year	**£262,740**	**£372,126**
Non–health care costs per life-year	£39,207	£49,366

NHS, National Health Service; OA, onasemnogene abeparvovec; PSS, personal social services.

Figure 2. Societal costs per life-year



NHS, National Health Service; OA, onasemnogene abeparvovec; PSS, personal social services.

Conclusions

- Direct and indirect costs associated with SMA type 1 impose a substantial financial impact on patients, families, and society
- We determined that patients treated with OA have a substantially greater life expectancy than those receiving treatment with nusinersen
- As a result, the lifetime cost for an OA-treated patient with SMA type 1 is slightly greater
- However, the total cost per life-year from a societal perspective is much lower for an OA-treated patient than for a nusinersen-treated patient

References

1. Kolb SJ, Kissel JT. *Neurol Clin*. 2015;33:831–46.
2. Sugarman EA, et al. *Eur J Hum Genet*. 2012;20:27–32.
3. Verhaart IEC, et al. *Orphanet J Rare Dis*. 2017;12:124.
4. Peña-Longobardo LM, et al. *Int J Environ Res Public Health*. 2020;17:5640.
5. Kim DD, et al. *Pharmacoeconomics*. 2020;38:1135–45.
6. Dean R, et al. *J Mark Access Health Policy*. 2021;9:1889841.
7. Muscular Dystrophy Association (MDA). Cost of amyotrophic lateral sclerosis, muscular dystrophy, and spinal muscular atrophy in the United States. Report, The Lewin Group, 2012.
8. Osmanovic A, et al. *Brain Sci*. 2020;11:8.

Abbreviations

NHS, National Health Service; OA, onasemnogene abeparvovec; PPP, purchasing power parities; PSS, personal social services; SMA, spinal muscular atrophy.

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