The most expensive treatment in the world

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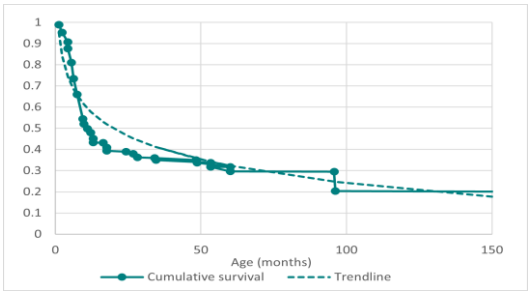
A treatment for **pre-symptomatic spinal muscular atrophy type 1 (SMA1)**, onasemnogene abeparvovec (OA), has been approved by NICE. At a cost of **£1.79M per treatment**, it is probably the **most expensive treatment yet**.[1]

SMA1 is a genetic disorder with an average survival of 2 years **AND…**

Cumulative survival for SMA1 [3]



Reece, father of Arthur an infant affected by SMA1 [2]

"When we found out that Arthur would get the treatment, and **be the first patient**, I just broke down."

The alternative treatment for SMA1 is **nusinersen** which halts disease progression but has to be **injected into the spine every 4 months** for the rest of a person's life . Each dose costs £75,000. Given the high cost why would these treatments be approved for use when others that are a fraction of the cost are not?

Systematic literature review of the economic burden of spinal muscular atrophy and economic evaluations of treatments [4]

Objective

- To summarise the studies assessing the costs of SMA and economic evaluations of treatments including nusinersen.

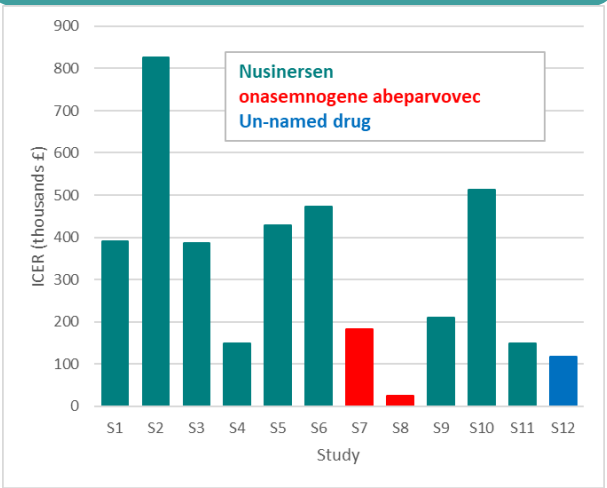
Methods

- Two systematic literature reviews were performed, following PRISMA guidelines – one for **cost studies** of SMA and one for **economic evaluations** of treatments for SMA.
- The databases searched were Medline (PubMed) and Scopus (Elsevier).

Results

- ✓ **Nine** cost studies were identified.
- ✓ Estimates of the **annual costs of SMA1** ranged from £54,034 to £141,428.
- ✓ The **cost per quality adjusted life-year gained** was £151,261 to £828,327 for **nusinersen** and £23,374 to £181,010 for **onasemnogene abeparvovec**.

Incremental cost-effectiveness ratio (ICER) or cost per quality-adjusted life-year (QALY) from each study. [4]



Our thoughts:

- NICE uses a threshold of £100,000 per QALY gained for orphan drugs rather than the £20,000 for standard drugs.
- The NHS has secured a 'substantial and confidential discount' that is 'fair to taxpayers'.[1,2]
- Initial evidence suggests affected babies meet normal milestones up to the age of 5 years, but we do not yet know what the durability of the effect of treatment is.
- Assuming lifelong cure, a crude estimate of the ICER is well below the £100K threshold.

Cost of treatment	£1,795,000.00
Life expectancy at birth	79.4 years
Median survival	2 years
QALY per year of life	0.905
ICER	£25,625.65

References

1. https://www.england.nhs.uk/2021/03/nhs-england-strikes-deal-on-life-saving-gene-therapy-drug-that-can-help-babies-with-rare-genetic-disease-move-and-walk/
2. https://www.england.nhs.uk/2021/06/nhs-treats-first-patient-with-the-worlds-most-expensive-drug/
3. Ge, X. et al. (2012) 'The natural history of infant spinal muscular atrophy in China: A study of 237 patients', Journal of Child Neurology, 27(4), pp. 471–477.
4. Dangouloff, T. *et al.* (2021) 'Systematic literature review of the economic burden of spinal muscular atrophy and economic evaluations of treatments', *Orphanet Journal of Rare Diseases*. BioMed Central, 16(1), pp. 1–16