Long-term durability in cost-effectiveness models of gene therapies:

Review of Health Technology Assessment cases

Connor Buffel[a,b], Inês Oliveira[a,b] , Geert Vander Stichele[a,b]

a. Integrated Strategic Market Access Services (ISMS), Zoersel, Belgium

b. GenBytes, Zoersel, Belgium

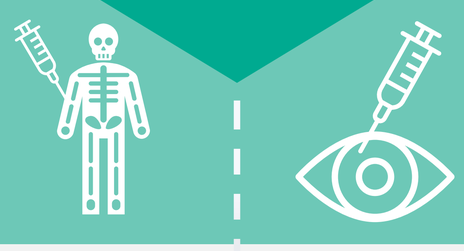
Several *in vivo* gene therapies have gained market access in the US and Europe. These gene therapies are designed to have a long-term effect after a single treatment. Several Health Technology Assessment (HTA) bodies have analyzed the cost-effectiveness of gene therapies over the long-term, despite the limited availability of clinical data.

In cost-effectiveness models of *in vivo* gene therapies reviewed by HTA bodies, one of the key value drivers is the durability of the treatment effect over the long-term.

How do HTA bodies approach the translation of short-term clinical trial evidence into long-term treatment effects of *in vivo* gene therapies?

Zolgensma®
onasemnogene abeparvovec
Indication: spinal muscular atrophy

Luxturna®
voretigene neparvovec
Indication: inherited retinal disorder



* Until end of model horizon.
** The company has a commercial arrangement. This makes the treatment available to the NHS with a discount. The size of the discount is commercial in confidence.
Pre-clinical rationale used to justify long-term effect.
Company submission.
HTA acceptance.

The acceptance of long-term treatment durability differed materially between HTAs in each case. However, at minimum, a 10-year treatment effect was accepted based on 2-year trial data, 5 years of follow-up data, and pre-clinical rationale.

This is critical for designing clinical trials and has relevant implications for health economic modeling and pricing strategy of similar gene therapies.

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