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Icer sets Novartis a gene therapy pricing challenge

Madeleine Armstrong

Novartis’s Zolgensma comes out on top versus Biogen’s Spinraza, but neither is deemed cost effective by Icer.

An eagerly awaited Icer report on Novartis’s spinal muscular atrophy gene therapy, Zolgensma, contained one surprise. The US pricing watchdog concluded that the project, despite its expected record-breaking price tag, would likely be more cost-effective than Biogen’s established SMA therapy, the antisense drug Spinraza.

The main outcome of the report was less surprising: Icer deemed, as it has with many other new drugs, that neither Zolgensma nor Spinraza was good value for money. Still, the $2m price that Icer has assumed for Zolgensma is not too far from meeting a key cost-effectiveness threshold, leaving the door wide open for negotiations between Novartis and payers.

Icer said the project would need to come in at around $1.6m to hit a standard cost-effectiveness threshold of $150,000 per quality-adjusted life year (QALY). The apparently surmountable gap between this and Icer's assumed $2m placeholder price tag could bode well for Zolgensma’s chances of getting reimbursed – the project is due an approval decision from the US FDA in May.

This is all hypothetical, with Novartis yet to announce the price for Zolgensma. The company has already indicated that the cost could exceed other drugs and even gene therapies, saying in November that $4-5m per treatment would be cost effective. However, it almost immediately backed away from this figure in what might have been an opening gambit designed to make Zolgensma’s eventual price seem more palatable.

Novartis does not seem to have ruled out pricing the therapy at the upper end of this band. A spokesperson told *Vantage* that Icer should use a cost-effectiveness threshold of $500,000 per QALY for Zolgensma, saying this was more appropriate for an ultra-rare disease. Icer concluded that even at a cost of $5.4m the therapy would meet this target.

Zolgensma vs Spinraza in SMA type 1 patients: Icer analysis					
	Drug cost ($'000)	**Non-treatment healthcare costs ($'000)****	**Total cost ($'000)****	**QALYs****	**Cost/QALY gained ($'000)**
Zolgensma	2,000*	684	2,684	10.87	247
Spinraza	1,738	541	2,279	1.43	1,595
**Placeholder price; **Adjusted vs standard of care. Source: Icer report, December 20, 2018.*					

In type 1 SMA, the most severe subtype and the only one in which Zolgensma has been studied, Icer calculated that at $2m per patient Zolgensma would cost $247,000 per quality-adjusted life year (QALY) gained over best supportive care.

The gene therapy came out looking much better than Spinraza, which costs nearly $1.6m per QALY gained in type 1 disease, Icer said. This was mainly down to better outcomes with Zolgensma, which added 11.3 QALYs versus 1.9 for Spinraza and 0.46 for best supportive care.

Biogen's product is approved across SMA subtypes, and Icer was more positive about Spinraza in presymptomatic patients, where the watchdog concluded that it cost $728,000 per QALY gained. Presumably both therapies will still have a place in the market, with Zolgensma being reserved for the most severely affected patients, but the gene therapy looks likely to cut into Spinraza sales in this subtype.

There are still unanswered questions about Zolgensma, including long-term safety and duration of therapy. Indeed, Icer raised the issue of whether its effect might wane over time and, if so, whether patients would then receive Spinraza. As well as raising safety concerns, this could be an expensive strategy.

EvaluatePharma sellside consensus forecasts Zolgensma revenues of $1.6bn by 2024. Novartis will need to hit this target to justify the $8.7bn it spent on the gene therapy's originator, Avexis, and Icer's analysis is a good sign also for others hoping to emulate a premium pricing strategy for gene therapies – providing that they can show a similarly strong benefit.

This story has been updated to include comments from Novartis.