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# At Over \$2 Million Zolgensma Is The World's Most Expensive Therapy, Yet Relatively Cost-Effective



**Joshua Cohen** Contributor ⓘ

Healthcare

*I write about prescription drug value, market access, healthcare systems, and ethics of distribution of healthcare resources*



FILE - This Aug. 13, 2016, file photo shows a logo of Swiss pharmaceutical company Novartis in... [+] ASSOCIATED PRESS

Last month, the Food and Drug Administration (FDA) approved a gene therapy for a rare childhood disorder; spinal muscular atrophy (SMA). The name of the therapy is Zolgensma. It is indicated for the treatment of pediatric patients less than two years of age with spinal muscular atrophy, and bi-allelic mutations in the survival motor neuron 1 gene. At approximately \$2.1 million per patient it is the **most expensive treatment** on

the market. Zolgensma can be a one-time, life-saving treatment that allows for children with SMA to function in ways unimaginable just a few years ago.

BETA

The therapy's sponsor, Novartis, set the price at around \$2.1 million, but has offered insurers the possibility of paying in annual installments of \$425,000 for five years.

As with any new treatment, there is invariably uncertainty about its real-world effectiveness, safety, and durability. This is especially true for orphan treatments, such as Zolgensma, which during the different stages of drug development often only include relatively small numbers of patients. Hence, there's a need for post-marketing evidence generation.

Absent post-marketing data at the present time, analysts from the Institute for Clinical and Economic Review (ICER) evaluated Phase III evidence to calculate the cost-effectiveness of Zolgensma. A value-based price for Zolgensma was estimated at between \$1.1 million to \$1.9 million per treatment, given a cost-effectiveness threshold of \$100,000 to \$150,000 per quality-adjusted life year gained. And, a [value-based price](#) for Zolgensma was estimated at between \$1.2 million to \$2.1 million, given an alternative threshold of \$100,000 to \$150,000 per life year gained.

Evidently, the price chosen by Novartis reflects the upper bound in ICER's calculations of what constitutes a cost-effective price.

Too often the discussion of a [drug's price](#) is fixated on eye-popping numbers and not value. It's laudable that ICER redirected us to the discussion of value. In some instances, high prices may be justified by value; in others, not. At the same time, low-priced products may sometimes produce great value, and in other instances no value at all.

The key thing to remember is that the value of a new technology relates to its opportunity cost, or what could have been purchased with the resources

used for a particular technology. In this instance, spending money on Zolgensma implies the need to assess the value of the next best alternative; in this case, [Spinraza](#). As Spinraza is not a one-off treatment it must be administered on a regular basis. It also doesn't restore functioning to the same degree as Zolgensma. Over a 10-year period Spinraza's costs would total over \$4 million.

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It's important to note that assessing differences between Zolgensma and Spinraza is not necessarily a straightforward apples to apples comparison. This is because Spinraza was approved by the FDA in December 2016 to treat all types of spinal muscular atrophy regardless of a patient's age. Therefore, for many with SMA Zolgensma will not be an option and Spinraza will be the best alternative available.

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**Joshua Cohen**

I'm an independent healthcare analyst with over 22 years of experience analyzing healthcare and pharmaceuticals. Specifically, I analyze the value (costs and benefits) of... **Read More**

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