

## Curing at A Cost: The Zolgensma Revolution

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Few discoveries and research in medical innovation have had the transformative potential of gene therapy. Amongst the most significant recent developments is Zolgensma, a pioneering spinal Muscular Atrophy (SMA) treatment. This infrequent but devastating genetic disorder disturbs the motor nerve cells in the spinal cord and is characterized by progressive muscle weakness and paralysis.

SMA has long been a source of despair for families worldwide; it claims the lives of many children before their second birthday. However, the advent of Zolgensma, approved by the FDA in 2019, represents a beacon of hope for families grappling with this disease, often seen as a death sentence for infants.

SMA affects approximately 1 in 6000 births and has no ethnic or geographic boundaries. It is caused by a mutation in the Survival Motor Neuron 1 (SMN 1) gene, leading to a deficiency of the SMN protein. This eventually leads to the loss of motor neurons, resulting in severe muscle weakness, impaired movement, and early death in its most severe form. Before Zolgensma, treatment options were limited and focused mainly on managing symptoms rather than addressing the underlying genetic cause.

### *Zolgensma: a game-changer*

Zolgensma changed this paradigm by directly delivering a functional copy of the SMN1 gene into the patient's cells. The vector that provides the SMN1 gene is made from a virus called AAV9. It is administered as a one-time intravenous infusion. This therapy works at the genetic level, halting the progression of the disease and, in many cases, reversing some of its effects. After a single infusion, Zolgensma replaces the function of the SMN1 gene to help the body produce SMN protein.

### *Clinical trials demonstrate remarkable efficacy with*

- Improvement in motor function.
- Increased survival.
- Reduced need for respiratory support.
- Improved quality of life.
- Potential for lifelong benefits.
- Minimally invasive.

However, while Zolgensma offers remarkable promise, its journey into the hands of patients has not been without challenges. The Zolgensma injection price in India is INR 17 crore (\$2.1 million) per dose, making it one of the most expensive drugs in the world. However, the high cost of Zolgensma has generated controversy and raised questions about its accessibility and affordability, especially in countries like India, where healthcare costs are a significant barrier to treatment. The high cost of Zolgensma has sparked heated debate about the pricing of life-saving treatments, with many questioning why a single treatment can cost so much. Here are some of the reasons.

- Research and development costs are high.
- Small market size.
- One-time treatment.
- Manufacturing costs are high.
- Health outcomes.
- Insurance coverage.

### Conclusion

In conclusion, Zolgensma is a highly effective gene therapy with a high success rate in treating Spinal Muscular Atrophy (SMA), a rare and often fatal genetic disease. However, the high cost of the treatment can be a significant barrier to access, especially in countries like India, where healthcare costs are generally lower.

However, several options for patients and their families are available to raise funds for Zolgensma treatment, including crowdfunding. By creating a compelling campaign and reaching a wider audience, patients and their families can increase their chances of raising the necessary funds to access this life-saving treatment.

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