Berkley Insights

Gene Therapy: Zolgensma

What is Gene Therapy?

DNA is the blueprint, or genetic code, that lives inside each cell. Occasionally one of these genes mutates and malfunctions, leading to a genetic condition or disorder. Gene therapy is a treatment designed to fix the genetic code by introducing a functioning copy of the misfiring gene.[1] A healthy copy of the gene is introduced into the patient's body and alters the faulty DNA, restoring its proper function. [Watch FDA Video: How Gene Therapy Works]

Intensive research and development have led to remarkable advances in the field of gene therapy. Some gene therapies have the potential to revolutionize medical treatment for cancer, muscular dystrophy, hemophilia, lymphoma, and other life-threatening conditions. They have the possibility to cure diseases once thought to be incurable, but these benefits may come with an extremely high price tag.

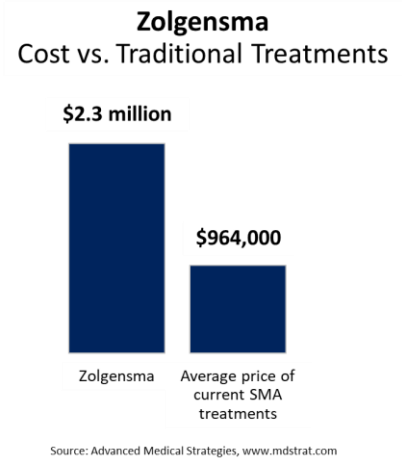
Spotlight on Zolgensma

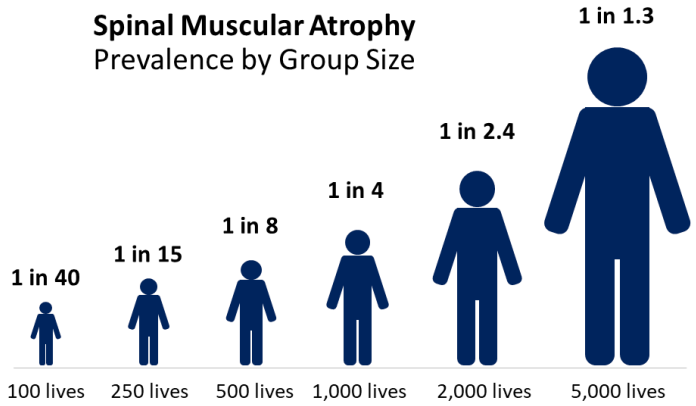
A recent example is Zolgensma, a gene therapy drug approved in 2019 to treat Spinal Muscular Atrophy (SMA) in patients under 2 years old. A single intravenous infusion costs $2.3 million, which is significantly higher than traditional SMA treatments.[2]
SMA is a rare disorder that:

- Occurs in 1 in 6,000-10,000 births worldwide[3]
- Affects an estimated 25,000 Americans, and 6 million others carry the gene

Risk for Self-Funded Groups

For self-funded groups, the likelihood of having a Zolgensma claim increases as the group size grows. For groups of 5,000, there is a 1 in 10 chance of having a covered member with SMA qualify to receive Zolgensma. Even smaller groups aren't immune to the very real risk of having a Zolgensma claim.

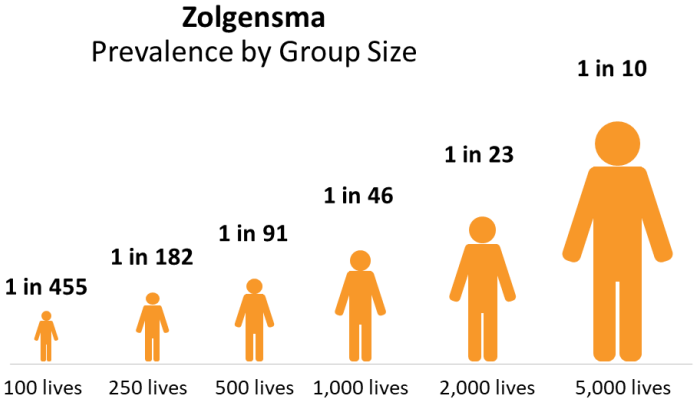




Likelihood of having a covered member diagnosed with SMA

Assumes each enrolled employee represents 2.2 members and the birth rate per employee is consistent with the national average. Likelihood is defined as the percentage chance in any calendar year.

Source: Cleveland Clinic, https://my.clevelandclinic.org/health/diseases/14505-spinal-muscular-atrophy-sma



Likelihood of having a covered member eligible for Zolgensma

Assumes each enrolled employee represents 2.2 members and the birth rate per employee is consistent with the national average. Likelihood is defined as the percentage chance in any calendar year.

Source: Proprietary industry databased accessed by Berkley Accident and Health, 2020

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Recommendations for Self-Funded Groups

Gene therapies like Zolgensma and their associated high price tags are poised to change the health care landscape in this new decade. In early 2020, the FDA introduced a number of policies that will allow it to fast-track gene therapy approvals. By 2025, the agency expects it will be reviewing and approving between 10 to 20 gene and cell therapies each year.[5]

In an apparent setback, the FDA delayed a decision in August 2020 on Roctavian, a potential treatment for adults with Hemophilia A. With an estimated price tag of $2 million to $3 million, Roctavian will be the most expensive drug in the world, if approved.[6] Unlike traditional drugs, gene therapies are designed to be one-time fixes for genetic defects, so the FDA has requested more long-term data on the durability of Roctavian's benefits, postponing approval for at least a year. Despite the delay with Roctavian, the FDA has signaled its strong support of gene therapy products and continues to partner with manufacturers to move therapies quickly through the approval process.

This trend may present challenges to self-funded employers, including the potential for increases to their Stop Loss premiums and/or greater frequency of higher individual deductibles ("lasering"). Due to these challenges, as well as the possibility of an unaffordable high-dollar claim, partnering with their Stop Loss carrier will be more important than ever to safeguard their plan's financial health.

Self-funded groups may also want to consider the following:

- Understand your group's risk and possible conditions that could be treated with gene therapy
- Inquire with your third party administrator to better understand their approach to managing potential gene therapy claims
- Explore billing and pricing negotiation strategies with the administrator and Stop Loss carrier
- Examine their plan document and directly address the use of gene therapy, including limitations and eligibility. One option some employers are considering is to exclude new drugs for the first two years following FDA approval to ensure proper efficacy.
- Identify potential gene therapy claims early, through pre-certification notices and claims for genetic testing

Berkley Accident and Health is closely monitoring the gene therapy market and has a demonstrated track record of helping lower catastrophic claim costs on behalf of our clients. We're actively developing solutions to support our policyholders, including a relationship with Emerging Therapy Solutions (ETS) for cell and gene therapy pricing solutions and data analytics. With our expertise and financial strength, clients can have confidence in Berkley Accident and Health.

For more information, contact your Berkley Accident and Health representative.

Prevalence of SMA and Zolgensma assumes each enrolled employee represents 2.2 members and the birth rate per employee is consistent with the national average. Likelihood is defined as the percentage change in any calendar year.

This content is for general informational purposes only; it is not legal advice or a legal opinion. You should seek the advice of legal and tax counsel before acting upon any of this information. In addition, you should perform your own due diligence on any potential vendor or solution.

[1]U.S. National Library of Medicine, *How does Gene Therapy Work*, https://ghr.nlm.nih.gov/primer/therapy/procedures

[2]Advanced Medical Strategies, https://www.mdstrat.com

[3]Cleveland Clinic, Spinal Muscular Atrophy, https://my.clevelandclinic.org/health/diseases/14505-spinal-muscular-atrophy-sma

[4]SMA Foundation, SMA Overview, https://smafoundation.org/about-sma/

[5]U.S. Food & Drug Administration, *FDA Continues Strong Support of Innovation in Development of Gene Therapy Products*, https://www.fda.gov/news-events/press-announcements/fda-continues-strong-support-innovation-development-gene-therapy-products

[6]Wall Street Journal, *BioMarin Explores Pricing Experimental Gene Therapy at $2 Million to $3 Million*, https://www.wsj.com/articles/biomarin-explores-pricing-experimental-gene-therapy-at-2-million-to-3-million-11579190318

All information accessed August 2020.

