AMWINS®
GROUP BENEFITS

Gene Therapy Solutions: Medical Breakthroughs Shouldn't have to Break the Bank

The cost-to-benefit analysis of gene therapy coverage is undoubtedly complex. As more treatments are approved, benefits brokers and employer groups are asking questions about these programs. Staying informed about the state of the industry—and tapping into a trusted partner that is well-versed in this evolving field—is the best way to navigate coverage for these promising new treatments.

CONTACT

To learn more about how Amwins can help you place coverage for your clients, reach out to your local Amwins broker.

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Introduction: What is Gene Therapy?

Understanding gene (and cell) therapy begins with appreciating the complex functionality of a single human cell. Trillions of cells receive information from genes and work together to form the tissues and organs driving our bodies to function. In some cases, abnormalities like defective, mutated or missing genes can lead to severe disorders and debilitating diseases. Common genetic disorders include hemophilia, cystic fibrosis, sickle cell anemia, spinal muscular atrophy and hereditary blindness.

As scientific and technological advancements continue to evolve, new and alternative treatment methods have emerged. These treatments are targeting, modifying, and even replacing genes for individuals with specific genetic conditions. Commonly known as gene therapy, these treatments are often curative and provide patients with improved quality and longevity of life. Coverage for these life-changing treatments remains a challenge, leaving employers to choose between excluding these classes of treatment from their benefit packages or risking going bankrupt.

Gene Therapy Solutions with Cost-Containment in Mind

Your benefits strategy relies on cost-containment solutions to provide financial protection. Still, the importance of supporting your employees and their families with today's health and wellness complexities cannot be understated. Amwins Gene Therapy Solutions supplements your current strategy and allows you to face unexpected costs with confidence.

Gene and cell therapies in the market today range from $850,000 to $4,250,000. Experts anticipate that an additional seven to ten therapies will be approved and market-ready by the end of 2024. Costs of these groundbreaking therapies are expected to continue to rise.

Our benefit solutions are evolving with medical advancements. We will continue to conduct cost-benefit analysis to determine if and when adding additional therapies adds value to your health plan.

"Developments in gene and cell therapies have the potential to provide life-changing treatments but also present significant financial risk to self-funded plan sponsors. Our mission is to support our broker partners by providing meaningful solutions that protect employers as they face the ever-evolving and complex gene therapy landscape."

– Josh McGee, Executive Vice President, Amwins Accident & Health Underwriters

There are 12 gene therapies approved and available in the market today. Amwins Gene Therapy Solutions addresses the disease state to provide portable, first-dollar protection for three Food and Drug Administration (FDA) approved treatments.

1. **Leber Congenital Amaurosis (LCA)**, referred to as hereditary or inherited blindness, impacts one in 80,000 people. It is a progressive disease, and a person can have impaired vision at an early age and later be classified as completely blind. LCA is triggered by a single gene mutation inhibiting the retina's functionality. Impressively, a one-time treatment with Luxturna can repair this mutation and improve vision. Gene Therapy Solutions covers Luxturna treatment for hereditary blindness up to $850,000.

2. **Spinal Muscular Atrophy (SMA)** is a neuromuscular disease affecting one in 11,000 U.S.-born children. Nearly one in 40 people (6.6 million Americans) may carry the disease. SMA impacts the skeletal and muscular system, driving loss of voluntary muscle movement. Persons with SMA are often unable to perform routine activities of daily living and have a shortened lifespan. There are five types of SMA. Gene Therapy Solutions provides coverage for types 1 and 2. Type 1 is the most common, accounting for 60% of diagnoses in children. Symptoms often develop before six months of age. SMA is serious and life-threatening. However, early treatment slows progression, helps individuals reach milestones and gives hope for greater independence and a more positive prognosis. Type 2 accounts for 27% of cases, and symptoms begin to show within six to eighteen months. Untreated individuals will eventually be unable to walk without assistance. Gene Therapy Solutions covers Spinraza and Zolgensma treatment for SMA Types 1 and 2 up to $2,200,000. Zolgensma is curative in nature and typically used to treat type 1. Spinraza prevents further progression of the disease for individuals with SMA.

Note: Clinical evidence for gene therapy for SMA types 3, 4, and 5 has limited data on clinical efficacy. These types could be covered in the future if more robust clinical data demonstrate appropriate clinical outcomes.

Gene Therapy Solutions with Cost-Containment in Mind (cont.)

3. **Transfusion Dependent Beta Thalassemia (TDT)** is a rare blood disorder that results from defective or missing genes that impact the production of hemoglobin. Hemoglobin is a protein in the blood that allows red blood cells to carry oxygen throughout the body. TDT is severe and requires patients to be dependent on blood transfusions for survival. It's estimated that there are 80-90 million TBT carriers (1.5% of the global population) and that there 60,000 symptomatic individuals born annually[1,2]. Patients living with TDT often have long-term care needs and face significant health complications. While regular infusions may temporarily improve the condition, it will not deliver the gene that would enable the body to produce adequate hemoglobin on its own. Zynteglo is the first and only gene therapy treatment for TDT and offers new hope for patients dependent on blood transfusions to treat their beta thalassemia. Gene Therapy Solutions covers Zynteglo treatment for Transfusion Dependent Beta Thalassemia up to $2,800,000 through GTS-5. The treatment leverages the patients own blood cells to create the hematopoietic stem cell-based treatment. In clinical trials, 89% of TDT participants were able to stop receiving blood transfusions and of those individuals, 100% were able to maintain transfusion independence for an extended duration[3].

4. **Cerebral Adrenoleukodystrophy (CALD)** is a rare genetic disorder caused by a mutation in the ABCD1 gene. This mutation leads to a dangerous buildup of a very long chain of fatty acids causing significant damage to the brain. Adrenoleukodystrophy is estimated to affect approximately 1 in 20,000 to 1 in 30,000 newborn males. CALD is the most severe and neurodegenerative form of this condition and affects about 40% of diagnosed males. Damage caused by CALD may lead to seizures, difficulty walking, intellectual disabilities, and difficulty performing activities of daily living[4]. Without treatment, 50% of CALD patients die within 5 years of their first symptoms. While there is no known cure for CALD, Skysona offers the opportunity for disease stabilization for boys ages 4-17 with early, active CALD. Skysona was clinically shown to prevent further damage to the nerves and death in clinical trial patients[5]. Gene therapy Solutions covers Skysona treatment for Celebral Adrenoleukodystrophy up to $3,000,000 through GTS-5.

While covering the cost of cell and gene therapy treatments is expensive, the costs of not treating the patient are financially, physically and emotionally significant. Patients left untreated will, in many cases, develop co-morbidities that will lead to increased medical expenditures and decreased lifespan. One must also consider costs for:

- medical equipment
- living-space modifications
- caregiver expenses
- extensive time off work
- lost productivity for parents
- long-term disability for the patient

1. Co-morbidities and mortality associated with transfusion-dependent beta-thalassaemia in patients in England: a 10-year retrospective cohort analysis - PubMed (nih.gov)
2. Increasing prevalence of thalassemia in America: Implications for primary care - PubMed (nih.gov)
3. Clinical Trial Results | ZYNTEGLO® (betibeglogene autotemcel)
4. Cerebral Adrenoleukodystrophy - Child Neurology Foundation
5. Summary Basis for Regulatory Action - SKYSONA (fda.gov)

Program Protection

Amwins Gene Therapy Solutions includes five available treatments. New treatments were recently released to the market and are currently being evaluated for our program.

Here is how Amwins Gene Therapy Solutions works:

Amwins Gene Therapy Solutions (GTS) offers reimbursement to group health plans and their coordinating stop loss carrier for qualified claims. Qualifying reimbursements extend from the first dollar of group health plan expense up to specified limits for each covered therapy.

Our program distributes the reimbursement according to the stop loss deductible. Following the adjudication of a qualified claim, our program reimburses the group health plan dollar one of their expenses up to their specific deductible and the balance of the reimbursement is distributed to the associated stop loss carrier.

If a group health plan chooses to switch carriers, administrative and/or stop loss, this program is transferrable. The group health plan does not run the risk of losing the program.

We have two available options:

Program details*:

GTS-3

Covered Pharmaceuticals	Treated Diseases	Maximum Payable Per Covered Person Per Benefit Period	Cost of Program
Luxturna	Leber Congenital Amaurosis (LCA)	$850,000	Please contact your broker for pricing information.
Zolgensma	Spinal Muscular Atrophy (SMA) Type I and Type II	$2,200,000	
Spinraza**			

GTS-5

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Zolgensma	Spinal Muscular Atrophy (SMA) Type I and Type II	$2,200,000	
Spinraza**			
Zynteglo	Transfusion Dependent Beta Thalassemia (TDT)	$2,800,000	
Skysona	Cerebral Adrenoleukodystrophy (CALD)	$3,000,000	

*Program disbursements are subject to coverage terms and exclusions.
**Amwins Gene Therapy Solutions addresses Spinal Muscular Atrophy as a disease state. While Spinraza is not classified as a gene therapy treatment, it is a Chronic Specialty Therapy for patients with SMA. Spinraza is costly, with the initial treatment expense of ~$750,000 and an additional ~$375,000 expected annually. We include Spinraza in our program to help reduce this financial burden.

Program Eligibility

The treatment for SMA included in this program are intended for children up to 24 months of age. There is no age limit for Luxturna,Skysona and Zynteglo. Program eligibility is based on a participant meeting the qualifications outlined in the table below.

	Luxturna / Skysona / Zynteglo	Zolgensma	Spinraza
Run-In Period	Not Applicable	Covered Person must be born and not have an existing diagnosis for covered diseases within 90 days from the Agreement Year inception date or from the enrollment start date of the covered person into reinsured' s plan within an Agreement Year.	
Agreement Year	Covered Person must be diagnosed by the last day of this 12-month period.	Covered Person must be born by the last day of this 12-month period.	
Treatment Period	Covered person must have the covered pharmaceutical initially administered between the first day of the agreement year and 24 months from that date.	Covered person must be diagnosed and the covered pharmaceutical initially administered between the first day of the agreement year and 24 months from that date.	Covered person must be diagnosed and the covered pharmaceutical initially administered between the first day of the agreement year and 36 months from that date.
Claims Period	Claims for Covered Pharmaceuticals must be filed and paid within 36 Months from the first day of the Agreement Year and are valid for Covered Pharmaceuticals administered during the Treatment Period.		

Program Advantages

As your trusted partner for cost-containment solutions, we continue to evaluate market needs. Our goal is to identify the most advantageous solutions so administrators can maximize their budget and mitigate unpredictable risk while supporting their workforce through innovative, responsive and robust offerings.

Meaningful Protection

First-dollar protection is given to the plan sponsor

Industry Experts

Our experts monitor the landscape of FDA approvals and make purposeful additions to our program that bring value and positive results to clients

Portability

Change carriers without disrupting coverage terms

Simplified Billing & Administration

Administrative burden is reduced by including the program fee in the stop-loss bill

*Program disbursements are subject to coverage terms and exclusions.

Next Steps: Find a partner who aligns with your strategy

Offering comprehensive benefits while also being economically efficient is a balancing act. Employers are tasked with designing a benefit strategy around the needs of their employees, while also considering the cost of new treatments in the market. They want financial security that won't compromise access to life-changing and potentially curative gene therapy treatments.

At Amwins, we offer an innovative and sustainable approach that is as evolutionary as the gene therapy treatments themselves. We recognize that employers need more than a program; they need a partner. Amwins Gene Therapy Solutions includes operational oversight, as well as ongoing market cost-benefit analysis. We are committed to offering a solution that allows employers to navigate the everchanging landscape of gene therapy with confidence. From diagnosis to reimbursement, Amwins Gene Therapy Solutions alleviates the administrative and financial burden that can accompany these treatments. Amwins is your trusted partner to navigate the current market and offers peace of mind as you face the future of gene therapy advancements.