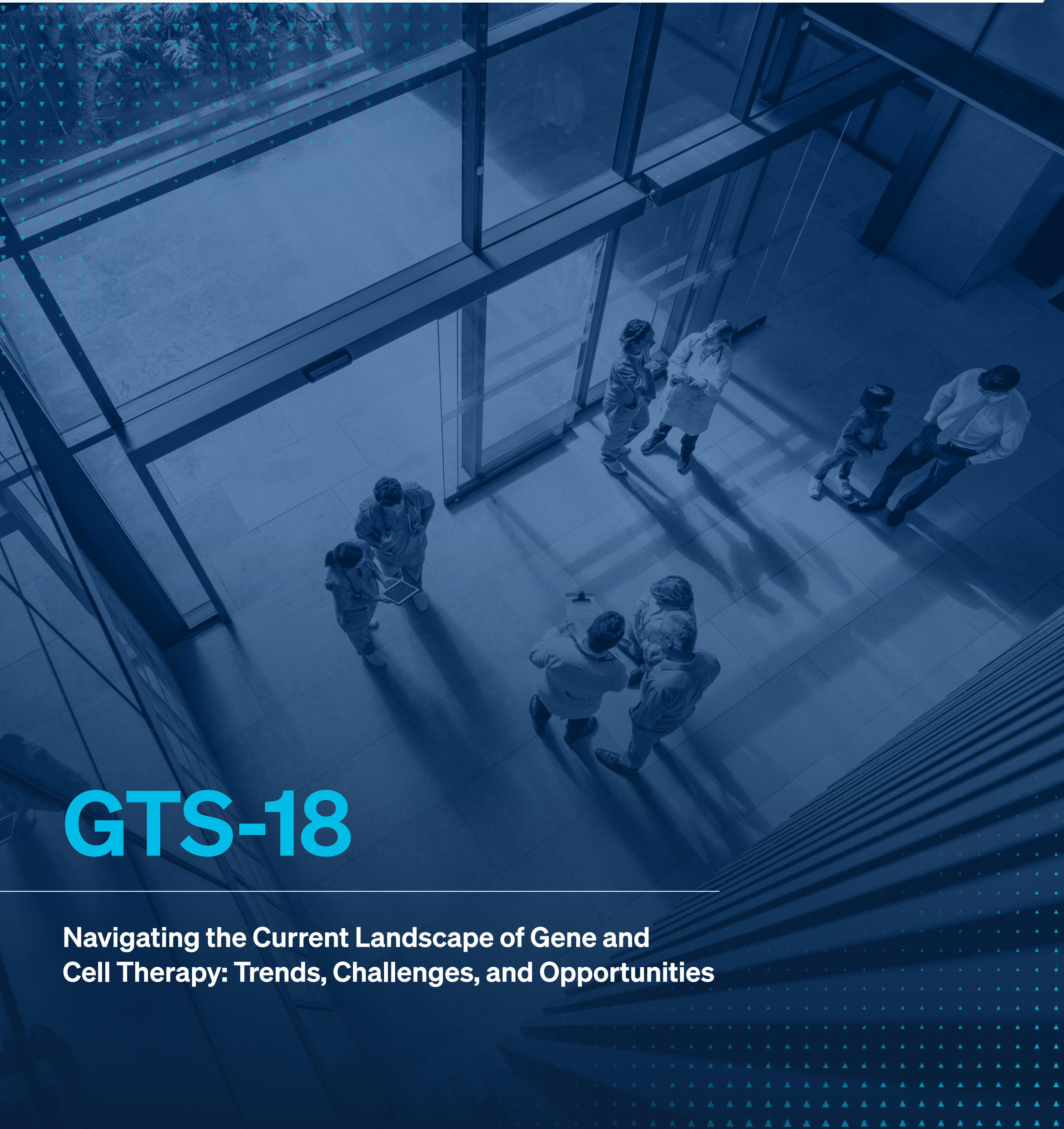
Breakthrough Care. Practical Costs.



GTS-18

Navigating the Current Landscape of Gene and Cell Therapy: Trends, Challenges, and Opportunities

The cost-benefit analysis of medical innovation is undoubtedly complex. As more life-changing treatments are approved and the potential patient pool widens, benefits brokers and employer groups need timely, thoughtful and informed solutions.

With the right partner and protective program, employers can confidently offer comprehensive group health benefits that include access to gene, cell and CAR-T therapies. Staying informed about the state of the industry — and relying on trusted partners well-versed in this evolving field — is a smart strategy to balance the promise, risk and price of health innovation for covered employees and families.

Gene, Cell and CAR-T Therapies: An Overview

Gene therapies introduce correct or healthy genes into a patient's body or alter/remove existing genes. The newly introduced — or edited — healthy genes then integrate with the host genome and transcribe to provide patients with improved quality and longevity of life. Some types of gene therapies can slow the progression of a disease state. Common genetic disorders that can be treated with gene therapies include hemophilia, Duchenne muscular dystrophy, sickle cell anemia, spinal muscular atrophy and hereditary blindness.

Cell therapy involves harvesting cells from a patient or donor, altering them to become healthy and functional, and reintroducing them into the patient's body to multiply and attack the diseased or unhealthy cells. Cell therapies may be recommended to treat certain blood cancers, such as leukemias and myeloma, as well as inherited immunocompromised disease states, such as congenital athymia. Autologous cell therapies utilize the patient's own cells, while Allogenic cell therapies use donor cells. Cell therapies hold promise for more common conditions like diabetes, Parkinson's disease, cardiovascular diseases, Alzheimer's and multiple sclerosis.

CAR-T therapies are formally recognized as Chimeric Antigen Receptor T cell therapy. CAR-T is a form of immunotherapy that enables a patient's T cells to attack diseased or damaged cells. This therapy modifies a patient's own T cells to carry a special receptor — called a CAR — on their surface, enabling them to recognize and target specific cells. Historically, CAR-T cell therapy has been used to treat certain types of blood cancers (leukemia, lymphoma and myeloma). Innovation in the CAR-T space indicates the potential to treat solid tumors, which account for over 90% of all new cancer cases worldwide. According to a report by the American Society of Gene & Cell Therapy, CAR-T therapies account for 32% of the total gene and cell therapy pipeline and target cancer treatment in 97% of cases.

▶ **In Action:**

Patients receiving cell therapy often have compromised immune systems, which increases the risk of adverse reactions and infections. By combining immunotherapy with cell therapy — as CAR-T does — the patient's immune system is strengthened during treatment to reduce complications and help patients tolerate the therapy more effectively.

CRISPR (short for "clustered regularly interspaced short palindromic repeats") is a new and evolving gene editing technology. Clinical trials for CRISPR began in 2019, using Casgevy to treat sickle cell disease. In February 2025, the first person in the world — a six-month-old baby — successfully received a customized CRISPR treatment for a rare metabolic disease. Innovation related to CRISPR technology continues to spark excitement in the scientific community for revolutionizing the study, prevention and treatment of genetic diseases.

In gene and cell therapies, cells are genetically modified outside of the body (ex vivo) or modified genetic material is directly inserted into the body (in vivo). Administration costs depend on the specific condition and the therapy; however, the cost ranges for in vivo therapies are substantially lower than those for ex vivo therapies. Ex vivo therapies include cell extraction, genetic modification in a controlled facility by skilled labor and reinfusion into the patient. Ex vivo therapies also involve longer preparation and processing timelines before a patient can receive the treatment. In contrast, in vivo therapies are delivered directly into a patient's body, eliminating complex handling processes and reducing associated infrastructure expenses. Naturally, a combination of lower cost and faster administration is meaningful to commercial payers as they assess the economic value and sustainability of emerging therapies. Most current CRISPR clinical trials take the ex vivo approach.

The U.S. Food and Drug Administration (FDA) is a federally regulated agency charged with protecting public health. Covering prescription and over-the-counter medications, vaccines and other biological products, the FDA approves new products and biologics, sets standards and regulations, monitors the marketplace and educates the public. The FDA is in place to ensure that gene and cell therapies are safe, effective and manufactured to high quality standards before they reach patients. Gene and cell therapies are regulated by the FDA's Center for Biologics Evaluation and Research (CBER), specifically its Office of Therapeutic Products (OTP), which was established in 2023 to improve review capacity, enhance expertise and streamline the development process for gene and cell therapies.

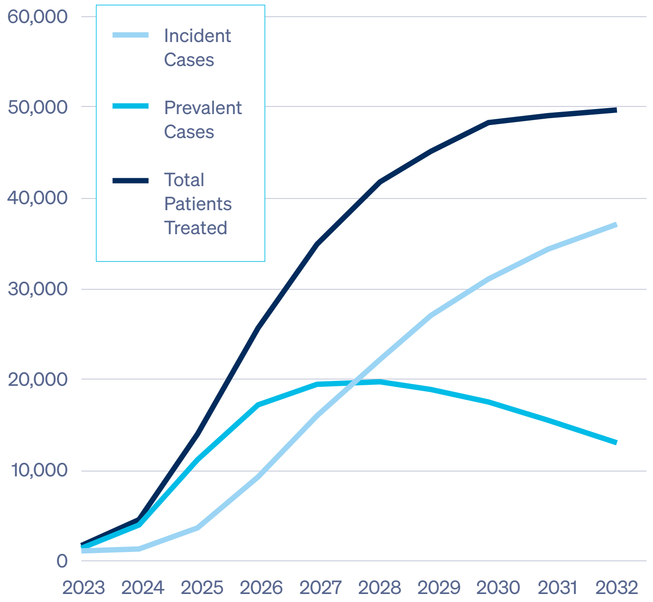
FDA officials are selected through a combination of political appointments and merit-based hiring. Agency leadership typically changes over time (or following federal election cycles), and, as such, its stance on health-related issues is prone to shifts as well.

Approved Therapies and Anticipated Utilization

As of September 2025, nearly 40 therapies[1] had received FDA approval. The majority of these approved therapies target rare or very rare diseases, but the pool of eligible patients will naturally grow as therapies come to market for more common disease states. The treatable population is expected to spike in the near future. By 2032, the projected number of patients treated (shown on the right) may encompass nearly 50,000 in the U.S. — **a growth of 163% in the next seven years.**

Understanding Prevalence vs. Incidence

Prevalence (or prevalent cases) reflects the market today, measuring the number of individuals diagnosed with and currently receiving treatment for a disease. Incidence (or incident cases) projects how many patients are likely to be diagnosed with a treatable disease over time. Incidence is influenced by factors such as birth rates, the development of new diagnostic tools, the introduction of more widespread screening panels and the availability of treatments that did not exist in the past. Together, prevalence and incidence can help guide short- and long-term decision-making.



Data Source: Managing the Challenges of Paying for Gene Therapy: Strategies for Market Action and Policy Reform: Institute for Clinical and Economic Review and NEWDIGS, Tufts Center for Biomedical System Design (April 2024)

Of the more than 4,000 gene and cell therapies in development, more than 200 are in late-phase clinical trials. Seven to 10 treatments are likely to receive FDA approval in 2026.

One source expects 85 new gene therapy[2] approvals by 2032 with an estimated 10-year list price spend of $35 billion to $40 billion.

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[1] Total number of therapies consistent with FDA recent recommendations to consider HPC cord blood treatments as a single therapy. Visit American Society of Gene + Cell Therapy to learn more.

[2] Iqvia Institute. Global Use of Medicines 2024, Outlook to 2028

While the prevalence and incidence of gene and cell therapy claims have been lower than expected to date[3], the risk should not be underestimated or ignored. It is important to understand how the timing of any new biologic's research and development, FDA approval, market introduction and patient adoption can impact a group's health benefits and claims expenses in the years ahead.

New biologics and therapies follow a similar "Ramp-up, Ramp-down" pattern. Ramp-up refers to the period after a new therapy is approved but before patients begin receiving treatment. This phase accounts for critical steps, including manufacturing, building a pipeline of eligible patients, distributing the drug to approved hospitals and preparing the health system to administer the therapy. A delayed six- to 12-month window before the first gene therapy is administered is typical.

▶ In Action:

Informal patient communities and online groups are powerful forces shaping how new drugs are perceived. Especially in rare disease communities, lived experiences of peers can be more influential than marketing materials or even clinical data.
A single positive or negative story shared in a Facebook group or rare disease forum can ripple through the community, influencing whether others feel hopeful, hesitant or vehemently skeptical. Informal spaces also become hubs for exchanging practical advice, such as how to manage side effects, navigate insurance hurdles or promote access to alternative pathways. The tone of peer-to-peer conversations can push the adoption of a therapy forward faster than formalized channels — or significantly slow it if concerns spread unchecked.

In the ramp-down phase, utilization begins to decline after a specific therapy's initial launch and peak adoption for several reasons. Naturally, a ramp-down can occur after most of the eligible patients who had been eagerly awaiting the new therapy receive treatment. New competitors, additional therapies or newer treatments may enter the market and take share. Clinical or safety considerations also impact the ramp-down phase, or emerging data may limit use in certain patients.

Essentially, ramp-up is the go-to-market build toward more steady use, while ramp-down reflects the natural decline in demand once the initial backlog is addressed. Outside of the economics of bringing a novel biologic mainstream, patients and providers are engaging in more philosophical discussions impacting gene and cell therapy adoption. Patients and providers must consider all options and alternatives. What might a person's life look like if they choose to receive a gene or cell therapy or if they don't? Is the disease state life-threatening or life-limiting? How can moving forward with treatment or foregoing it impact quality of life? Weighing the risks, benefits and costs of gene and cell therapies is incredibly personal.

With a thoughtful cost-containment solution, the covered member and their provider are empowered to choose the appropriate course of action at the right time without being limited in choice by a therapy's staggering price tag.

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[3] Many moving parts have impacted the number of gene and cell therapy claims to date. COVID-19 shifted focus away from clinical trials for rare and ultra-rare diseases, and stalled FDA reviews resulted in a backlogged approval pipeline. Today, factors such as manufacturer readiness, healthcare provider and system capacity, patient eligibility, FDA labeling and real-world treatment outcomes all influence how quickly gene and cell therapies translate into measurable claims activity.

▶ In Action:

Roctavian is a real-time example of how slower-than-anticipated patient adoption does not eliminate the gene therapy cost risk to a group health plan. This therapy was approved in 2023 for the treatment of hemophilia A in adult males. Of the estimated population living with the disease, approximately 2,500 (10% to 11%) are eligible for Roctavian. Eligibility is limited to adult males over the age of 18 and dependent on antibody levels, which may resist the virus/gene therapy treatment due to years of factor treatment. Of the eligible individuals who have not yet received Roctavian, they are either content with their current treatment regimens, hesitant to make the lifestyle changes required after therapy or prefer to wait for “less risky” and “better” developments in care. While adoption has been slow, 134 individuals have received the treatment, according to the most recently reported clinical study.

Real-Time Impact

Of the approximately 4 million members in Stealth Partner Group’s book of business, 1 in 115,000 (or 35 people) are expected to experience a gene and cell therapy claim this year. Across the first half of 2025, this block experienced 11 claims, totaling nearly $22 million. Nine more cases are expected before the year’s end, driving the total claims expense to $36 million. In 2024, this same population pool saw one gene and cell claim per 150,000 covered lives. Data indicates a 24% increase in risk over a recent one-year period. Approximately 55% of the book is enrolled in a gene therapy program. Shared risk, distributed across groups without appropriate cost-containment programs, will continue to impact the entire commercial market.

Large-scale investment in health innovation paired with the evolving FDA stance on expedited approvals in general has the potential to transform patient options across a wide range of costly and complex diseases. Of course, not every therapy will make it to market, and many will face challenges in safety, efficacy or commercial viability. Still, the pace of development and introduction of new therapies into the broader healthcare system underscores the urgent need for stakeholders, ranging from health plans and employers to providers and patients, to prepare for a future where cell, gene and CAR-T therapies become increasingly central to care, access and cost management.

The costs for the therapeutic drugs themselves range dramatically, from $250,000 to $4.25 million, and administration costs can vary from tens of thousands to upwards of $1 million. In the absence of protective cost-containment programs, high-cost gene and cell therapy claims will not only impact a particular group but also the commercial insurance market. While stop loss carriers have experienced a handful of significant gene and cell therapy claims across their blocks of business thus far, the lower-than-anticipated frequency of such claims has kept related losses manageable.

Federal Support for Removing Barriers to Gene and Cell Therapies

In response to industry requests and a need for innovation, the FDA is exploring flexible and novel trial designs, especially for pediatric rare diseases. The agency’s Center for Biologics Evaluation and Research (CBER) and Office of Therapeutic Products (OTP) improve review capacity, enhance expertise and streamline the development process for gene and cell therapies. The Accelerated Approval and Regenerative Medicine Advanced Therapy (RMAT) is intended to streamline and expedite the traditionally lengthy and high-cost approval process. Collaboratively, these offices and programs aim to spur innovation and deliver new gene and cell therapies, particularly for rare and life-threatening diseases.

Health and Human Services Secretary Robert F. Kennedy Jr. has openly expressed support for eliminating barriers that keep gene and cell therapies from the market. At a June 2025 FDA roundtable on gene and cell therapies, he committed to doing “everything in our power to sweep away the barriers from you getting those solutions to market and getting them funded and do everything that we can to support you. We are going to continue to figure out new ways... of accelerating approvals for drugs that treat rare disease, and we’re going to make this country the hub of biotechnology innovation.”

The FDA formally eliminated the Risk Evaluation and Mitigation Strategies (REMS) requirement for all currently approved CAR-T cell therapies in June 2025. This change reflects increasing confidence in providers’ capacity to manage risk effectively, expanding the possibilities for treatment delivery beyond specialized centers and hospitals to include outpatient clinics or at home. The FDA has also emphasized that the elimination of REMS will help reduce administrative burdens, expand patient access and streamline delivery of potentially curative treatments . Ongoing safety monitoring and long-term follow-up requirements remain in place.

In step with the agency’s commitment to innovation in biologics, the FDA introduced its Rare Disease Evidence Principles (RDEP) program in early September 2025. RDEP intends to expedite approval of gene therapies for genetic diseases affecting fewer than 1,000 people. Under RDEP, sponsors can seek approval based on a single-arm trial supplemented by supportive data and patient experience. Treatments for disease states that cause severe disability or death and lack existing, effective treatments may be considered for the RDEP program.

Understanding FDA Labeling and Its Role in Patient Adoption

FDA labeling is the official, legally required description of how a therapy should be used. The label is based on evidence reviewed during the approval process and may change over time. Initial FDA labels are typically limited to a smaller subset of the total prevalent population affected by a specific condition. Knowledge about a therapy doesn't stop at approval. Naturally, FDA labels for pharmaceuticals can change over time as the risk-benefit profile becomes clearer.

Projected therapy approvals in 2026 will address more common conditions such as neovascular (wet) age-related macular degeneration — the most common cause of severe vision loss across the globe — knee osteoarthritis, prostate cancer and diabetic peripheral neuropathy. While these types of health conditions impact millions of people, initial FDA labeling decisions are typically narrower than the full scope of the prevalent population.

▶ **In Action:**
The FDA's 2024 accelerated approval to expand the use of Elevidys (a therapy used to treat Duchenne Muscular Dystrophy) to individuals ages 4 and older came center stage in the summer of 2025. Two non-ambulatory boys linked to the gene therapy died from acute liver failure in March and July 2025. This spurred an FDA investigation and subsequent recommendation to put further clinical trials involving non-ambulatory patients on hold. GTS-18 restricts Elevidys' eligibility to ambulatory patients between the ages of four and eight, aligning with the parameters outlined in the FDA's initial 2023 approval of the treatment.

Misalignment between FDA labels and patient desire — or desperation — for treatment can further complicate coverage limitations and decisions. Clinical recommendations related to rare disease treatments must be medically sound, and, in the best cases, cost-effective for employers and patients.

Gene and Cell Therapy Solutions with Cost-Containment and Care in Mind:

Introducing Amwins' GTS-18

Our Gene Therapy Solutions — in its most recent, expanded iteration as GTS-18 — supplements your current employee health and group benefits strategy. In some cases, these life-changing and life-saving treatments are the only option for babies born with debilitating and torturous conditions. Payers should not be tasked with interfering with or restricting access to care due to insurmountable cost — especially as some therapies must be administered within a very limited window.

> **Gene and cell therapies can completely change the course of a person's life. Especially when genetic diseases are diagnosed in babies, the entire trajectory of that newborn's life — and its family's — depends on thoughtful planning that happens now. Covering high-cost health conditions that stem from personal lifestyle choices but not covering a therapy that could cure a genetic condition in a baby or child is a tough stance to justify.**
>
> *— Scotty Campbell,*
> *Executive Vice President, Stealth Partner Group*

The costs of not treating a patient with an FDA-approved therapy push far beyond a number on a balance sheet. Patients left untreated will, in many cases, develop co-morbidities that decrease their lifespan while increasing lifetime medical expenditures. One must also consider costs for:

- Medical equipment
- Living-space modifications
- Caregiving expenses
- Extensive time off work
- Lost productivity for parents/ familial caregivers and/or patient
- Long-term disability for the patient
- Internal and external reputational damage related to care denials and coverage disputes

GTS-18 provides first-dollar reimbursement for 18 high-cost, clinically appropriate therapies relevant to Group Health Plan (GHP) participants.

When evaluating which therapies to include and exclude from our program, we research the treatable disease states to understand the potential risks, financial impacts and human-centric considerations related to GHPs and covered members. For example, Adstiladrin (a gene therapy for patients in advanced stages of non-muscle-invasive bladder cancer) is not included in our program. The median age at diagnosis is 73; therefore, the value in including this therapy in a GHP program is minimal.

As gene and cell therapies earn FDA approval, our experts do not blindly wrap all new treatments into our program. Although some cost-containment programs may highlight inclusion of 20+ gene and cell therapies, a critical fiduciary may realize such programs include therapies in late-phase clinical trials that have not yet received FDA approval. Even slight adjustments in a drug's labeling — due to a high-ranking administrator's strong desire to appease impacted families without an alternate care path — may open doors for less-than-desirable or even dangerous results. Amwins' in-house experts and our partners at Emerging Therapy Solutions are accessible resources for real-time updates on patient safety, the robust research and development pipeline, drug costs and relevant legislative actions.

> **Our team and partners are deeply attuned to relevant policy changes and process shifts that may impact the gene and cell therapy space. I've seen firsthand the impacts of a group without a plan. It's unfortunate but it's also preventable. When employers proactively put protective programs in place, they can confidently provide robust and responsive health coverage to their team members — especially in high-stakes situations.**
>
> *— Meredith Hunter,*
> *Senior Vice President, Stealth Partner Group*

Exclusive GTS-18 Advantage: The Emerging Therapy Solutions (ETS) Partnership

Emerging Therapy Solutions is the only organization dedicated exclusively to the complex field of gene and cell therapies and transplants. Backed by more than 30 years of specialized expertise, ETS is the leading resource for hundreds of payers, brokers, consultants, stop loss and reinsurance carriers, translating uncertainty into foresight, cost savings and peace of mind. ETS also works to align various stakeholders (providers, payers, manufacturers, to name a few) to overcome cost-related hurdles and deliver strategic reimbursements.

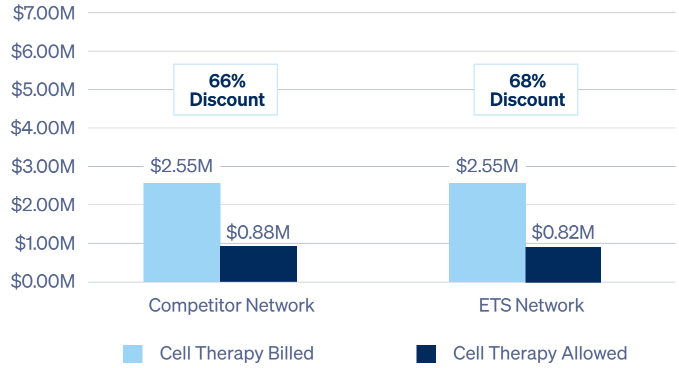
The ETS partnership is an especially relevant value-add today. As the adoption of gene and cell therapies has been slower than expected, many providers and payers have yet to build the necessary infrastructure and processes to truly estimate systemic costs. Actuarial forecasts provided by ETS can demystify a group's potential utilization and projected claim spend.

ETS also manages proactive patient outreach, provider network management and payment structuring. ETS works to guarantee transparent and competitive drug pricing based on Wholesale Acquisition Costs (WAC) and intense evaluation of provider-to-provider contracts. Cost nuances can also be driven by regions and geography, making the ETS-provided insights especially valuable to plan fiduciaries and decision-makers who expect favorable and reasonable prescription drug charges.

Estimated Savings Opportunities: ETS Network vs. Competitor Networks

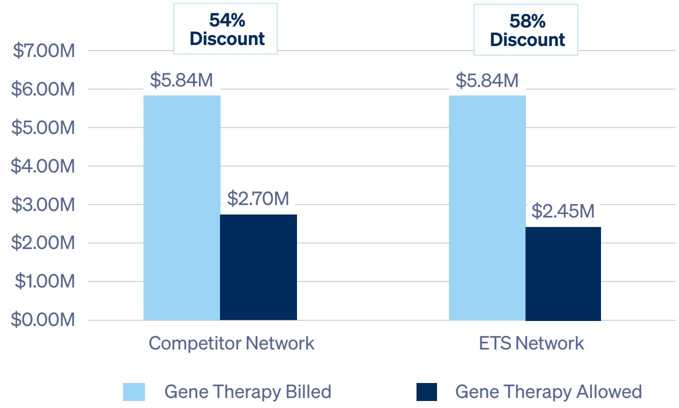
Cell Therapy

Savings: $62K per CAR-T Case



Gene Therapy

Savings: $256K per Gene Therapy Case



Savings estimates are based on ETS commercial data from 2022 to 2024. Savings are calculated by comparing ETS-allowed amounts at contracted centers to average allowed amounts from other health plan networks for the same service. Analysis includes complete episodes of care.

▶ **In Action:**

Acquiring a pharmaceutical at its WAC is significantly more cost-effective than the widely accepted buy-and-bill purchasing style deployed by the majority of hospital systems. Commonly referred to as a drug's "list price," WAC can be accessed through wholesalers and strategic negotiations. All gene and cell therapy programs reimburse only up to WAC. If a network pays more for a drug, the stop loss carriers are left with the balance.

Hospitals often purchase high-cost therapies directly from manufacturers and apply markups through network contracts. Even small percentages can have a major financial impact on multi-million-dollar drugs. (A modest 1.5% markup on $3.5 million Hemgenix increases the cost by $52,500.) The ETS partnership helps keep pharmaceutical costs close to or parallel with WAC, reducing excess and unexpected financial liability for stop loss carriers and commercial payers.

> **Our goal is to ensure that every patient receives the right therapy at the right time — delivered seamlessly and responsibly in the right place. Of course, managing and mitigating risk when it comes to healthcare costs is complex, but we believe the group benefits space is still a very human-centric business. We're completely dedicated to educating patients, identifying high-quality providers, securing market leading pricing for groups and ensuring access to life-changing care.**
>
> — Ashley Hume,
> President, Emerging Therapy Solutions

ETS Center of Excellence: A Simple-to-Elect Value-Add

Included in GTS-18, participants can simply elect to integrate the ETS Center of Excellence (COE) into their benefit structure. Participants benefit from:

- Pre-negotiated case rates or a competitive percentage of billed charges for all FDA-approved gene and cell therapies, not just those eligible for reimbursement through GTS-18
- Negotiated cost coverage that accounts for treatment and associated administration expenses
- Ready-to-implement Summary Plan Description language for seamless benefit integration

Please note: Some TPAs may restrict access to the ETS COE network, requiring clients to use their own COE providers. Approval may vary by client situation.

For some disease states, GTS-18 covers more than one therapy. Promoting access to care and encouraging patient choice (when clinically sound) without adding significant expense to plan sponsors is a core priority for us.

GTS-18 Reimburses Therapy Expense for the Following Disease States:

Aromatic L-amino Acid Decarboxylase (AADC) Deficiency is a very rare and fatal childhood genetic disorder with symptoms presenting within the first months of life. Most affected individuals experience movement disorders and autonomic dysfunction, which can lead to orthopedic and cardiac complications, temperature instability and a low level of glucose in the blood. Of the less than 60 patients in the U.S. who have been diagnosed with AADC, 100% of the prevalent commercial population is eligible for treatment with Kebilidi, the only approved gene therapy to treat this disease. The therapy is administered in vivo, through four direct infusions into the brain in a single surgical session. Efficacy is estimated for up to five years, eliminating the need for a combination of medicines that are limited to managing disease symptoms. **GTS-18 covers Kebilidi up to $3,950,000.**

Cerebral Adrenoleukodystrophy (CALD) is a rare genetic disorder causing significant damage to the brain. Symptoms

Information related to specific disease states, incidence, prevalence, therapies and treatments has been gathered from a variety of publicly available reports, articles and documents, along with proprietary research. All products, brands and names are the property of their respective owners.

typically present between three and 10 years of age, and, without treatment, 50% of CALD patients die within five years of initial symptoms. Prevalence of CALD is 340; incidence is 81. A known cure for CALD does not exist; however, Skysona, a gene therapy, offers the opportunity for disease stabilization for boys ages four through 17 with early, active CALD. Skysona is a one-time, ex vivo infusion to halt disease progression, but it must be administered at an early age. Almost 11% of the prevalent commercial population is eligible for treatment with Skysona. **GTS-18 covers Skysona up to $3,000,000.**

Congenital Athymia means that a baby is born without a thymus, a gland at the top of the heart responsible for producing T-cells. Without T-cells, the newborn's immune system is severely dysregulated and deficient from the first minutes of life. Children with congenital athymia who do not receive supportive care will live only two to three years. Within their short lifespan, pediatric patients will face lengthy hospital stays and acute medical interventions. Prevalence of congenital athymia is 30; incidence is 21. One hundred percent of the prevalent population is eligible for treatment with Rethymic, the only known treatment for this devastating disease. Rethymic is a one-time, allogenic cell therapy administered in vivo using processed thymus tissue to aid in immune system reconstruction. **GTS-18 covers Rethymic up to $2,811,385.**

Duchenne Muscular Dystrophy (DMD) is a rare, genetic and fatal form of muscular dystrophy. It only impacts males, and symptoms (including delays in muscle development, lack of physical motor function or minimal muscular control) present by age three. Most individuals with DMD will become wheelchair dependent by the age of 12 and require assisted ventilation around age 20. DMD causes significant damage to a person's heart and respiratory symptoms, shortening their expected lifespan to 23.7 years. Prevalence of DMD is 10,015; incidence is 362. Although there is no cure for DMD, multiple treatment options intending to delay disease progression are available. Approximately 31% of the prevalent population is eligible for treatment with Elevidys, a one-time, in vivo gene therapy for ambulatory patients. **GTS-18 covers Elevidys (up to $3,200,000) for individuals between the ages of four and eight who meet all current FDA-labeling guidelines and have been diagnosed within the plan period.**

Hemophilia A and B are inherited disorders that prevent blood from clotting. Depending on the type, the disease may be diagnosed within the first year of life or even prenatally. Mild cases may remain undetected until adulthood, often surfacing after a bleeding episode or following a surgery.

- **Hemophilia A** causes abnormal development of coagulation factor VIII. This disease can pose a life-threatening risk (based on the severity of the condition in situations of injury or trauma) and can result in long-term joint and organ damage. Individuals with hemophilia A have historically been reliant on an interim solution: factor replacement infusions, multiple times per week, for the duration of their life. Prevalence of hemophilia A is 19,712; incidence is 400. Various treatment options exist, including infusions of coagulation factor VII multiple times per week for a lifetime. Approximately 15% of the prevalent commercial population is eligible for treatment with Roctavian. The duration of effect for Roctavian, one-time, in vivo gene therapy, is estimated to be years. **GTS-18 covers Roctavian up to $3,031,840.**

- **Hemophilia B** is caused by a gene mutation that prevents the production of coagulation factor IX. Similar to hemophilia A, the condition is mild, moderate or severe, with between 30% to 50% of the patient population classified as severe. Prevalence of hemophilia B is 6,242; incidence is 100. Multiple treatment options exist, including Hemgenix, a one-time, in vivo gene therapy with durable curative effects. Following treatment with Hemgenix, individuals with hemophilia B begin to produce their own factor IX, eliminating the need for lifelong infusions. Almost 16% of the prevalent commercial population is eligible for treatment with Hemgenix. **GTS-18 covers Hemgenix up to $3,500,000**. Although Beqvez was previously included in our Gene Therapy Solution program, it is no longer an approved treatment. Beqvez was discontinued by the manufacturer and withdrawn from the market.

Idiopathic Macular Telangiectasia Type 2 (MacTel 2) drives central vision loss in middle-aged adults. This progressive disease affects both eyes equally, but often at different rates, across a 10- to 20-year span. Prevalence of MacTel 2 is 129,794; incidence is 14,749. Almost 54% of the prevalent population is

Information related to specific disease states, incidence, prevalence, therapies and treatments has been gathered from a variety of publicly available reports, articles and documents, along with proprietary research. All products, brands and names are the property of their respective owners.

eligible for treatment with Encelto, the only available therapy for MacTel 2. This one-time, cell-based gene therapy is delivered in vivo, by surgically implanting a small membrane — about the size of a grain of rice — inside the eye to release a protein. This treatment received FDA approval in March of 2025, and the first Encelto surgery was performed in August of the same year. **GTS-18 covers Encelto up to $400,000.**

Leber Congenital Amaurosis (LCA), referred to as hereditary or inherited blindness, impacts one in 71,387 people. LCA is a progressive disease triggered by a single gene mutation that inhibits the retina's functionality. Typically, a patient is diagnosed with LCA between birth and the age of five. Impaired vision can begin at an early age, and most people living with LCA are classified as legally blind by the age of 20. Incidence is 3,729, and 2.4% of the prevalent commercial population is eligible for treatment with Luxturna. While there is no known cure for LCA, Luxturna, a one-time, in vivo gene therapy, can repair this mutation and improve vision, allowing patients to perform activities of daily living with more ease. **GTS-18 covers Luxturna up to $913,750.**

Metachromatic Leukodystrophy (MLD) is a genetic condition that impacts the brain, spinal cord and peripheral nervous system. More than half of patients diagnosed with MLD do not survive past childhood. MLD prevents the body from breaking down sulfatides, which can contribute to developmental delays, speech impairments, seizures and loss of motor function. Prevalence of MLD is 2,028; incidence is 95. Almost 77% of the prevalent commercial population is eligible for treatment with Lenmeldy, a one-time, ex vivo gene therapy. While no curative treatment options for MLD exist, Lenmeldy has been shown to significantly reduce the risk of severe motor impairment and extend a child's life. All of the children participating in clinical trials were alive at age six, compared to the 58% survival rate when MLD is left untreated. Seventy-one percent of Lenmeldy patients could walk unassisted by age five. Lenmeldy is the only treatment option for individuals diagnosed with MLD at this time and no additional therapies to treat this disease state are in the development pipeline. **GTS-18 covers Lenmeldy up to $4,250,000.**

Multiple Myeloma is a cancer of the plasma cells (a type of white blood cell) within bone marrow. Plasma cells generate antibodies to fight infection and disease, but cancerous myeloma cells multiply rapidly and negatively impact the number of healthy blood cells. Myeloma cells break down bone tissue, can form tumors within the bone marrow and may also release proteins that adversely alter kidney function. Prevalence of multiple myeloma is 128,989; incidence is 32,270. A variety of treatments are available, including two FDA-approved autologous T-cell therapies. Seventy-nine percent of the prevalent commercial population is eligible for Carvykti as a 2nd line treatment and 63% are eligible for Abecma as a 3rd line treatment. Within this same population, 41% are eligible to receive either therapy as a 5th line treatment. Carvykti and Abecma are both one-time, autologous, ex vivo infusion therapies that target the cancerous cells and leave healthy cells unharmed. **GTS-18 covers Carvykti (up to $555,310) and Abecma (up to $528,312).**

Recessive Dystrophic Epidermolysis Bullosa (RDEB) is a rare genetic skin disorder that presents at birth or shortly thereafter. Babies born with RDEB do not have enough functional collagen, and their extremely fragile skin separates and tears easily. The severity of RDEB varies between individuals and can lead to blisters, wounds and scarring. Prevalence of RDEB is 1,136; incidence is 324. Two currently approved treatment options (Zevaskyn and Vyjuvek) are intended to reduce life-threatening complications, promote wound healing and reduce pain. Approximately 67% of the prevalent commercial population is eligible for treatment with Zevaskyn, an ex vivo cell-based gene therapy administered through a one-time surgical application. **GTS-18 covers Zevaskyn up to $3,100,000.**[4]

Sickle Cell Disease (SCD) is characterized by abnormally shaped red blood cells. A mutation in the HBB gene affects hemoglobin, a protein essential for transporting oxygen in the bloodstream. Abnormal hemoglobin will change the red blood cells' shape from disc-shaped to c-shaped or "sickle." SCD can cause blockages within vessels, infections, multi-organ failures, heart attacks, strokes and vaso-occlusive crises characterized by debilitating pain. Prevalence of SCD is

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[4] Vyjuvek is a topical, multi-use treatment also approved to address RDEB. T The expected lifetime costs of Vyjuvek are likely to exceed $20 million. Following a careful analysis of cost, utilization and overall program impact, Vyjuvek was not included GTS-18.

Information related to specific disease states, incidence, prevalence, therapies and treatments has been gathered from a variety of publicly available reports, articles and documents, along with proprietary research. All products, brands and names are the property of their respective owners.

100,000; incidence is 1,607. Treatment options for SCD include bone marrow or stem cell transplants, blood transfusions and two gene therapies (Casgevy and Lyfgenia) introduced weeks apart in 2023. Approximately 17% of the prevalent commercial population is eligible for treatment with Casgevy (a CRISPR therapy) and Lyfgenia. Both are ex vivo infusions, and both are demonstrating the potential to be a one-time, functional cure. Patient adoption of both FDA-approved gene therapies has been relatively slow, with experts citing wariness of side effects and potential for cancer risk. **GTS-18 covers Lyfgenia (up to $3,100,000) and Casgevy (up to $2,200,000).**

Spinal Muscular Atrophy (SMA) is a neuromuscular disease often diagnosed through routine newborn screening. Symptoms show within six to 18 months. SMA is serious and life-threatening, impacting the skeletal and muscular system and driving loss of voluntary muscle movement. Patients with SMA Type 1 will not live beyond two years of age. However, early treatment slows progression, helps individuals reach milestones and gives hope for greater independence and a more positive prognosis. Untreated individuals will eventually be unable to walk without assistance. Prevalence of SMA Type 1 is 1,610 and prevalence of Type 2 is 3,944; incidence is 228 and 76 respectively. Nearly 87% of the prevalent commercial Type 2 population is eligible for treatment. There is no cure for SMA, but approved treatments (including Zolgensma, Spinraza and Evrysdi) can prevent death, manage symptoms and delay disease progression. **GTS-18 provides coverage to treat SMA Types 1 and 2[5] with Zolgensma and Spinraza, both up to $2,511,291.**

Synovial Sarcoma is a rare type of soft tissue cancer that typically develops around large joints in the extremities but can occur almost anywhere in the body. One third of patients are diagnosed before they reach 30 years of age. Prevalence of synovial sarcoma is 11,494; incidence is 580. Current treatment options include tumor removal (surgery), chemotherapy, radiotherapy, a targeted oral cancer therapy (Votrient) and Tecelra, a genetically modified autologous T-cell immunotherapy. Tecelra is a one-time, ex vivo infusion approved for adults 18 years of age or older. It is used when other treatments do not work. Of the prevalent commercial population, 34% are eligible for treatment with Tecelra. **GTS-18 covers Tecelra up to $727,000.**

Transfusion Dependent Beta Thalassemia (TDT) is a rare blood disorder that affects the production of hemoglobin. TDT is diagnosed in early childhood – typically between six months and two years of age – when severe anemia symptoms appear. People living with TDT are dependent on blood transfusions for survival and often have long-term care needs paired with significant health complications. Regular infusions may temporarily improve the condition but do not deliver the gene that would enable the body to produce adequate hemoglobin on its own. Prevalence of TDT is 1,500; incidence is 152. While lifelong red blood cell transfusions can be used to treat TDT, approximately 95% of the prevalent commercial population is eligible for treatment with Zynteglo, and 80% of the same population is eligible for treatment with Casgevy (a CRISPR therapy). **GTS-18 covers Zynteglo (up to $2,800,000) and Casgevy (up to $2,200,000).**

Amwins Pro Tip

Evaluating historical claims data — including J-codes (medical codes for injectable and infused drugs) and ICD-10 codes (diagnostic codes for conditions and diseases) — may help groups understand their potential exposure for future gene and cell therapy claims. Reviewing the codes can provide insights into certain diseases and health conditions that may lead to high-cost gene and cell therapy claims. It is also important to note that J-codes and ICD-10 codes are long-tail indicators of risk. The timing lag from a member's visit to the claim processing and, eventually, the plan sponsor's evaluation of the codes does pose a challenge. Ask your Amwins representative for our curated list.

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[5] Limited data exists to validate the use of gene therapy to treat SMA Types 3, 4 and 5. As such, GTS-18 only covers therapies to treat SMA Types 1 and 2.

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Amwins Gene Therapy Solutions (GTS-18): A Strategic and Sensible Cost-Containment Strategy

GTS-18 – our most recently expanded program — includes 18 life-changing therapies for new and renewing groups. This program reimburses group health plans and their coordinating stop loss carrier for qualified claims. Qualifying reimbursements extend from the first dollar of group health plan expenses up to specified limits — typically set or guided by Wholesale Acquisition Costs (WAC) — for each covered therapy.

Following the adjudication of a qualified claim, our program reimburses up to the specific stop loss deductible or the full value of the laser (if applicable). The stop loss carrier will receive the balance of the reimbursement up to the maximum value.

GTS-18 is transferable, and the group health plan does not run the risk of losing the program, even if fiduciaries elect underlying plan changes to their network, PBM, TPA or other partners.

GTS-18 carries a cost of $4.75 per enrolled employee per month (PEPM). Our commitment to ensuring access to this evolving program is backed by our decision to maintain pricing for 2026, while also incorporating additional covered therapies.

GTS-18 Program Eligibility

Treated Disease	Covered Pharmaceutical	Maximum Reimbursement	Administration Type
Metachromatic Leukodystrophy (MLD)	Lenmeldy	$4,250,000	Ex Vivo
Aromatic L-amino Acid Decarboxylase (AADC) Deficiency	**Kebilidi**	$3,950,000	In Vivo
Hemophilia B	Hemgenix	$3,500,000	In Vivo
Duchenne Muscular Dystrophy (DMD)	Elevidys	$3,200,000	In Vivo
Sickle Cell Disease (SCD)	Lyfgenia	$3,100,000	Ex Vivo
	Casgevy	$2,200,000	CRISPR
Recessive Dystrophic Epidermolysis Bullosa (RDEB)	**Zevaskyn**	$3,100,000	Ex Vivo
Hemophilia A	Roctavian	$3,031,840	In Vivo
Cerebral Adrenoleukodystrophy (CALD)	Skysona	$3,000,000	Ex Vivo
Congenital Athymia	Rethymic*	$2,811,385	Allogeneic
Transfusion Dependent Beta Thalassemia (TDT)	Zynteglo	$2,800,000	Ex Vivo
	Casgevy	$2,200,000	CRISPR
Spinal Muscular Atrophy (SMA) Types 1 and 2 (children ages 2 and under)	Zolgensma	$2,511,291	In Vivo
	Spinraza**	$2,511,291	In Vivo
Leber Congenital Amaurosis (LCA)	Luxturna	$913,750	In Vivo
Synovial Sarcoma	**Tecelra***	$727,000	Autologous
Multiple Myeloma	Carvykti*	$555,310	Autologous
	Abecma*	$528,312	Autologous
Idiopathic Macular Telangiectasia Type 2 (MacTel)	**Enceltro**	$400,000	In Vivo

* Cell Therapies
** Maintenance drug; Cost in year 1 is ~ $750K and $~375K in subsequent years.
Bold designates a new addition to our robust gene and cell therapy program, GTS-18, effective 1/01/2026.
Beqvez, a therapy to treat Hemophilia B, has been removed from our current program. The manufacturer discontinued this therapy and pulled it from the market in February 2025.

Coverage Criteria: Reach out to your Amwins representative for full eligibility details.
All FDA-approved gene and cell therapy treatments qualify for Emerging Therapy Solutions (ETS) Center of Excellence (COE) pricing. To qualify, a group must adopt plan language provided by ETS.

▶ In Action:

A newborn was diagnosed with Type 1 Spinal Muscular Atrophy within days of birth. Amwins' Gene Therapy Solution was in place for this municipal group of over 5,000 lives, as well as a plan to seek timely care for the newborn. The Zolgensma gene therapy was approved by the health plan and administered within a week of the newborn's birth. An $800,000 specific deductible was in place, reducing the plan's exposure by $1.4 million. While the stop loss carrier was responsible for a $54,000 balance, a 0% increase at renewal was significant. Aside from the financial benefits realized with a cost-containment program, today the child is crawling, walking, talking and eating — all of which would not have been possible without access to life-changing therapy.

A group without a gene and cell therapy cost-containment program also experienced a Zolgensma claim but with vastly different results. The newborn also received life-saving care, and the stop loss carrier bore the brunt of the $2.2+ million claim. At renewal, the group received a 33% rate increase. While the rate increase was not solely attributed to the gene therapy claim, it will have a lasting impact on the group for years to come.

Through our strategic affiliation with ETS, clients can benefit from cost mitigation strategies related to all gene and cell therapy claims, not only the therapies included in GTS-18. This partnership allows Amwins to reimburse for only the highest-risk treatments while helping clients broaden coverage and manage immediate and long-term stop loss impacts.

> **Decisions related to Group Health Plans have to make financial sense, but they cannot be driven by cost alone. The human impact matters, and that's why we will continue to expand our strong GTS program with relevant, life-saving therapies. Our strategic partnership with ETS reflects an ongoing commitment to offering cost-effective protection for businesses that prioritize thoughtful, people-centric health plans.**
>
> – Mitch Rogers,
> Senior Vice President, Stealth Partner Group

Benefits and Advantages of GTS-18

As your trusted partner for cost-containment solutions, Amwins continues to evaluate market needs. Our goal is to identify the most advantageous solutions to help administrators maximize their budget, mitigate unpredictable risk and support their workforce through innovative, responsive and robust group health offerings.

Cost-Effective Pricing and Simplified Billing

With significant program enhancements, we've maintained competitive pricing structures that deliver exceptional value. Program fees are included in the stop loss protection to reduce administrative burden and related complexities. Our 2026 pricing — $4.75 PEPM — remains steady from last year.

Access to Independent Industry Experts

Our in-house experts and industry-leading partners who actively monitor the FDA approval pipeline, clinical outcomes and the legislative landscape are available as resources to our clients.

Portability

GTS-18 is a fully portable, independent cost-containment program that stays with the group regardless of changes to TPAs, stop loss carriers or PBMs. Unlike cost-containment programs directly tied to specific TPAs or carriers, GTS-18 ensures continuous coverage without resetting benefits or triggering pre-existing condition exclusions. Changes to the underlying plan (except for stop loss termination) do not affect GTS-18 coverage terms.

Meaningful Protection

First-dollar protection is given to the plan sponsor. Removing the cost burden of a qualifying gene or cell therapy claim for an employer not only reduces the exposure for the stop loss carrier but also helps manage stop loss rates for years to come.

Strategic Partnerships that Drive Savings

The GTS-18 collaboration with ETS delivers measurable cost-saving opportunities, clear guidance and guardrails around eligible charges. ETS' COE contracts include favorable pricing for drug expenses and administration.

Expanded Treatment Coverage and Responsive Program Adjustments

We've broadened our therapy options to include breakthrough gene and cell therapy treatments, ensuring that your members have access to the latest medical innovations. From year to year, we will strategically add (or remove) therapies to balance competitive pricing, program value and achievable results for patients and families.

GTS-18 is our broadest offering yet. Previous iterations of our program (GTS-5 and GTS-15) will be discontinued for new enrollments and renewals, allowing us to ensure that our participants receive the most comprehensive coverage.

> **With dollar-one coverage, expert clinical support and the ETS partnership, GTS-18 helps protect clients from catastrophic claims and compounding premium hikes. Simply put, this program delivers measurable long-term ROI and it goes where you go.**
>
> — Clifton Browning, Senior Vice President, Stealth Partner Group

Turn Strategy into Action. Partner with Purpose.

Balancing comprehensive health benefits, financial risk and economic efficiency is no small task — especially in the absence of manufacturer warranties and amid changing clinical data and FDA approvals. But with GTS-18, employers gain more than just smart coverage for gene and cell therapy claims. Backed by the combined expertise of Amwins and Emerging Therapy Solutions, groups can take advantage of ongoing cost-benefit analyses, streamlined administration and relief from the financial and operational burdens of high-cost therapies. From diagnosis to reimbursement, we are a trusted partner — offering foresight, strategic insights and a cost-effective, sustainable approach to a future where gene and cell therapy advancements are already here.

CONTACT

Your local Amwins broker is ready to help you place coverage for your clients. Connect with us and learn more today.

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