Rare Diseases
Lurking in Your Workforce

Written By Laura Carabello

While some diseases are classified as "rare," they are not uncommon. Unfortunately, most rare diseases are serious or life-threatening, leading to significant morbidity and mortality.

In the U.S., the National Institutes of Health defines rare diseases as those affecting fewer than 200,000 individuals, the majority of whom are children. With a greater understanding of disease genetics, experts agree that there are more than 10,000 rare diseases -- 80% of which are genetic.

But rare diseases are not just genetic and can also stem from infections, environmental factors, allergies or even rare forms of cancer. According to the organizers of Rare Disease Day, 1 in 5 cancers is classified as rare, touching lives with more than just medical concerns as they shape everyday life, work and education in ways that are often invisible. Individuals and families affected by rare diseases often experience problems, such as delays in obtaining an accurate diagnosis, difficulty finding a healthcare provider with expertise in their condition and a lack of access to treatments or ancillary services.

Source: Charles River Research

The impact of rare disease on employer health plans in 2026 will be significant, primarily the result of the high cost of new specialty drugs and gene therapies. With one-time costs of well over $2 million, these innovative, high-impact treatments --

though used by a small number of people -- contribute to a projected median 9% increase in overall healthcare costs for employers.

Estimates published in Drug Discovery Today project that by 2030, there may be over 63 FDA-approved Cell and Gene Therapies (CGTs) in the US. Despite these advances and increased understanding of the causes and mechanisms of many rare diseases, the Food and Drug Administration (FDA) reports that while more than 880 drugs and biologics have been approved for the treatment of rare diseases, 95% of rare diseases do not have an FDA-approved treatment.

One major challenge is that research on rare diseases is especially reliant upon global partnerships. With so few patients available to study in any one country, scientists are forced to search extensively to identify the causes and test potential treatments.

Source: Global Genes

EMPLOYERS CONFRONT MULTIPLE CHALLENGES

Plan sponsors face the soaring, high costs of specialty drugs, including CGTs for genetic disorders, complex conditions and rare cancers. This landscape is about to change as the FDA is expected to approve around 50 CGTs in 2026, many of which target these diseases. These innovative treatments will impact the employer spend, with Deloitte projecting that employers with 7,500 employees have a 1 in 4 chance of experiencing a CGT claim by 2029.

While these therapies offer dramatic improvements in advancing optimal health outcomes, employers are grappling with how to make these groundbreaking but expensive treatments available. In response to these exorbitant costs, especially the multi-million-dollar specialty and rare disease drugs, employers are expected to implement tighter utilization controls and prior authorization requirements to mitigate the proliferation of high-cost claims.

BURDEN OF RARE DISEASE

In the workplace, employees and their families are dealing with delayed diagnosis, as the advocacy organization Global Genes reports that it takes an average of 7 years to get an accurate diagnosis for a rare disease – and in many cases, it may take much longer, or even a lifetime. This can lead to missed treatment opportunities and a prolonged impact on their work and personal lives.

The burdens of rare disease extend beyond the employee who has the condition. A worker may take on significant caregiving responsibilities – often for a child or relative -- imposing complex medical, social and economic challenges that affect on-the-job performance, productivity and retention. To address these issues, employers are beginning to focus on condition support services, escalating the number and scope of condition management and care coordination programs to better support employees and families with complex and chronic conditions, including rare diseases.

COST-CONTAINMENT OPPORTUNITIES

Many employers are also considering other tactics, such as increasing employee cost-sharing through higher deductibles and out-of-pocket maximums or utilizing risk-pooling strategies, where multiple health plans contribute to a shared fund for high-cost therapies, allowing them to distribute financial burdens more equitably. Mortgage models offer another approach, amortizing costs over time with subsequent payers assuming payments if members change plans.

These models can incorporate portability mechanisms, allowing the initial payer's investment to follow the member. While each model has limitations, they aim to better align payer incentives with patient outcomes and therapy value. As employers reassess their benefit strategy, they remain sensitive to member needs for improved access to life-changing therapies and are partnering with Centers of Excellence (COEs) for complex conditions, like cancer.

The FDA reminds plan sponsors to align their coverage policies with FDA-approved indications. FDA reviewers carefully consider the scientific evidence and consistencies in disease process across different groups, as well as a drug's overall benefits and risks, in determining the patient population appropriate for treatment.

These regulators advise all payers to uphold the FDA's authority in determining the safety and efficacy of medical products, including accelerated approval drugs, and cover the entire population included in the gene therapy's "indication and usage" section of the prescribing insert. They further recommend upholding physician authority in determining medical necessity as physician specialists are experts in their field, spending years in training, research and clinical practice. Health plan decision-makers should consult these experts in developing coverage policies to ensure alignment with therapeutic area understanding and clinical practice, and specialists should also be included in external reviews.

Source: Skyquest


Melinda Alba

INNOVATIVE DATA AND TECHNOLOGY SOLUTIONS

To navigate this environment, benefit leaders are emphasizing data-driven strategies and evidence-based support to manage costs while doing the "right thing" for their employees. Advanced data analytics and new technology platforms answer the need for effective management of these conditions through more accurate forecasting and ensure measurable, valuable results.

"While healthcare overall remains a significant challenge for employers, rare diseases present an even more daunting one," says Melinda Alba, M.D., chief medical officer, Evidium, a healthcare AI company. "They create a particularly unique challenge for self-insured employers, who are responsible for ensuring the right provision and care for their employees while managing significant uncertainty, all while maintaining the risk. That challenge is compounded because evidence is fragmented across guidelines, studies, and real-world data, and rarely structured in a way that supports transparent clinical and financial reasoning, so that the best clinical/financial decisions can be made."

Dr. Alba points out that traditional analytics struggle when patient numbers are small and care pathways are complex, adding, "That's where a computational approach matters. A technology platform should structure trusted medical evidence - including guidelines, published studies, and real-world observations - into computational models that represent clinical states and potential future pathways, all explicitly traceable back to the underlying evidence."


Arnav Saxena

Rather than treating rare disease as a black box, Dr. Alba says the technology platform should enable benefit leaders and their advisors to explore what is known, what is uncertain, and how different evidence-based scenarios affect utilization, treatment timing and cost exposure over time.

Arnav Saxena, Machine Learning (ML) Engineer, Evidium, explains, "Self-insured stakeholders are often forced to make decisions without clear visibility into the prevalence of rare conditions within their employee population, how these conditions typically progress or where costs may concentrate. By integrating structured medical knowledge with claims and other data, the right platform helps stakeholders to reason more clearly about risk and make better-informed decisions."

He says the result is a shared, traceable foundation for decision-making - one that supports more informed planning for rare disease therapies, including high-cost interventions, while aligning financial stewardship with responsible care.

Dr. Alba and Saxena invite self-insured employers to ask themselves these questions:

1. Are you familiar with the definition of rare disease in your employee population?
2. Do you know how many of your employees or their family members face the challenges of a rare disease?

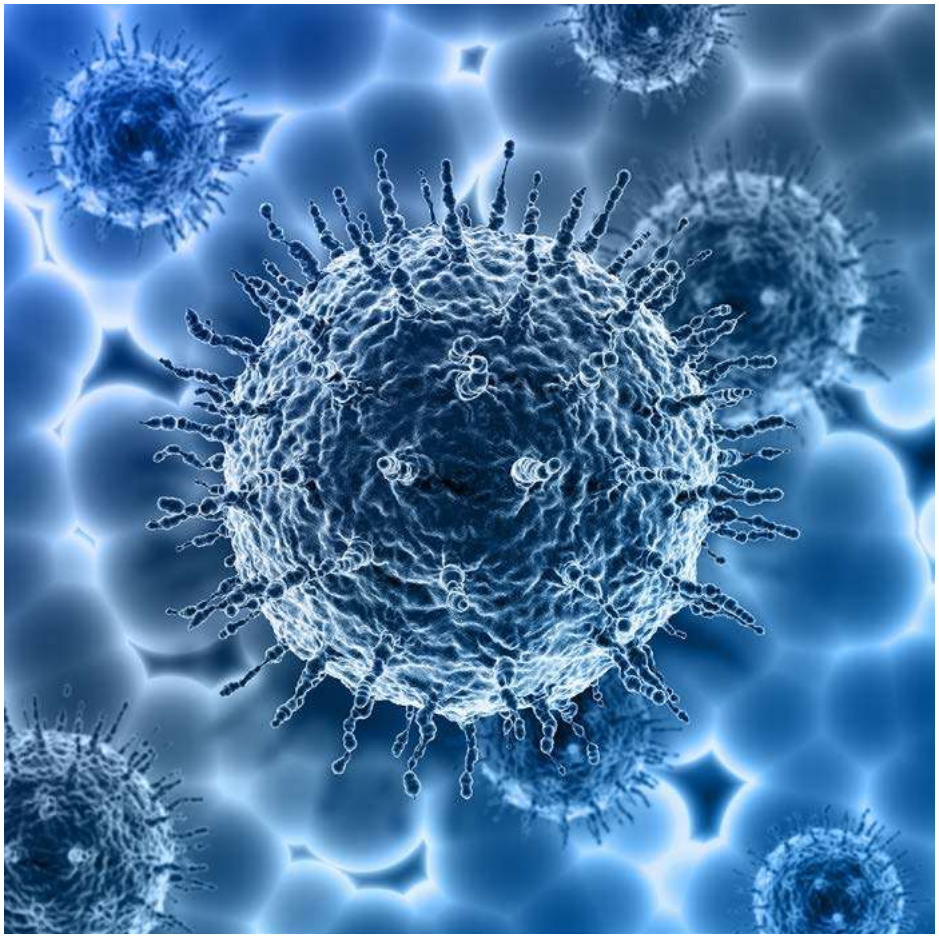
3. Are you addressing the education, utilization and coverage of therapies for rare diseases?

4. What impact do you anticipate rare diseases will have on your 2026 budget and beyond?

"To navigate this landscape, benefit and industry leaders are emphasizing real, data-driven strategies and evidence-based support to manage costs while doing the right thing for their employees over a longer period of time," Saxena continues. "While there is trusted medical evidence for rare diseases, it's often harder to find."

Together with their colleagues at Evidium, Dr. Alba and Saxena recognized the need for an AI platform based on computational knowledge and transparent reasoning to operationalize medical knowledge computationally and enhance the reliability of AI.

"Small patient numbers and low prevalence of rare disease mean there is greater reliance upon registries, case studies and real-world data (RWD) alongside traditional trials," they state. "This is precisely where the introduction of a computational platform transforms reliable medical evidence into precise, transparent and actionable intelligence that plan sponsors can trust. It is particularly valuable for rare diseases because the platform helps close the knowledge gap. A shared knowledge foundation that can be utilized by all stakeholders helps improve not only clinical insights but also the prediction of disease progression."



Self-insured stakeholders are often left in the dark regarding the rare disease patient journey. A computational platform that ingests trusted sources of medical evidence, including clinical guidelines, published studies and care pathways specific to rare conditions and complex diseases, helps to drive better clinical and financial outcomes. It also enables data integration, allowing for the construction of dynamic, patient-specific models of current health states and future trajectories for rare disease progression, along with potential treatment pathways and associated financial impacts. Empowered with this robust ability, organizations can share transparent results and reasoning across their teams and advisors.

"For the first time, employers will no longer be hamstrung by simply guessing about what comes next for their employees and members facing the challenges of rare disease," they conclude. "When it comes to risk management for rare disease, with probabilistic

forecasting and associated costs, there is now a transparent, traceable source of evidence-based medical and clinical knowledge, normalized, shareable and ready to power more precise predictions."

AI COULD ENABLE FASTER RARE DISEASE DIAGNOSIS

David Talby, PhD, MBA, CTO of John Snow Labs, advises that rare disease symptoms often look like something ordinary – until years later. While the evidence was there, it was just scattered across years and specialties. He says rare diseases are not invisible, but the healthcare system often sees them through a keyhole:

Each visit is a snapshot. A lab result here, a symptom note there, maybe an MRI five years later – but never viewed together.

Different data lives in different systems. Your labs might be at one hospital, your imaging at another, and your genetic report in a lab portal no one checks twice.

The "common first" mindset. Because rare diseases are, by definition, rare, the odds seem stacked against them, until you realize those odds reset every time a new doctor starts from scratch.

What he suggests is a longitudinal, multi-modal patient record – one that brings together every clue across the years: blood tests, imaging reports, pathology results, doctor's notes, even data from wearable devices. A unified, longitudinal approach doesn't just speed up diagnosis — it changes, and in some cases saves,

lives. It gives clinicians the full canvas instead of a handful of puzzle pieces. And if it works as intended, it helps more patients hear their doctor say: "We finally know what this is and we can treat it."

An AI-powered vision:

Connects the timeline. Pull together labs, imaging, procedures, and doctors' notes into one unified patient journey.

Teaches the system what to look for. Build a digital "fingerprint" library of rare diseases – combinations of features that tend to cluster together over time.

Blends rules with learning. Some patterns come straight from medical literature ("bilateral carpal tunnel before 60 plus thickened heart walls – think ATTR"). Others can be learned automatically by algorithms scanning thousands of historical cases.

Keeps the human in the loop. Doctors still make the final call, but now they're alerted to patients who deserve a second look.

GENETIC TESTING FOR RARE DISEASES

Employers are inundated with opportunities to conduct genetic testing as a wellness benefit, potentially identifying an individual's genetic mutation and likelihood of developing a rare disease. However, employers should be aware of major ethical issues that may raise concerns for privacy breaches, data security and potential distrust about misuse of information.

Source: Cousin Tree – chart illustrating the different types of cousins, including genetic kinship marked within boxes in red, which shows the actual genetic degree of relationship (gene share) with 'self' in percentage (%).

As with any workplace diagnostic initiative, genetic testing should not be done merely to satisfy patient curiosity – it should only be conducted when medically advised and clinically efficacious. Data from Business Group on Health's 2026 Employer Health Care Strategy Survey reveals that 57% of surveyed employers already cover genetic testing based on family history for certain diseases, and another 7% are considering doing so for 2027/2028.

There are always issues associated with genetic testing, such as increased costs, unnecessary follow-up tests and procedures, undue mental hardship for patients and a potential for limited actionability. Employers should be judicious about coverage of genetic testing and insist upon evidence-based utilization for members who can benefit from it and receive appropriate navigation support.

Employers can select the appropriate vendor to manage various aspects of genetic testing, including their pharmacy benefit managers (PBMs), lab partners and some third-party partners such as lifestyle support programs and condition-specific solutions. Rather than making coverage decisions based on test categories for all genetic tests, it is a better course to determine the clinical utility of individual tests and evidence-based treatment guidelines. An important directive is to be aware of new-to-market, diagnostic genetic tests and remain mindful of prior authorization and utilization management requirements.

Finally, employers must also navigate the Genetic Information Nondiscrimination Act (GINA), which protects employees from discrimination based on genetic information and strictly limits how employers can acquire and use such information.

Genetic Testing Glossary of Terms

To make sound decisions about genetic testing, it is helpful to have some background knowledge.

- Genes: The basic unit of heredity transferred from parents to offspring, present in all cells.
- Genetics: The study of heredity and variation in characteristics of people based on their heredity.
- Genome/Genomics: The complete set of genes in an organism and the study of these genes.
- Genetic Testing: Analysis of a particular gene, or set of genes, which may indicate the presence of or increased likelihood of developing a disease.
- Pharmacogenomic (PGx) Testing: A type of genetic test that is used to predict an individual's response to a given medication or therapy.
- Biomarker: A measurable indicator of a certain biological process or condition, often linked to an individual's genes, which can be used by providers to help diagnose certain health conditions, track disease progression and predict the effectiveness of a particular treatment approach.
- Precision Medicine: Medical care that focuses on identifying approaches that would effectively treat a particular patient based on their individual genetic, environmental and lifestyle factors.

Sources: National Library of Medicine

Workplace genetic testing: which employees are likely to participate, what are their concerns with employer sponsorship, and which design features could reduce barriers and increase participation? - PMC

FINANCIAL IMPLICATIONS OF GENE THERAPY FOR RARE DISEASE

This year, employers can expect to learn of rapid advancements in gene therapy for rare diseases with more approvals, better diagnostic tools and increased collaboration for accessible, single-intervention, potentially life-saving cures. As of early 2025, the FDA has approved over, and industry experts anticipate 30-50 additional cell and gene therapy approvals by 2030. Forecasts from the National Bureau of Economic Research project that by 2026, annual spending on gene therapy products and patients in the U.S. is estimated to reach $25.3 billion.

Rare diseases are typically also ideal candidates for gene therapy research and development, as approximately 80% result from a single genetic mutation, according to American Gene Technologies. By correcting or compensating for this mutation, gene therapy has the potential to provide long-term, curative treatment, often with a single administration.

Source: Kaiser Permanente Business

At the end of 2025, for example, the notable approval of Waskyra for Wiskott-Aldrich Syndrome generated widespread awareness of increased research funding and faster regulatory pathways for personalized genetic medicines. New drug delivery systems are fueling precision editing for previously untreatable genetic conditions.

It's also worth noting that genetic testing of newborns may result in increased prescribing of gene therapies. In mid-December 2025, the Department of Health and Human Services added.

Two rare genetic disorders, Duchenne Muscular Dystrophy and Metachromatic Leukodystrophy, were added to the federal newborn screening list to enable early treatment. As a result, employers should not be surprised by requests for coverage of ELEVIDYS, a prescription gene therapy used to treat ambulatory and non-ambulatory people with Duchenne muscular dystrophy who are at least 4 years old and have a confirmed mutation in the dystrophin gene.

Every day, employees may hear about new lifesaving treatments for their own or a family member's rare disease. This is largely the result of multiple pharma companies collaborating with regulatory agencies to use a common approval pipeline to fast-track approval of therapeutics. Late last summer, a rare and aggressive type of brain cancer that often becomes fatal a year after its diagnosis in children and young adults now has its first FDA-approved therapy, a once-weekly capsule of the drug Modeyso. An estimated 2,000 patients in the U.S. are affected by this cancer each year.

Managing the costs of gene therapies - which can run into millions of dollars – remains the primary challenge to ensuring treatment accessibility. An analysis of 230+ health benefits executives from health plans, employers and unions conducted by the Pharmaceutical Strategies Group (PSG) reveals that more than 70% of employers and health plans expect affordability of gene therapy for their health plan members and workers will be a "moderate or major challenge" over the next 2 to 3 years.

Despite these results, most responders express low confidence in their understanding of the financial impact, and nearly 40% don't currently rely upon any financial protection product to manage their financial risk related to cell and gene therapies.

Cost of Rare Diseases

- The total indirect and non-medical cost of rare diseases is estimated at $548 billion annually: $64 billion for children and $484 billion for adults.
- Absenteeism accounts for nearly $150 billion (27%), followed by presenteeism ($138 billion, 25%) and forced retirement ($136 billion, 25%).
- For adults, caregiver absenteeism costs match those of individuals with rare diseases ($64 billion vs. $60 billion), while for children, caregiver absenteeism costs may exceed those of the affected child ($89 billion vs. $60 billion).
- Broader impacts on quality of life when assessing the value of a gene therapy beyond what is included in traditional health technology value assessments: Parents or caregivers may be able to return to work, reducing lost productivity and increasing workforce participation.
- Healthcare system savings can be substantial due to reduced hospitalizations and long-term care needs.
- A lifetime of care for hemophilia (~67 years) can cost ~$12M.

Source: American Society of Cell and Gene Therapy; Journal of Molecular Therapy

ROLE OF STOP-LOSS

However, a growing number of plan sponsors are turning to their stop-loss partners for protection from unknown financial risk. BCS characterizes stop-loss for gene therapies as a standalone product that can be a great fit for larger employers above 3,000 employees that do not buy stop-loss coverage but want to be shielded from the frequency of expensive CGTs. For small to mid-size groups of 101 or more employees, they say gene therapy stop-loss sits alongside a traditional stop-loss policy, carving out the gene therapy ingredient cost risk from the traditional stop-loss policy.

A recent Mercer survey paints a different picture. When asked about gene-therapy-only stop-loss policies for employers that don't otherwise purchase stop-loss, just 1% of survey respondents with 5,000 or more

employees purchase GT-only stop-loss. Mercer analysts conclude that, given the current low incidence of gene therapy claims, they may not feel compelled by the GT-only stop-loss products currently available. But this may change as more gene therapies reach the market.

Finally, Vickram Pradhan at Sopris Capital advises that stop-loss premiums are already increasing at a ~10% CAGR, and that's with only 50-60% of stop-loss plans covering the few dozen approved CGTs. He says premium inflation could accelerate if 1) CGT coverage expands across carriers and/or 2) CGT approvals proliferate.

As reimbursement uncertainty continues with newly approved CGTs, many stakeholders are optimistic that meaningful changes can be made. One such change is in the development of innovative payment models, which 79% of the survey respondents thought would improve reimbursement and access challenges. In fact, implementing strategic initiatives between payers and manufacturers was a top development in a survey conducted by Cardinal Health.

Increased adoption of high-trust centers of excellence for specific treatments is becoming especially relevant when there are geographic barriers to accessing care. Milliman advises that less than half of patients with sickle cell disease have a treatment center in the region where they live.

Source: Kaiser

Regardless of which payment model or approach a stakeholder selects, there is a need to reexamine existing underwriting and cost containment tools to manage CGT expenses. Plan sponsors should also be aware of the trials and tribulations of certain approved gene therapies that have not met expectations.

AT ISSUE: MEMBER ACCESS

Portability

For gene therapies in the rare disease space, patient portability is a key concern as individuals frequently change jobs with concomitant changes to their insurance plans and coverage. Plan eligibility through a family member differs, and purchasing plans on the marketplace is proving to be a challenge with regulatory changes. Therefore, an employer is unlikely to receive the full benefit of reduced total cost of care for covering a one-time gene therapy, an issue that is exacerbated due to the upfront cost and the expectation of health system savings over a patient's lifetime.

Prevalence

Physicians at Kaiser Permanente/Georgia advise that even if a company has 1M employees, they are likely to only have a couple of people each year who would be considered for gene therapy. But the cost of a single employee or an employee's family member who needs gene therapy could be substantial. Astute employers must consider strategies around gene therapies that can protect their employees and their plan's financial performance.

Restrictive Coverage

Plan sponsors determine coverage policies using many different data sources, including clinical criteria recognized by the Centers for Medicare and Medicaid Services (CMS), clinical trial data and FDA prescribing information. Some adopt narrow clinical trial inclusion/exclusion criteria that were represented in the pivotal clinical trial vs. using the FDA-approved indication.

Varying Value-based Payment Models

Innovative payment arrangements impact the requirements for self-insured companies to cover the entire upfront cost of a gene therapy. This includes installment plans, subscription agreements, outcomes-based agreements (OBAs), and warranties. Mortgage models offer another approach, amortizing costs over time with subsequent insurers assuming payments if patients change plans. Tangential to all these arrangements is careful coordination of health services, medical management, healthcare data and specialty pharmacy expertise to provide a holistic approach to care management for rare diseases.

Many companies are also utilizing portability mitigation strategies: risk-pooling strategies, where multiple payers contribute to a shared fund for high-cost therapies, and can distribute financial burdens more equitably. These models can incorporate portability mechanisms, allowing the initial payer's investment to follow the patient.

Inconsistent Payment Methodologies

The complex patchwork of payment systems, varied billing practices and reimbursement methods for rare disease gene therapies that vary by administration route requires extensive time and resources for members to navigate. These often pose unnecessary administrative barriers to accessing timely treatment and should be removed when possible.

Member Support Programs

Federal safe harbors reduce financial challenges for patients and their families, alleviating significant barriers to accessing these potentially curative but logistically complicated therapies. The creation of these provisions provides legal certainty that employers can allow drug developers to offer limited support programs, such as lodging or transportation, to patients and caregivers who must travel to specialized treatment facilities.

Travel for Treatment

Stem cell tourism has emerged as a fast-growing global phenomenon, with patients traveling across borders and potentially thousands of miles to seek treatments that may not be available or approved in the US. This form of medical tourism is particularly appealing to those suffering from chronic or terminal illnesses where conventional therapies have failed.

Employees may be lured to stem cell clinics worldwide. However, many authorities, such as the Harvard Stem Cell Institute (HSCI), warn against this option since some of these clinics offer unproven treatments with promises of miracle cures for conditions ranging from Parkinson's disease to spinal cord injuries. While this choice offers hope, it also raises significant ethical, medical and regulatory concerns. There are some reports that these unproven treatments carry substantial risks, including tumor formation, tissue rejection, infection, permanent disability and death.

According to the HSCI, in the U.S., stem cell treatments are tightly regulated, and the only FDA-approved therapies are hematopoietic (blood) stem cells from cord blood for blood cancers and immune disorders, plus several specific CAR T-cell therapies (genetically modified immune cells) for certain cancers. But many other uses, such as for arthritis or Parkinson's, are still experimental, with the FDA cracking down on unapproved "regenerative medicine" clinics. Instead of encouraging travel for unproven treatments, employers are well-advised to focus on providing coverage for FDA-approved CGT within their existing health plans.

Moreover, there are legal and compliance issues for employers. For example, an employer could face significant legal risks if an employee suffers harm or complications from unapproved treatment in a foreign country. Furthermore, attorneys at Alston & Bird advise that offering medical travel benefits, even for a specific treatment, could cause the employer's health plan to be classified as a group health plan under the Employee Retirement Income Security Act (ERISA) and the Affordable Care Act (ACA). This could trigger complex compliance requirements and potential excise taxes for non-compliance.

GO-FORWARD COVERAGE DECISIONS

Coverage decisions for CGTs should not rest solely upon the current absence or prevalence of rare diseases in the workforce. By integrating CGTs into your health plans, employers can potentially provide:

1. Access to new, transformative treatment options: For certain diseases, when no other treatments exist, or current options are costly and only temporarily manage symptoms.

2. Slowed or halted disease progression: In some cases, CGTs may be able to halt disease progression entirely by addressing its underlying genetic cause.

3. Reduced long-term healthcare costs: With the right price point and benefit structure, CGTs have the potential to prevent costly lifelong medical interventions and may offset the costs of long-term treatment.

4. Quality-of-life improvements: CGTs may offer improved function, reduced or eliminated pain and suffering, provide a psychological sense of well-being, and reduce strains on time and caregiving.

"Sun Life's latest high-cost claim and drug trend analysis shows million-dollar claims have increased 29% over the prior year and 61% over the past four years, driven not just by cost but by rising frequency," says Robert McCollins, chief community organizer, Employers Healthcare Alliance (EHA), a non-profit managed 'employer built by employers for employers and their work families.' "The question for employers is no longer if a rare disease will impact their workforce, but when."


Robert McCollins

McCollins, who leads the EHA to provide support for HR/Business Professionals who desire the best healthcare for their workplace through a peer community, educational programs and actionable resources and solutions that are easy to implement and use, attributes the success to optimizing outcomes requiring unencumbered access to data, early identification and intervention.

"This will only be accomplished with a benefits support team of the advisor, TPA, PBM, clinical risk managers and data analytics partners that are all aligned to support the patient and employer," he continues. "Strategies such as centers of excellence, charitable and government programs with roll-up your sleeves, hands-on clinical advocacy can dramatically change outcomes."

In one case, his team was able to involve a clinical expert in a particular rare disease to reduce a $1.7M patient drug spend by over $1M, providing access to a disease-specific center of excellence for enhanced care and oversight for the patient.

Source: Tufts University NEWDIGS

RISKS OF EXCLUDING COVERAGE

While the prospect of a multi-million-dollar expense can be a deterrent to coverage, there are stop-loss policies to protect a plan by transferring the risk of high-cost claimants. Moreover, the downside of not covering CGTs carries many risks, primarily compliance and negative public relations.

Lockton maintains that excluding coverage can trigger a disability-based discrimination claim, even if the exclusion is targeted at an employee's dependent. If a plan amendment adding the exclusion is installed midyear because of an existing claim or impending claim, the amendment risks a HIPAA violation.

The advisory firm further states that if a self-insured employer chooses to exclude gene therapy, and then decides to make an exception to cover the treatment gene therapy due to extenuating circumstances (negative publicity, child of an executive needs the treatment, etc.), the claim will not be eligible for stop-loss reimbursement because the service is not listed as covered in the underlying health plan.

Therefore, it is critical to align the terms of the underlying health plan and the stop-loss coverage to ensure stop-loss reimbursement of claims related to these expensive treatments. Some carriers/claims administrators will not allow excluding some or all gene therapies.

The consequences of exclusions may also result in adverse publicity since these treatments are often viewed as essential, particularly for children. Imagine the adverse publicity implications for not covering FDA-approved gene therapy for a child with a life-threatening condition with limited, if any, treatment options.

NEXT UP: COMMON CHRONIC DISEASES

Employers must be prepared for every eventuality since new technology is poised to bring CGTs to common chronic diseases. While expenses for treating not-so-common rare diseases were taxing health plan budgets, these new treatments targeting more common conditions, which are certainly "lurking" in the workforce, may become even more financially challenging.

New research from Penn Medicine reveals a safe delivery system of therapeutic DNA to cells, which could transform the treatments for common chronic diseases like heart disease, diabetes and cancer. This breakthrough overcomes the harmful, adverse effects of past attempts to treat chronic disease.

- In the pipeline, there are several gene therapies for various cardiovascular indications, including congestive heart failure, with some expected to file for Biologics License Applications this year.
- Gene-targeting approaches for Alzheimer's disease and related dementias are a major area of research focus, with grant programs specifically funding early-stage human trials for 2026 and beyond. A gene therapy for Huntington's disease is also showing promise in clinical trials.
- CGTs are already a significant part of cancer treatment, and there is continued expansion into solid tumors and new immunotherapies.
- Executives at Breakthrough T1D, formerly the Juvenile Diabetes Research Foundation, believe that novel cell therapies will transform T1D management and make them a reality.

Diabetes is certainly a key target, so be on the lookout for really promising treatments. Novo Nordisk and Aspect Biosystems recently announced they are entering a new phase of their partnership to develop transformative cell therapies toward clinical development and potentially generate a functional cure for people living with diabetes.

While this development could take years, it could be a gamechanger for employers. BSwift benefit administrators report that U.S. companies incur$413 billion annually in diabetes-related costs alone, and the yearly healthcare cost for an employee with diabetes is $20,000–six times higher than someone without. Roughly 6.4% to 8.2% of the employed U.S. adult population has diabetes.

As costs continue to rise, this annual figure is predicted to rise 20% year-over-year. Comorbid conditions like obesity, musculoskeletal (MSK) pain and depression often co-occur with diabetes, making health management more complex and further raising the stakes for workforce health management.

As with all new treatments, employers should be skeptical of products or therapies that promise a guaranteed cure for diabetes or any other condition. Any genuine cure will have undergone rigorous clinical trials and received approval from regulatory bodies, like the FDA. It is always prudent to consult with a healthcare professional before making significant changes to plan coverage. ■

About the Author

Laura Carabello holds a degree in Journalism from the Newhouse School of Communications at Syracuse University, is a recognized expert in medical travel and is a widely published writer on healthcare issues. She is a Principal at CPR Strategic Marketing Communications. www.cpronline.com