Gene & Cell Therapy

Transforming health care at the foundational level

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What are gene and cell therapy?

While they are similar in principle, gene and cell therapy work in different ways.

- Gene therapy involves modifying or manipulating genes that cause specific medical conditions. This could include replacing defective genes with healthy copies, inactivating disease-causing genes that are not functioning correctly, or introducing new or modified genes to treat a specific disease or condition.
- With cell therapy, cells are modified and reintroduced to the body for medicinal purposes such as boosting immunity or regenerating diseased tissues.

While health care innovation is nothing new, the pace at which science and technology are changing medicine today is unparalleled. Nowhere is that more evident than in the areas of gene and cell therapy.

These cutting-edge medical advancements provide opportunities for life-changing treatment for many conditions. And while costly up-front, the long-term benefits of these therapies generally pay for themselves over a recipient's lifetime.

These therapies present remarkable opportunities for not only treating—but potentially curing—major health conditions. Although only two gene therapies have been approved by the Food and Drug Administration (FDA) in the U.S. to date, many cell therapies are currently approved by the FDA for conditions including multiple myeloma, lymphoma, melanoma, retinal dystrophy, cartilage defects, major burns, prostate cancer and more. That number will likely increase significantly over the next five years.

What are the costs?

Because these therapies involve highly specialized research and development, and are not mass-produced but customized to individual needs, the cost of treatment is very high.

For example:

- Zolgensma, a gene therapy for children under 2 with a diagnosis of spinal muscular atrophy, has an approximate cost of $2.1 million for a single shot.
- Chimeric antigen receptor T-cells (CAR-T), a cell therapy for treating certain types of leukemia, costs between $350,000 and $2 million per dose.

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To keep these numbers in perspective, however, you must consider their success in treating and curing expensive conditions in a short timespan, rather than treating conditions continuously for a patient's potential lifetime.

In other words, if you compare the initial $2.1 million cost of Zolgensma to a lifetime of treatment for a child born with spinal muscular atrophy, the initial investment becomes much more reasonable.

What's the impact?

The growth of cell and gene therapy will have implications for many as the research advances into more health conditions. While limited in scope for now, these therapies already affect health care consumers and facilitators in increasing ways.

For individual health care consumers, cell and gene therapy is a reason for optimism. Life-altering conditions are already being treated—and sometimes cured—with these therapies, and more treatments are on the way. Many states have passed legislation to encourage genetic testing at birth and identify candidates for treatment early on. Early intervention via fetal testing is also recommended, but only certain tests are currently covered.

For employers, getting over the initial shock at the cost of these therapies may take some time, but the benefits of alleviating a lifetime of costly condition management will become evident. Employers, brokers and stop loss carriers will need to work together to manage, mitigate and strategize on the risk of these costs. These costs are likely to affect policyholder renewals and the entire industry.

Cost-saving opportunities

As cell and gene therapy touch more and more health care consumers, partnering with stop loss carriers who closely and proactively monitor their costs and effectiveness can help employers determine the best approach for managing the expense risk. To alleviate some concerns, proposals are underway for manufacturers to offer performance guarantees for the treatments, so cost-sharing may be possible if a condition isn't resolved.

Therapy manufacturers also offer multiyear payment options, but the challenge is that health care plans are renewed annually. If an employer changes carriers or an employee leaves the company, the long-term financial benefit of an initial high-cost treatment becomes more complicated.

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Employer considerations

For employers who self-fund their medical plan, incorporating gene and cell therapy into their plan document is essential. When working with their stop loss carrier, employer considerations should include:

- Does the plan document cover genetic testing?
- Are all cell and gene therapies approved in the U.S. covered under the plan?
- Can covered participants travel to other countries to receive treatments not approved in the U.S.?
- Are travel costs for cell and gene therapy covered by the employer?

More questions will inevitably arise as cell and gene therapy progresses. A qualified stop loss carrier may be your best resource for keeping up with these advances so you can focus less on the cost and more on the benefits these therapies can provide to employees and their families.

To learn more, contact your stop loss representative.

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