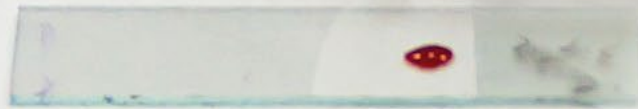


# INNOVATIVE STRATEGIES FOR INTRODUCING AND PROVIDING COVERAGE FOR CELL & GENE THERAPIES



Written By Laura Carabello



**F**or the first time, self-insured group health plan sponsors and leading manufacturers of cell and gene therapies (CGT) will come together during a SIIA-hosted Forum, May 31 – June 1, 2023, that is solely dedicated to discussing the introduction, cost and coverage options for these potentially lifesaving drugs.

Many ask the question: what are these therapies and what makes them different from drugs that are already in the market? Demonstrating that the therapies are providing a lifelong cure and not just another type of ongoing treatment will be key to justifying the pricing and gaining acceptance from patients, providers and employers/payers.

Gene therapy does not treat symptoms – rather it targets the cause, the genetic defect behind a disease. Faulty genetic code may be replaced by inserting a gene that’s missing. Sometimes this can occur in a petri dish and the healthy cells are transferred to the patient. Alternatively, a vector, usually a virus, delivers the genetic material to the patient’s cells.

Treatment is currently restricted to those diseases caused by a single gene mutation and primarily target conditions that are typically rare, with patient populations in the hundreds or low thousands. But the landscape is rapidly changing and therapies for more common conditions, like sickle cell disease, are expected to arrive.

At the upcoming meeting, attendees will benefit from in-depth explanations.

As background, here are some brief definitions:

Cellular therapy (CT) is the transplantation of human cells to replace or repair damaged tissue and/or cells. With new technologies, innovative products, and limitless imagination, many different types of cells may be used as part of a therapy or treatment for a variety of diseases and conditions. Some of the cells that may be used include

hematopoietic (blood-forming) stem cells (HSC), skeletal muscle stem cells, mesenchymal stem cells, lymphocytes, dendritic cells, and pancreatic islet cells.

*Source: Association for the Advancement of Blood & Biotherapies*

## **GENE THERAPY**

Gene therapy seeks to modify or manipulate the expression of a gene or to alter the biological properties of living cells for therapeutic use. Gene therapy is a technique that modifies a person’s genes to treat or cure disease. Gene therapies can work by several mechanisms:



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- Replacing a disease-causing gene with a healthy copy of the gene
- Inactivating a disease-causing gene that is not functioning properly
- Introducing a new or modified gene into the body to help treat a disease

Gene therapy products are being studied to treat diseases including cancer, genetic diseases, and infectious diseases. Gene therapy products are biological products regulated by the FDA's Center for Biologics Evaluation and Research (CBER). Clinical studies in humans require the submission of an investigational new drug application (IND) prior to initiating clinical studies in the United States. Marketing a gene therapy product requires submission and approval of a biologics license application (BLA).

*Source: US Food and Drug Administration*

Here's the flip-side of sticker-shock over the cost of therapies: some call CGTs a relative bargain since they have, in some cases, the potential to cure illness with a single dose. Many regard this as a reprieve from the physical, emotional and financial burdens of living with a serious disease that often requires ongoing and highly expensive treatments.



## SPOTLIGHT ON CANCER

The Business Group on Health named cancer as the top driver of employer healthcare costs, a spot previously held by musculoskeletal conditions. In fact, 13% of employers surveyed say they have seen more late-stage cancers impacting their workforce, and 44% predict they will see an increase in cancer diagnoses in the future.

Researchers point to medication as the biggest expense associated with cancer care and that costs for breast, lung, lymphoma and colorectal cancers incurred the most expenditures. For example, Zynteglo, a gene therapy for a genetic blood disorder, debuted at \$2.8 million.

CGTs harness the power of a patient's own immune system to destroy cancer cells without harming healthy tissue. They work by changing the DNA within an individual's existing cells to give those

cells a new 'set of instructions' that can help them find and fight cancer. This is called genetic engineering, such as CAR T cells.

In laymen terms, CAR T-cell therapies genetically engineer DNA in an individual's T cells (which are part of that person's immune system) using chimeric antigen receptors (CARs). This enables the T cells -- a natural and essential part of a person's immune system -- to detect and destroy cancer cells. Today, the first-ever FDA-approved CAR T-cell treatments for blood cancers are saving lives.



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Different kinds of cancers have different kinds of biomarkers, which means different kinds of CGTs are required to fight them. They are personalized to attack specific cancer cells for specific patients.

Source: *Alliance for Cancer Gene Therapy*

### SPOTLIGHT ON HEMOPHILIA

According to the CDC, hemophilia is usually an inherited bleeding disorder in which the blood does not clot properly. This can lead to spontaneous bleeding as well as bleeding following injuries

or surgery. Blood contains many proteins called clotting factors that can help to stop bleeding.

People with hemophilia have low levels of either factor VIII (8) or factor IX (9). The severity of hemophilia that a person has is determined by the amount of factor in the blood. The lower the amount of the factor, the more likely it is that bleeding will occur which can lead to serious health problems.

In rare cases, a person can develop hemophilia later in life. The majority of cases involve middle-aged or elderly people, or young women who have recently given birth or are in the later stages of pregnancy. This condition often resolves with appropriate treatment.

Hemgenix, a gene therapy that was launched at a cost of \$3.5 million for a one-time infusion, treats adults with hemophilia B, a genetic bleeding disorder in which people do not produce a protein needed to create blood clots.

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About 1 in 40,000 people have the disease, most of whom are men. Typical treatment requires routine intravenous infusions to maintain sufficient levels of the missing or deficient clotting factor, but the new gene therapy is meant to be a one-time IV infusion.

This new treatment option for patients with Hemophilia B represents important progress in the development of innovative therapies for those experiencing a high burden of disease associated with this form of hemophilia. In a recent cost-effectiveness analysis of the drug, weighing health benefits against offset costs, the Institute for Clinical and Economic Review (ICER) suggested that a fair price for the drug to be between \$2.93 million and \$2.96 million.

But the pushback to the high cost of current therapies for hemophilia is already underway. As pharmacy benefit managers (PBMs) increasingly exclude drugs from their formularies, advocates for patients with hemophilia are concerned about the drugs for bleeding disorders that BlueCross BlueShield of Tennessee (BCBST) has dropped from its 2023 formulary.

Also, be on the lookout for Roctavian for hemophilia A which may be approved by the FDA in July 2023 and is likely to be priced to cost between \$2 million and \$3 million per treatment.

### MANAGING HIGH COSTS OF GENE THERAPIES

It should be noted that while gene therapies have the potential to treat or even cure life-limiting diseases, these advancements come at an enormous price tag of \$850,000 to \$3+ million per patient. When Hemgenix for the treatment of adults with Hemophilia B hit the market in November 2022, CNN reported that Hemgenix became the world's most expensive drug at a \$3.5 million price tag.

With FDA approval and commercial launch of novel CGT therapies, a bevy of these treatments for rare disease and complex conditions are now on the market. Ushering in a new era of medical care, these super-expensive drugs are challenging the acumen of even the most experienced benefits decision-makers and risk strategists.

This is largely because products of this magnitude and cost have never before been available, leaving brokers, advisors, TPAs, stop-loss carriers or other service providers thirsting for information and credible guidance that they can offer to their employer-clients.

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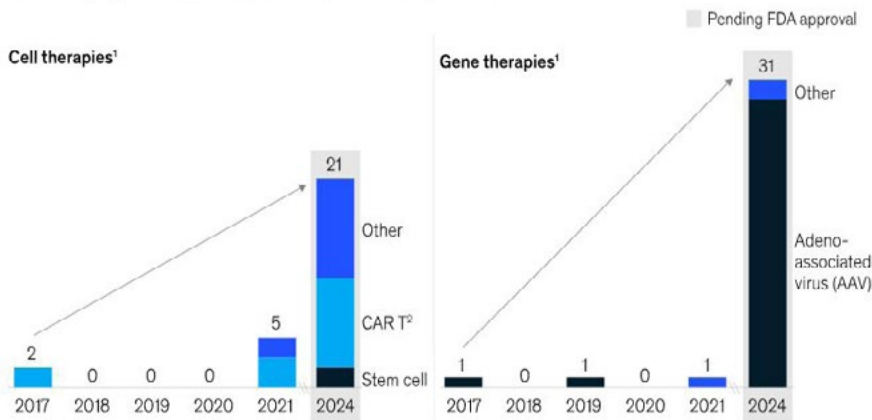
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As background, here is a snapshot of past and projected launches:

We are at a critical inflection point in the commercialization of cell and gene therapies.

Past and projected launches in the United States, 2017–24



<sup>1</sup>A database of the therapies referenced can be found at "Approved cellular and gene therapy products," US Food & Drug Administration, accessed July 28, 2022. The data used here exclude COVID-19-related products and cord blood.  
<sup>2</sup>Chimeric antigen receptor T-cell therapy.  
 Source: Evaluate Pharma August 2022 data, Evaluate I tri; US Food and Drug Administration; McKinsey analysis

Attendees at the upcoming meeting will not be disappointed in the scope and depth of data, material content and evidence that will be presented by some of the most credible sources in the industry.

**SIIA Cell + Gene Therapy Forum**

May 31 – June 1  
 JW Marriott Minneapolis  
 Mall of America  
 Minneapolis, MN

**LEARNING OBJECTIVES**



Working with Liz Middtlen, Chairwoman, SIIA Board of Directors, Dr. Surya Singh, chief medical officer, Emerging Therapy Solutions, oversees the program development and chairs the opening panel which presents the manufacturers' perspectives.

Providing an overview of the event, he says, "As the first event of its kind, manufacturers will engage with this audience and

Source: McKinsey and Company. <https://www.mckinsey.com/industries/life-sciences/our-insights/eight-imperatives-for-launching-cell-and-gene-therapies>

As of this writing, as many as 13 brand new cell or gene therapies could be approved for use in the US, Europe or both by the end of 2023. This puts us within reach of the FDA's well-publicized 2019 prediction that it would approve 10-20 new cell and gene therapies a year by 2025. Please review the chart below:

Anticipated regulatory decisions in 2023			
United States		Europe	
<b>Afami-cel (Cell Therapy)</b> Adaptimmune Therapeutics Advanced synovial sarcoma	<b>bb1111 (Gene Therapy)</b> bluebird bio Sickle cell disease	<b>CT-053 (CAR-T Therapy)</b> CARsgen Therapeutics R/R multiple myeloma	<b>lidanacogene elaparovvec (Gene Therapy)</b> Pfizer (formerly Spark Therapeutics) Hemophilia B
<b>HPC cord blood (Cell Therapy)</b> StemCyte Unrelated Donor hematopoietic progenitor cell transplantation	<b>Libmeldy (Gene Therapy)</b> Orchard Therapeutics Metachromatic leukodystrophy (MLD)	<b>Lifileucel (TIL Therapy)</b> Iovance Metastatic melanoma	<b>Omidubicel (Cell Therapy)</b> Gamida Cell Hematological malignancies
<b>Roctavian (Gene Therapy)</b> BioMarin Hemophilia A	<b>SRP-9001 (Gene Therapy)</b> Sarepta Therapeutics Duchenne muscular dystrophy	<b>Tab-cel (Cell Therapy)</b> Atara Biotherapeutics Inc Epstein-Barr virus associated post-transplant lymphoproliferative disorder (EBV+PTLD)	<b>Upstaza (Gene Therapy)</b> PTC Therapeutics Aromatic L-amino acid decarboxylase (AADC) deficiency
<b>B-VEC (Gene Therapy)</b> Krystal Bio Dystrophic epidermolysis bullosa	<b>CTX001 (Gene Editing Therapy)</b> CRISPR Therapeutics & Vertex Pharmaceuticals Sickle cell disease, β-thalassemia	<b>B-VEC (Gene Therapy)</b> Krystal Bio Dystrophic epidermolysis bullosa	<b>CTX001 (Gene Editing Therapy)</b> CRISPR Therapeutics & Vertex Pharmaceuticals Sickle cell disease, β-thalassemia
<b>EtranaDez (Gene Therapy)</b> uniQure & CSL Behring Hemophilia B		<b>Lumevoq (Gene Therapy)</b> GenSight Biologics SA Leber hereditary optic neuropathy (LHON)	

Source: American Society of Cell and Gene Therapy [www.ASCGT.org](http://www.ASCGT.org)

share their knowledge of the CGT pipeline, manufacturer viewpoints on pricing and outcomes, value-based pricing and payment considerations, potential financial mitigation/risk strategies, plan document guidance and more. Panelists will cover key areas of interest that span pending product approvals, patient access considerations and development updates for rare diseases.”

Singh explains the four program pillars:

I. CLINICAL FOCUS

Attendees will benefit from a scientific discussion of CGTs and an insider look at the costs of administering gene therapies – with both types being studied to treat medical conditions like certain types of cancer as well as genetic and inherited disorders.

- Ex-vivo which involves the genetic modification of cells outside of the body to produce therapeutic factors and their subsequent transplantation back into patients. Various cell types can be genetically engineered.
- In-vivo which uses viruses or other methods to deliver genes directly into the body’s cells.

II. PRODUCT PIPELINE UPDATES

2023 is expected to be a pivotal year for the introduction of new gene therapies, marked by the commercialization of Hemophilia A, sickle cell drugs and others as noted in the chart above. The anticipated approval and launch of additional therapies opens up opportunities for much larger populations, with expanded access through hospitals and outpatient facilities. In fact, 50% of hemophilia treatment centers are free-standing and independent of US health systems while the other half are affiliated with a major health system.

III. OPTIONS FOR RISK TRANSFER

Employers need to be resilient as they face the economic headwinds for coverage of CGTs. Amid the struggle to fit these therapies into the traditional benefit design for treatment and reimbursement of drugs to treat chronic diseases, they are prompted to collaborate with manufacturers and others that are developing multiple access pathways. These options for risk transfer are designed to help enable therapies to reach patients.



Current Emerging Models for Risk Transfer

Coverage Guideline	BUCA Programs	Independent Risk Transfer	Risk Facilities	Carrier Specific Programs*	Broker Specific Programs*
Gene Therapy Cost	Approved Gene Therapies	Approved gene therapies plus Spinraza	Approved Gene Therapies	Approved Gene Therapies	Approved gene therapies plus Spinraza
Cell Therapy Cost	No coverage	Abecma & Carvykti	Approved Cell Therapies	No additional coverage outside of standard policy	No coverage
Hospital/Professional Services Cost	No coverage	Coverage for CAR -T administration	Included	Included in standard policy	Included in standard policy
Pipeline Therapies Covered	Unknown, but added Zytiglo immediately upon approval	None	Included	Included as approved	None
Out-of-Network Coverage	None	None	Covered at some level	Covered at higher deductible follows standard policy language	None
Self-funded Groups without Underlying ESL Coverage	None	In development	Available	None	None
Coverage Description	Covers cost of therapy when acquired through affiliated specialty pharmacy	Covers ground up cost of therapy to a limit and cost of administration for CAR-T	Covers cost of therapy & administration including facility and professional fees	Covers via policy at a stepdown deductible with use of specific network	Covers ground up cost of therapy to a limit

\*outlining a specific program, additional programs are on the market or emerging

Source: Emerging Therapy Solutions

IV. FINANCIAL SOLUTIONS

Manufacturers are increasingly turning to innovative payment models that address employer/payer uncertainty across both financial (through discounts, price volume agreements, sales caps, annuities, etc.) and clinical outcomes (through outcomes-based agreements, coverage with evidence development, etc.). Many types of innovative agreements are now being presented and there are several options for manufacturers and employers to fulfill short- and long-term strategic access priorities and meet their distinct needs and expectations.

Attendees will have an opportunity to ask questions, get answers and interact with speakers and participants. This is one event the self-insured community cannot afford to miss.

Visit [www.siiia.org](http://www.siiia.org) to access program information and registration. ■

*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](http://www.cpronline.com)*

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The self-insured community may never have this opportunity again in 2023 to learn how to offer CGTs and cost-effectively bring these potentially lifesaving therapies to employees. Plan documents will require re-structuring with new language that spells out changes. Making coverage determinations that account for risk will also be a concern as Health Plan Sponsors finalize underwriting decisions both from stop-loss and reinsurance perspectives.

