2024 Cell and Gene Therapy Report: **Advancing the Future of Medicine**

McKESSON

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Welcome

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— Joe DePinto
Head of Cell, Gene, and Advanced Therapies
McKesson

The field of cell and gene therapy (CGT) is transforming, driven by scientific breakthroughs and a deepening understanding of genetic medicine.

As we enter this new era of healthcare, McKesson is proud to present the *2024 Cell and Gene Therapy Report: Advancing the Future of Medicine*. This report aims to illuminate the current landscape, future trajectory, and the critical steps necessary to develop and deliver these revolutionary therapies to patients in need.

The last decade has witnessed remarkable advancements in CGT, with therapies that were once considered science fiction now becoming a clinical reality. With dozens of CGTs on the market, we are already seeing adoption of life-changing—and life-saving—medicines. And with thousands more in development, the potential to impact millions of people seems within reach.

Inside this report you will hear from top experts and leaders from across the industry who provide their perspectives on the pain points, success stories, concerns, and opportunities for CGTs. You'll see the results of a research study with 124 oncologists nationwide about their personal experiences referring and treating patients with CGTs, and their views on how CGTs will impact patient care in the future. We also share key learnings and potential future steps the industry can take to aid wider adoption of these medicines.

At McKesson, bringing a paradigm changing class of medicines to patients is a core part of our 200-year history. From the birth of the biotech revolution in the 1980s through today's cutting-edge cell and gene therapies, McKesson's solutions help patients access life-changing therapies. Our team continues to press forward and innovate. Our recent launch of the **InspiroGene** brand, which brings together our premier suite of services to support every stage of CGT commercialization, is just the latest demonstration of our commitment to transforming the future of patient care.

This report is not just a reflection of where we are today but a roadmap for where we need to go. We invite you to join us on this journey to transform the landscape of medicine and advance health outcomes for all.

Sincerely,

Joe DePinto

Head of Cell, Gene, and Advanced Therapies, McKesson

U.S. Pipeline for Cell and Gene Therapy

Exploring the impact of cell and gene therapy on the future of medicine

Over the past decade, cell and gene therapies (CGT) have evolved from a theoretical approach to disease treatment into one of healthcare's most important innovations, offering unprecedented potential to treat, and in some cases cure, a growing number of diseases.

These groundbreaking approaches are reshaping the landscape of medicine and providing new hope for patients with serious and life-threatening conditions.

As exciting as that prospect is, ushering in this new era will require a paradigm change in the healthcare industry.

The rise of CGT has been rapid, and the category is slated to accelerate at an exponential rate over the next decade. Ten years ago, only a handful of CGTs were approved in the U.S.—now, more than 30 are approved.[1] In 2019, former U.S. Food and Drug Administration (FDA) Commissioner Scott Gottlieb predicted that the agency would be approving 10 to 20 cell and gene therapy products a year by 2025.[2]

"Gene therapy products now have the potential to cure intractable diseases, and fundamentally alter the trajectory of many other vexing illnesses," Gottlieb said in a statement at the time.

The importance of CGTs lies in their ability to address the root causes of diseases at a genetic or cellular level. This offers the potential for long-lasting and even curative treatments for a wide range of conditions, from rare genetic disorders to more common diseases and conditions.

Already, CGTs have led to new standards of care in a range of rare diseases and cancer indications. In spinal muscular atrophy, a rare genetic neurodegenerative disorder that affects 1 in 10,000 newborns, children who would typically die before the age of two can now hit normal developmental milestones with a one-time treatment with gene replacement therapy ZOLGENSMA (onasemnogene abeparvovec), approved by the FDA in 2019.[3, 4]

Gene therapies are also changing standards of care in sickle cell disease, a rare blood disorder that impacts more than 100,000 Americans, predominantly of African heritage, which is associated with chronic pain, organ damage, stroke, and shortened life expectancy. Two gene therapies approved in December 2023, Vertex's CASGEVY[5] (exagamglogene autotemcel) and Bluebird Bio's LYFGENIA[6] (lovotibeglogene autotemcel), have demonstrated the ability to modify the disease to eliminate episodes of pain, and may potentially be curative.[7]

"These approvals represent an important medical advance with the use of innovative cell-based gene therapies to target potentially devastating diseases and improve public health," said Peter Marks, MD, PhD, Director of the FDA's Center for Biologics Evaluation and Research, in an FDA press release announcing the approvals.[8]

The potential expansion of CGTs into more prevalent conditions, including cardiovascular disease, diabetes, and neurological disorders, could markedly change health outcomes. But to get there, the industry will need to scale to create capacity for the many products in the pipeline, all of which require specialized facilities, processes, and clinical providers. Stakeholders will also need to address inherent challenges related to manufacturing, delivery, and accessibility—and will need to adopt new models to manage the significant upfront costs of these therapies.

The following section of the report provides a snapshot of where the industry stands today and new developments expected over the next few years. We'll also share interviews with Ellen Moore, Principal Consultant at GlobalData, and John Bishai, Managing Director at Bank of America Securities, Healthcare Sector, who will share their views on the scientific innovations ahead and some of the roadblocks standing in the way of progress.

FDA-approved cell and gene therapies (CGTs)

The FDA approved a record-setting number of CGTs in 2023—but it's just the start of a new era.

Cell Therapies*				
PRODUCT	**COMPANY**	**MODALITY**	**INDICATION**	**APPROVAL DATE**
PROVENGE	Dendreon Corp.	**Cell**	Prostate cancer	April 29, 2010
GINTUIT	Organogenesis Incorporated	**Cell**	Topical application to a surgically created wound bed	March 9, 2012
MACI	Vericel, Corp.	**Cell**	Cartilage defects of the knee	December 13, 2016
KYMRIAH	Novartis Pharmaceuticals Corporation	**Cell**	Leukemia and lymphoma	August 30, 2017
YESCARTA	Kite, A Gilead Company	**Cell**	Lymphoma	October 18, 2017
TECARTUS	Kite, A Gilead Company	**Cell**	Leukemia and lymphoma	July 24, 2020
BREYANZI	Juno Therapeutics, Inc., a Bristol Myers Squibb Company	**Cell**	Leukemia and lymphoma	February 5, 2021
ABECMA	Bristol Myers Squibb & 2SeventyBio	**Cell**	Relapsed/refractory multiple myeloma	March 26, 2021
STRATAGRAFT	Mallinckrodt Pharmaceuticals	**Cell**	Debrided thermal burns	June 15, 2021
RETHYMIC	Enzyvant Therapeutics	**Cell**	Immune reconstitution in patients with congenital athymia	October 8, 2021
CARVYKTI	Janssen Biotech, Inc. & Legend Biotech	**Cell**	Relapsed/refractory multiple myeloma	February 28, 2022
OMISIRGE	Gamida Cell Ltd.	**Cell**	Hematologic malignancies	April 17, 2023
LANTIDRA	CellTrans Inc.	**Cell**	Type 1 diabetes	June 28, 2023
AMTAGVI	Iovance Biotherapeutics, Inc.	**Cell**	Melanoma	February 16, 2024
TECELRA	Adaptimmune Therapeutics	**Cell**	Synovial sarcoma	August 1, 2024

*Cell Therapies includes gene-modified cell therapies and other cellular/tissue-related therapies.
LAVIV is excluded from this list as our research indicates the product is no longer commercially available.

Gene Therapies				
PRODUCT	**COMPANY**	**MODALITY**	**INDICATION**	**APPROVAL DATE**
IMLYGIC	BioVex Inc., a subsidiary of Amgen Inc.	**Gene**	Melanoma	October 27, 2015
LUXTURNA	Spark Therapeutics, Inc. & Novartis Pharmaceuticals Corporation	**Gene**	Biallelic RPE65 mutation-associated retinal dystrophy	December 19, 2017
ZOLGENSMA	Novartis Pharmaceuticals Corporation	**Gene**	Spinal muscular atrophy (SMA)	May 24, 2019
ZYNTEGLO	Bluebird Bio, Inc.	**Gene**	ß-thalassemia	August 17, 2022
SKYSONA	Bluebird Bio, Inc.	**Gene**	Active cerebral adrenoleukodystrophy (CALD)	September 16, 2022
HEMGENIX	Unique and CSL Behring	**Gene**	Hemophilia B	November 22, 2022
ADSTILADRIN	Ferring Pharmaceuticals	**Gene**	Bladder cancer	December 16, 2022
VYJUVEK	Krystal Biotech, Inc.	**Gene**	Dystrophic epidermolysis bullosa	May 19, 2023
ROCTAVIAN	BioMarin Pharmaceutical, Inc.	**Gene**	Hemophilia A	June 29, 2023
LYFGENIA	Bluebird Bio, Inc.	**Gene**	Sickle cell disease	December 8, 2023
CASGEVY	Vertex Pharmaceuticals Incorporated & Crispr Therapeutics	**Gene**	Sickle cell disease and ß-thalassemia	December 8, 2023
ELEVIDYS	Sarepta Therapeutics, Inc.	**Gene**	Duchenne muscular dystrophy	January 10, 2024
LENMELDY (LIBMELDY)	Orchard Therapeutics	**Gene**	Metachromatic leukodystrophy (MLD)	March 18, 2024
BEQVEZ	Pfizer, Inc.	**Gene**	Moderate to severe hemophilia B	April 25, 2024

Cord Blood Cell Therapies				
PRODUCT	**COMPANY**	**MODALITY**	**INDICATION**	**APPROVAL DATE**
HEMACORD	New York Blood Center	**Cell**	Transplantation procedures	November 10, 2011
HPC, Cord Blood	Clinimmune Labs, University of Colorado Cord Blood Bank	**Cell**	Transplantation procedures	May 24, 2012
ALLOCORD	SSM Cardinal Glennon Children's Medical Center	**Cell**	Transplantation procedures	May 30, 2012
DUCORD	Duke University School of Medicine	**Cell**	Transplantation procedures	October 4, 2012
HPC, Cord Blood, Life South	LifeSouth Community Blood Centers, Inc.	**Cell**	Transplantation procedures	June 13, 2013
HPC, Cord Blood, Bloodworks	Bloodworks	**Cell**	Transplantation procedures	January 28, 2016
CLEVECORD	Cleveland Cord Blood Center	**Cell**	Transplantation procedures	September 1, 2016
HPC, Cord Blood, MD Anderson	MD Anderson Cord Blood Bank	**Cell**	Transplantation procedures	June 21, 2018

Source: U.S. Food and Drug Administration. Approved Cellular and Gene Therapy Products. Updated August 2, 2024. Accessed September 18, 2024. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products

Oncology dominates the CGT pipeline, but broader indications could expand the field in coming years.

Late-stage CGTs in U.S. pipeline by therapy area

Total number of unique drugs is 734. Some drugs are represented in multiple therapy areas.

Source: GlobalData Filtered Drug Database (Q1 24)
See Glossary for notes on process to select late-stage drugs in development in the U.S.

Glossary of terms

PIPELINE CHARTS

CGT Defining Criteria
Cell, gene, and gene-modified cell therapies are defined by parameters set forth by the Alliance for Regenerative Medicine (ARM), an international advocacy organization dedicated to realizing the promise of regenerative medicines and advanced therapies.

- *Exclusionary Criteria* includes: Cells without engineering modifications, therapies involving scaffolding, and therapies with sustained or self-perpetuating gene modifications after dosing are not considered CGT under ARM's industry standard definitions.

Cell Therapy
Cell therapy is the administration of cells into a patient's body as a therapy to treat a disease. This includes administration of autologous (from the patients themselves) or allogeneic (from another human being) cells. Cell therapy incorporates cells that have been substantially manipulated chemically or physically or cells that are not intended to be used for the same essential functions in the body.

Gene Therapy
A therapy in which foreign genetic material (DNA or RNA) is inserted into a person's cells to prevent or fight disease.

Gene-Modified Cell Therapy
In gene-modified cell therapy, specific cells are genetically modified outside the body and placed into the patient's body in order to help the patient fight a disease. These cells may be autologous (from the patients themselves) or allogeneic (from another human being).

Allogeneic
A type of cell therapy in which the therapeutic cells are derived from a different person than the intended recipient. The donor cells are cultured, modified, and then administered to the patient's body.

Autologous
A type of cell therapy in which the therapeutic cells are derived from the patient who is undergoing treatment. The cells are cultured, modified, and then re-infused into the patient from whom they were derived.

Global Geography
The pipeline drug geography "Global" is a geographical placeholder that is tagged to pipeline drugs where the company or institution has not yet specified the exact geographical region or country in which they intend to pursue market approval.

Late-stage U.S. Pipeline
Drugs in development in the U.S. and/or Global geographies in Phase 2 or Phase 3, or Pre-Registrational, based on public data.

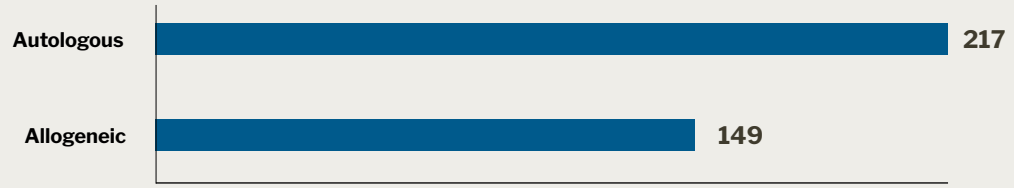
Source: GlobalData

Gene therapies outnumber cell therapies and GMCTs in the pipeline.

Allogeneic cell therapies represent a growing portion of the pipeline.

Late-stage CGTs in U.S. pipeline: by autologous or allogeneic type*

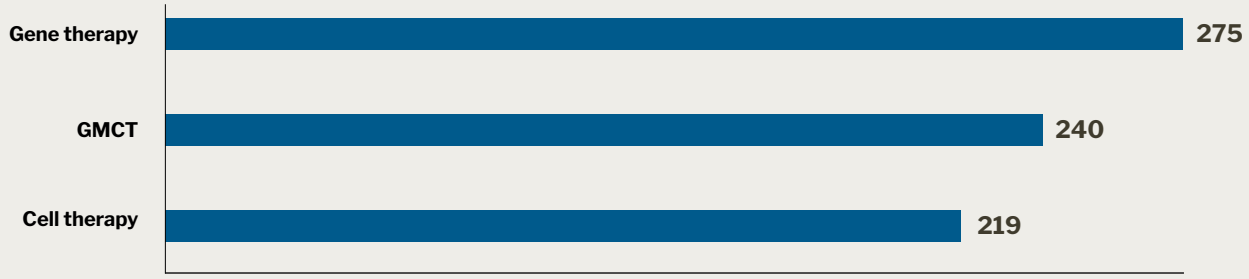
366 out of the 734 CGT late-stage pipeline drugs in the database are tagged as autologous or allogeneic.



* GlobalData classifies these labels as "Molecule Type." Only 366 out of 734 unique drug database records have an autologous/allogeneic designation.

Source: GlobalData Filtered Drug Database (Q1 24)

Late-stage CGTs in U.S. pipeline: by cell, gene, or gene-modified cell therapy (GMCT)

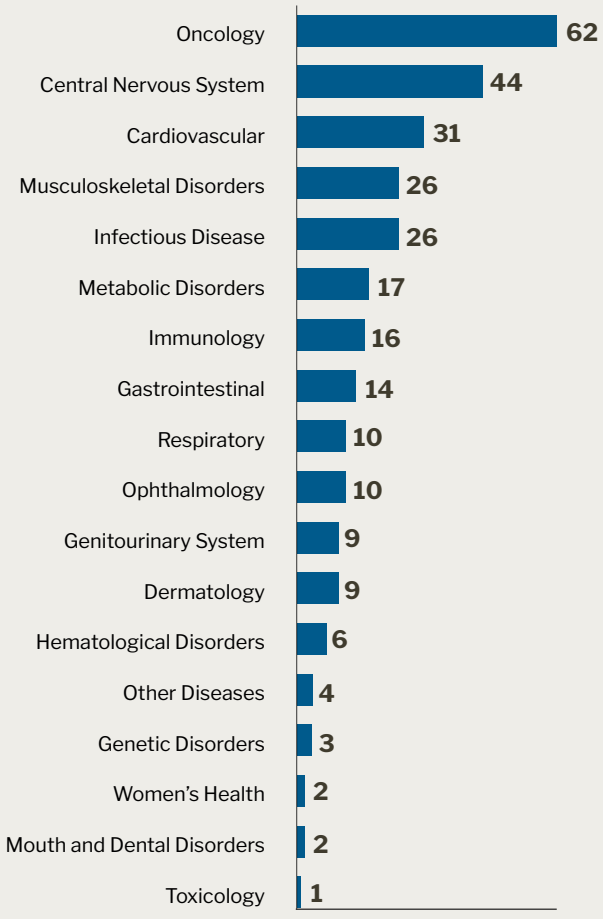


Source: GlobalData Filtered Drug Database (Q1 24)

Therapies for neurological, cardiovascular, and metabolic conditions represent a growing portion of the CGT pipeline.

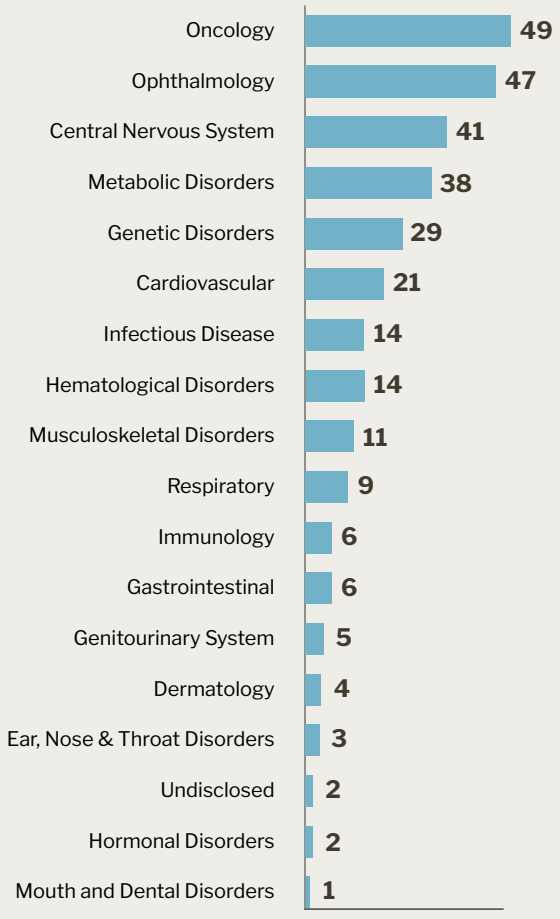
Late-stage cell therapies in U.S. pipeline by therapy area

Total unique drugs is 219. Some drugs are represented in multiple therapy areas.

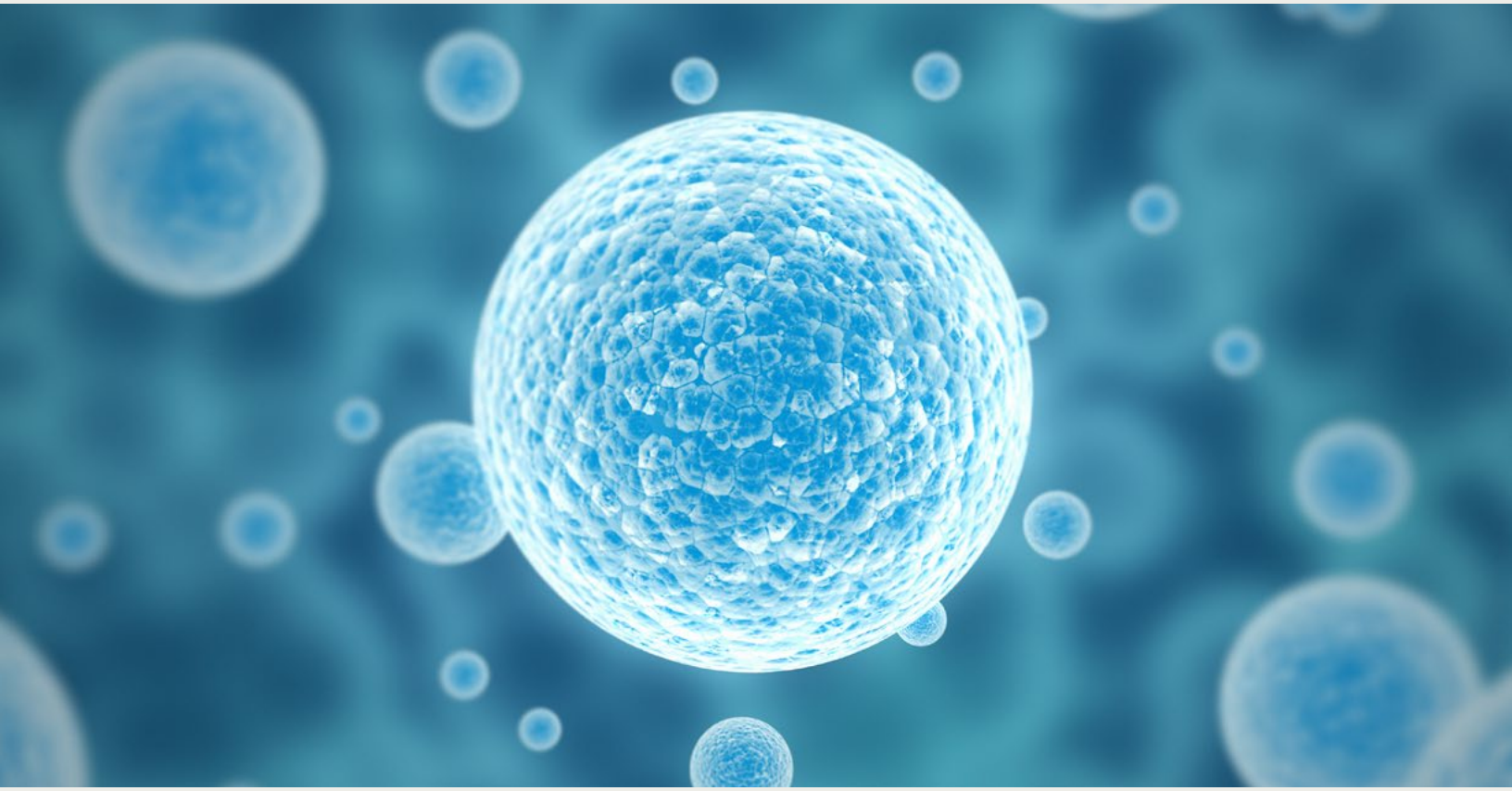


Late-stage gene therapies in U.S. pipeline by therapy area

Total unique drugs is 275. Some drugs are represented in multiple therapy areas.

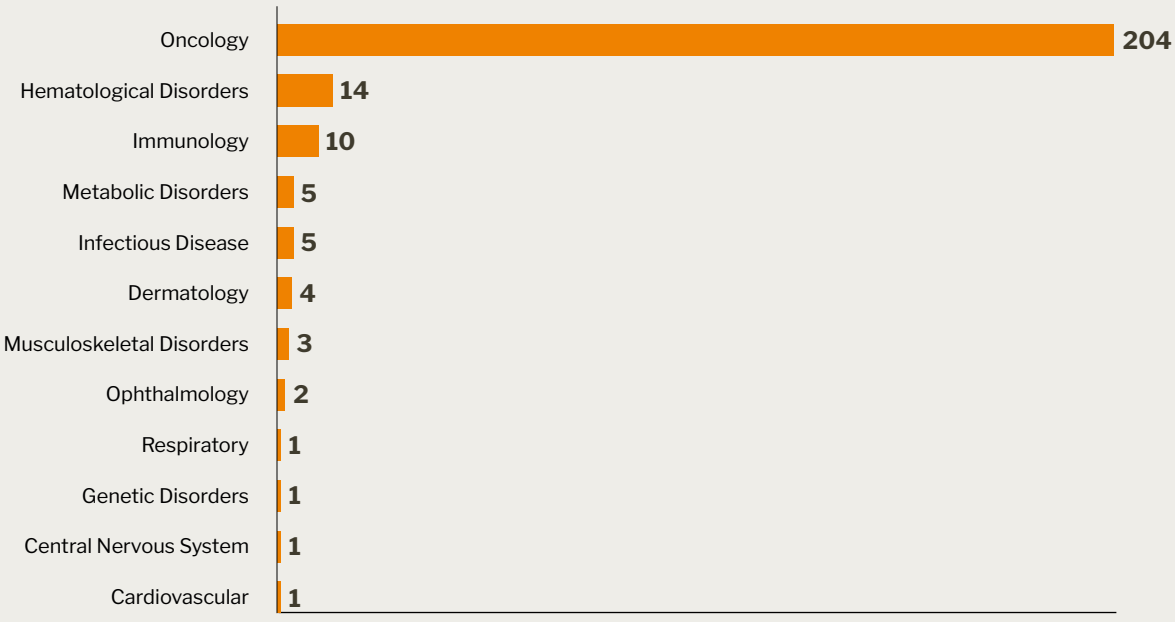


Source: GlobalData Filtered Drug Database (Q1 24)



Late-stage GMCTs in U.S. pipeline by therapy area

Total unique drugs is 240. Some drugs are represented in multiple therapy areas.



Source: GlobalData Filtered Drug Database (Q1 24)

Expert perspective: ELLEN MOORE

What's next for cell and gene therapies: Innovative approaches, broader indications

As cell and gene therapies (CGTs) continue to prove their ability to offer new and better solutions than existing medicines, drug developers are widening their research into more prevalent disease categories.

"These efforts will require new scientific approaches, improved manufacturing capabilities, and unique partnerships," said Ellen Moore, Principal Consultant at GlobalData. Moore brings a unique perspective as the leader of GlobalData's CGT Consulting team, a group with decades of experience providing intelligence to biopharma companies, manufacturers, and suppliers.

"There are innovations in trial design, in platform technologies, and with cell types being combined with gene editing technologies," Moore said. "It's actually difficult to stay on top of what's most promising in any one area, given the rate of evolution in the space."

On the drug development front, she pointed to recent milestones including the FDA's approval of the first cell therapy for solid tumors[1] and the first approval for a CRISPR therapy[2] as indications of the field's progress.

While the majority of cell and gene therapies approved today target oncology and rare diseases, drug manufacturers are increasingly expanding their focus to other indications. Moore said central nervous system (CNS) disorders, including Alzheimer's disease, amyotrophic lateral sclerosis (ALS), multiple sclerosis (MS), and Parkinson's disease are of particular interest to biopharma companies developing cell and gene therapies.

"According to our internal reports, oncology will still represent about 40% of cell and gene therapy revenue in 2029, but only 40%," she said. "Meanwhile, there are currently over 1,000 different cell and gene therapies in development for more than 100 CNS indications."

After CNS, metabolic disorders are the next largest category for drug developers, particularly type 1 diabetes, a condition that affects approximately 1.7 million Americans, according to the U.S. Centers for Disease Control.[3] Moore also sees promising developments in osteoarthritis.

"There are some osteoarthritis assets in late-stage development that have disease-modifying potential, where cartilage could be regenerated. If that works, then it could attract patients who would otherwise be receiving knee replacement surgery," Moore said. "You could see how that would have a huge impact."

"It's actually difficult to stay on top of what's most promising in any one area, given the rate of evolution in the space."

— Ellen Moore
Principal Consultant
GlobalData

Innovation is needed to improve patient access to these novel therapies. However, one limitation has been the reliance on autologous cells or cells from an individual patient. If drug developers can improve the production and safety of allogeneic cells, or cells from a universal donor, while achieving comparable efficacy, they could theoretically create more cost effective off-the-shelf drugs. To help achieve this, the FDA in April released draft safety guidance for companies manufacturing allogeneic cell therapies, to provide clearer rules for drug developers.[4]

"I would expect that this guidance will lower the risk for some companies looking to take cell therapies into the clinic," Moore said. "And so maybe we will see even more allogeneic cell therapies entering development once this guidance is finalized."

Complex manufacturing is also a factor that impacts chimeric antigen receptor (CAR) T-cell therapy. Moore discussed the need for new manufacturing processes and partnerships to help lower the production cost of CAR T therapies, especially outside of the U.S. She cited a collaboration between Brazil's Ministry of Health and a U.S. nonprofit dedicated to accelerating the development of advanced medicines as an early example.[5] The goal of the arrangement is to lower the product cost to about one-tenth the price of currently approved CAR Ts in the U.S., and with local production, allow Brazil to provide the medicines free of charge to its citizens.

"That's just one example of innovation happening in the manufacturing space that would of course improve access," Moore said. "I imagine that there are similar innovative partnerships happening across the board for cell therapies and gene therapies that would help mitigate the risks of unsuccessful commercialization."

Overall, the early success of CGTs has been encouraging, but predicting the future with any certainty is challenging at this point, given the rapid progress and various innovations in development.

"The market is still trying to assess just how quickly the need will increase across the board for everything, from developing cell lines to delivering to the patient and having capacity at hospitals," she said. "Innovation is happening at an incredibly rapid pace and industry standard processes are catching up."

Expert perspective: JOHN BISHAI, PhD

Innovation in CGT is flourishing, but investors remain uncertain.

The cell and gene therapy landscape has seen significant shifts over the past few years, marked by both scientific breakthroughs and market volatility. John Bishai, Managing Director at Bank of America Securities, Healthcare Investment Banking, analyzes the short- and long-term prospects for companies in the space, and sees both challenges and opportunities within this dynamic sector.

A scientist by training with a doctorate in physiology from Johns Hopkins, Bishai started his career as regulatory manager at the FDA before moving into the financial industry and building a specialized team focused on CGT. Looking at the industry today, he sees a mixed landscape where the science holds great potential for improvements in patient care, but the commercial outlook is more challenging.

"We're at the crossroads right now, and it's sad because gene therapies should have a massive impact," Bishai said. "The scientific advancement of cell and gene therapies has been tremendous, but the business results have not always translated. When applying gene therapies, you have to always understand, what am I solving for? What's the need and what's the current treatment landscape, and can this particular therapy make a significant impact?"

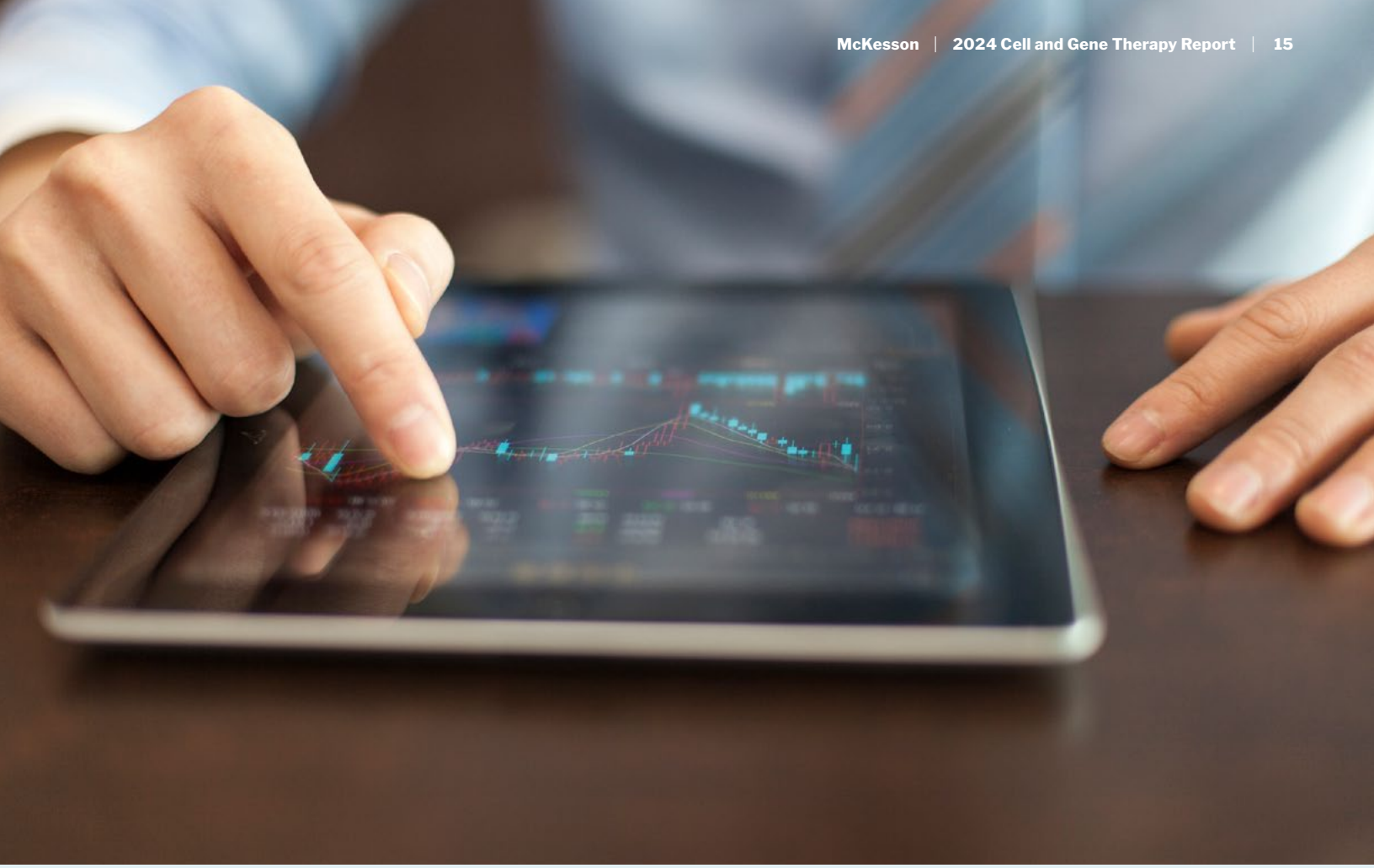
While the innovation in CGT continues to generate optimism, the current outlook for investment in the sector is less rosy. That's a big change from just six years ago, when a string of early gene therapy companies were snapped up by pharmaceutical companies in large deals. These included Novartis snagging AveXis Inc. for $8.7 billion,[1] Roche purchasing Spark Therapeutics for $4.3 billion,[2] Astellas acquiring Audentes Therapeutics for $3 billion,[3] and Bayer buying AskBio for $2 billion.[4]

However, the market peaked in early 2021, and since then, the S&P Biotech ETF (XBI), a key benchmark for biotech stocks, has seen a significant decline, losing approximately 50% of its value, reflecting broader market volatility and investor caution. This downturn has contributed to a notable decrease in the number of biotech IPOs, from 104 three years ago to just 19 last year, according to BioPharma Dive.[5] In addition, venture capital funding of CGT is at its lowest level in nearly a decade.[6]

The weak capital market and greater regulatory scrutiny around safety issues has resulted in "a tempered excitement in the space," Bishai said. Still, "the fervor for gene edited therapies has continued," as well as gene therapies that have been successful. Now, next-generation gene editing technologies are emerging to challenge first-wave therapies, but investors are wary, wanting to see lab results replicated in the real world before funding these approaches.

"The scientific advancement of cell and gene therapies has been tremendous, but the business results have not always translated."

— John Bishai , PhD
Managing Director, Healthcare Investment Banking
Bank of America Securities



Despite the complex market dynamics, Bishai is optimistic about the innovation happening in CGT and how those advances may improve both outcomes and quality of life for patients.

"On the monogenetic side for rare diseases, the market opportunity is clearly there," Bishai said. "For more prevalent conditions, companies have started to reimagine how they administer gene therapies, experimenting with delivering smaller doses directly to specific organs instead of larger doses systematically, which may reduce adverse events. It feels like the pendulum has swung back to that kind of an approach, and I think that will lead to broader commercial opportunities within gene editing."

Bishai also says he's excited about the potential opportunity for companies that are exploring medtech solutions for delivering CGTs to improve efficacy, including examples of using a gastrointestinal scope to inject a diabetes gene therapy directly into the pancreas, and a catheter to coronary arteries to locally bathe the heart with a therapy for cardiovascular disease.

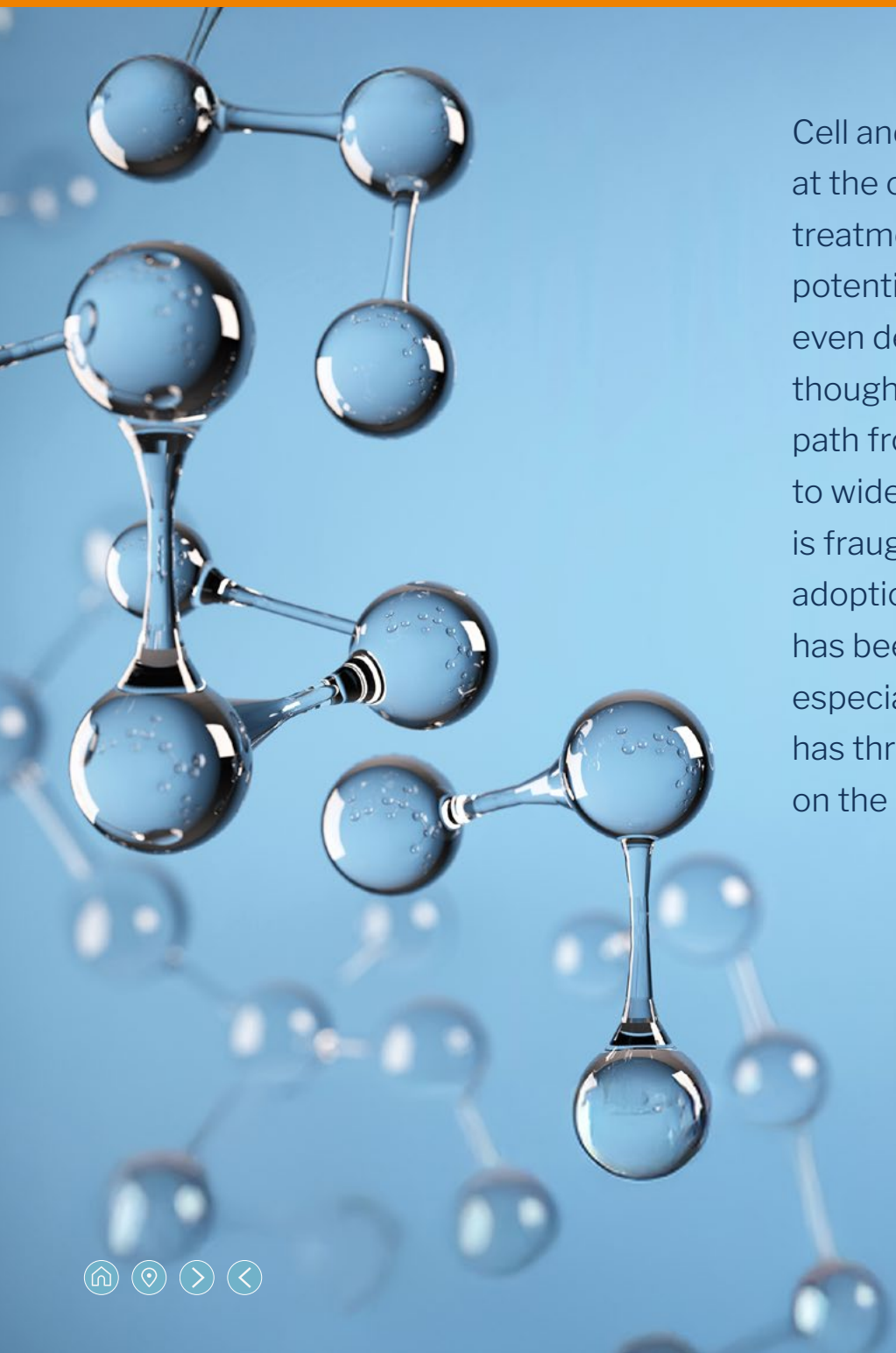
"That's a game changer," Bishai said. "Some companies are thinking outside the box by harmonizing biotech with medtech approaches to drive better results. While this may make scientific sense, getting biotech and medtech investors to buy in is very difficult because they operate differently. Now companies need to get these people over the hump because, 'this isn't your traditional way.' You're incorporating two different kinds of worlds, it just takes a little bit of time."

Ultimately, Bishai predicts capital will be available for companies that are close to entering the clinic and provide access to new targets and indications. This scenario is already playing out in cell therapy, as companies go beyond liquid tumors and into solid tumors.

"We've already hit the bottom," Bishai said. "I feel like we're on the way back up and I think it's just a matter of finding the right delivery and the right diseases."

Survey of U.S. oncologists about CGT

Optimism for cell and gene therapy abounds, but barriers may curb broader adoption



Cell and gene therapies (CGTs) stand at the cusp of transforming medical treatment, offering unprecedented potential to improve outcomes, or even deliver cures, for diseases once thought untreatable. However, the path from scientific breakthrough to widespread use with patients is fraught with challenges—and adoption of certain CGT products has been slower than expected, especially in hemophilia, which now has three FDA approved treatments on the market.[1,2]

To better understand the current and future CGT landscape, we conducted a survey of 124 oncologists to capture their views on how, when, and why they prescribe these medicines to patients. We wanted to hear directly from doctors about their hopes and concerns for these new classes of therapies, why they prescribe CGTs or choose not to, the hurdles they and their patients face, and the solutions needed to drive greater adoption.

Our survey reveals a striking dichotomy: while optimism about CGTs is nearly universal among oncologists, significant barriers persist that could impede broader adoption. An overwhelming 99% of oncologists surveyed agree that CGTs are among the most important medical innovations of our time, with 97% expressing optimism about their potential benefits for patients, including the potential for a cure.

> Three out of five oncologists say CGTs are "still largely unproven."

However, this optimism is tempered by practical realities. Three out of five oncologists say CGTs are "still largely unproven." Many also cite safety concerns, side effects, and affordability issues for patients as top reasons they don't prescribe CGTs to patients who qualify for the treatment.

Accessibility remains a critical issue, with 64% of oncologists agreeing that CGTs are not easily accessible for patients who meet the labeled indications. In addition, three out of five physicians say patients they refer for CGTs often receive other treatments instead. When asked, they cite insurance coverage and out-of-pocket costs as the most common reasons.

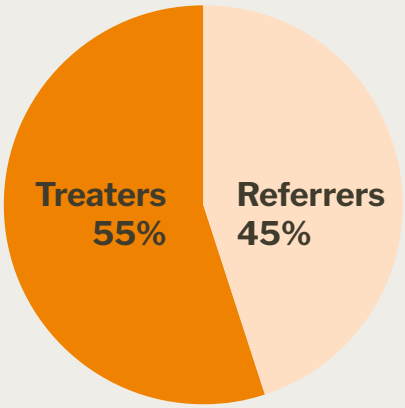
Oncologists have differing views on the changes needed to address accessibility issues, but there is broad consensus that expanding care into community settings is a critical step. There is optimism that innovative payment models will lead to broader adoption of CGTs and will help address disparities in access.

This section will examine these research findings and the insights they provide about CGT adoption. We'll also hear expert perspectives from Mark Trusheim, Strategic Director of NEWDIGS (New Drug Development Paradigms), at Tufts Medical Center, and Robb Richards, Corporate Director of Cell Therapy and Transplant at University of Pennsylvania, who will provide their insights on addressing two of the challenges highlighted in the research—specifically how to pay for high-cost CGTs and how to prepare sites-of-care to meet the growing scale that will be necessary as more CGTs come to market.

Survey participants represent a mix of treaters and referrers, but those who only refer are most likely to be based in the community setting.

Users by type

(n=117)

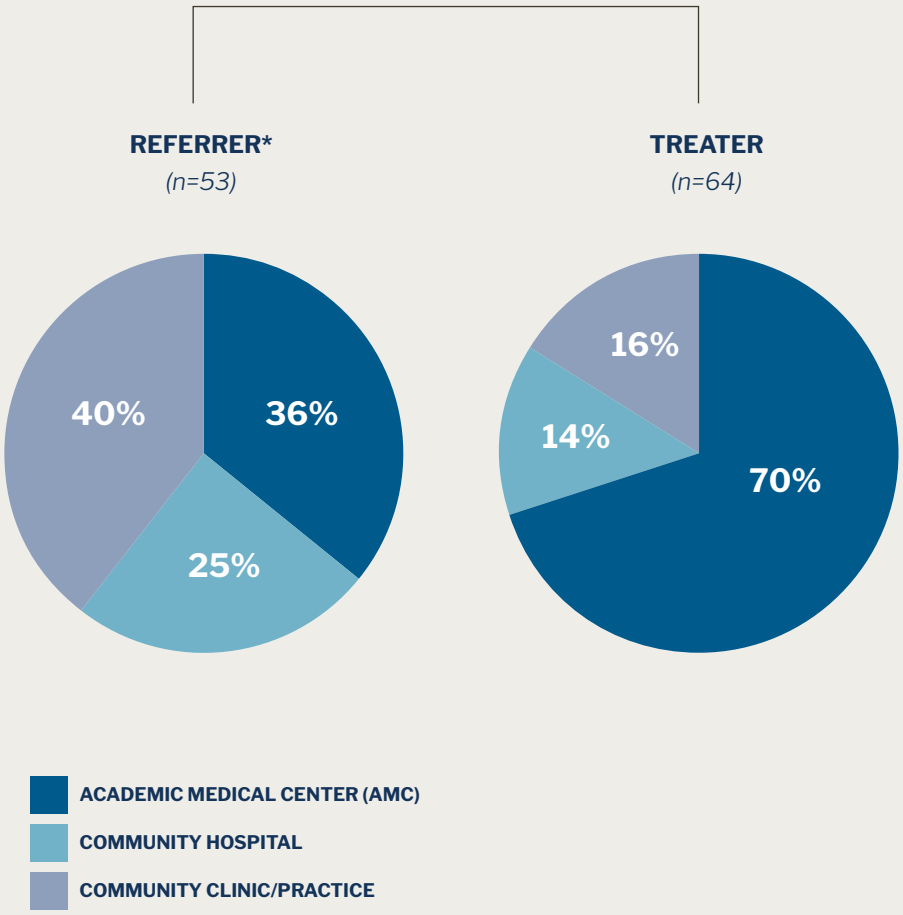


Treaters are inclusive of those who only treat, and those who both treat and refer

Referrers are defined as those who only refer (and do not treat)

Which best describes the setting where you primarily work?

Users by setting

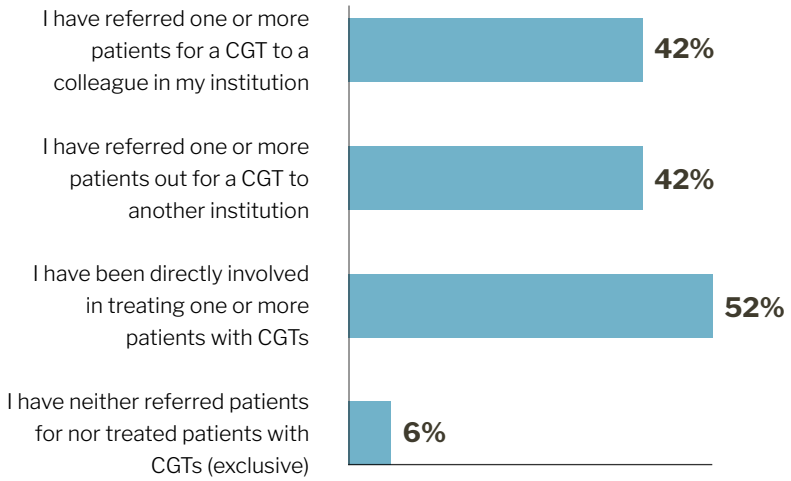


***Note:** Total may not sum to 100% due to rounding

A majority of oncologists say their institutions provide at least one CGT to patients.

Which best describes your experience with CGTs (including approved indications and clinical trials)? Select all that apply.

(n=124)

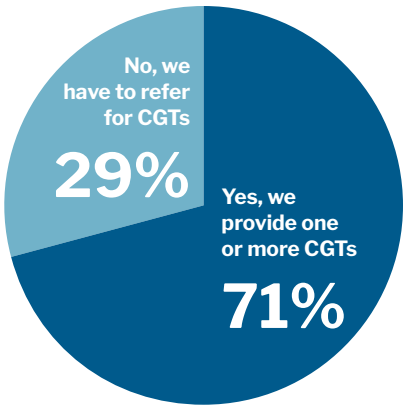


Base: Total Answering

***Note:** Experience subgroups are not mutually exclusive and do not sum to 100%.

Does the main institution where you work treat patients with CGT?

(n=124)



Methodology for Oncologist Survey

McKesson worked with a third party market research firm to conduct a **double-blind online survey of 124 U.S. medical oncologists and hematological oncologists** in June and July 2024. Participants represented a range of geographies, years in practice, and practice sizes.

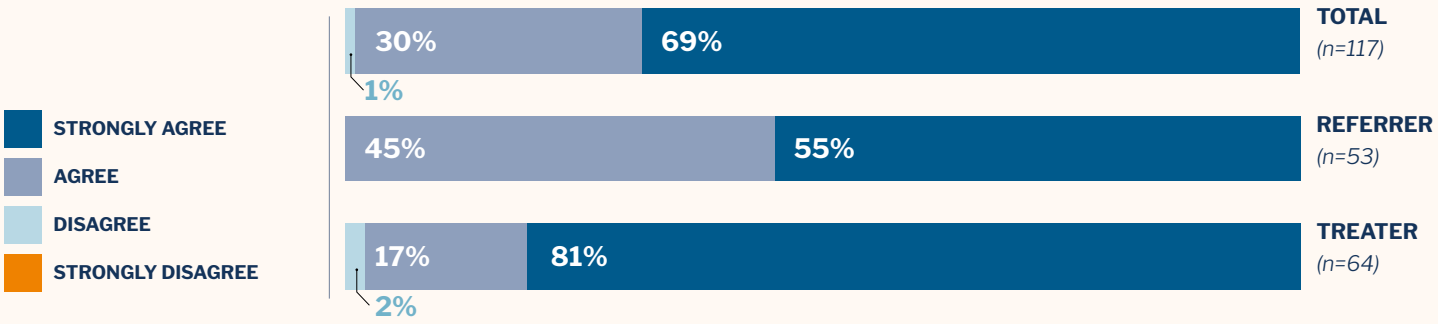
All survey participants reported being familiar with cell and gene therapy (CGT), and 117 had direct experience treating patients with CGTs or referring patients for CGT. Of the 117 oncologists with experience, 55% were classified as treaters, indicating that they have experience treating patients or both treating and referring; 45% were classified as referrers, indicating that they only have experience referring patients.

Participants represent a variety of practice settings: 56% are based in academic medical centers and 44% are in the community (18% at community hospitals and 26% at community clinics).

Nearly all oncologists agree that cell and gene therapies are important medical innovations, but three out of five say they are still largely unproven.

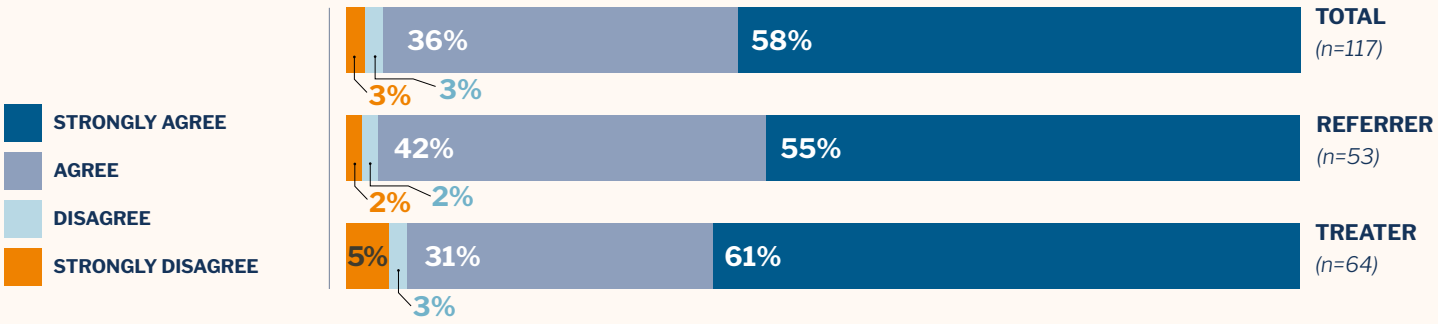
To what extent do you agree or disagree with the following?

When thinking about innovation in healthcare, cell and gene therapies are among the most important medical innovations of our time.



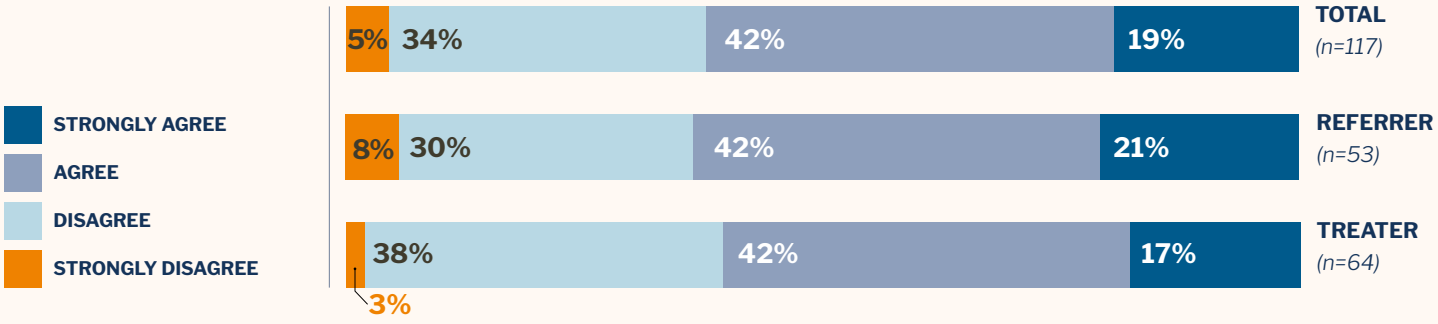
To what extent do you agree or disagree with the following?

CGTs have the potential to dramatically improve patients' quality of life.



To what extent do you agree or disagree with the following?

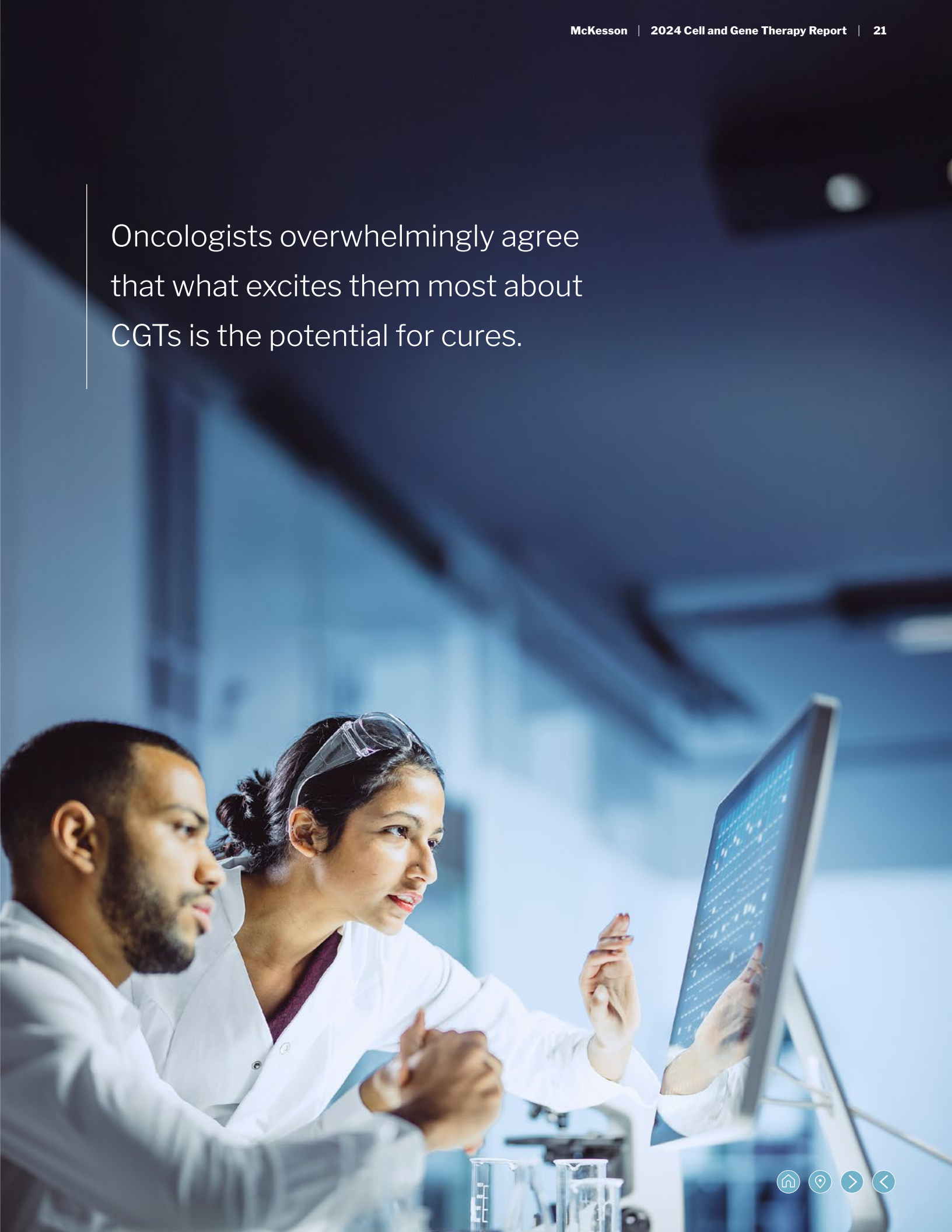
CGTs hold promise for many disease areas, but are still largely unproven.



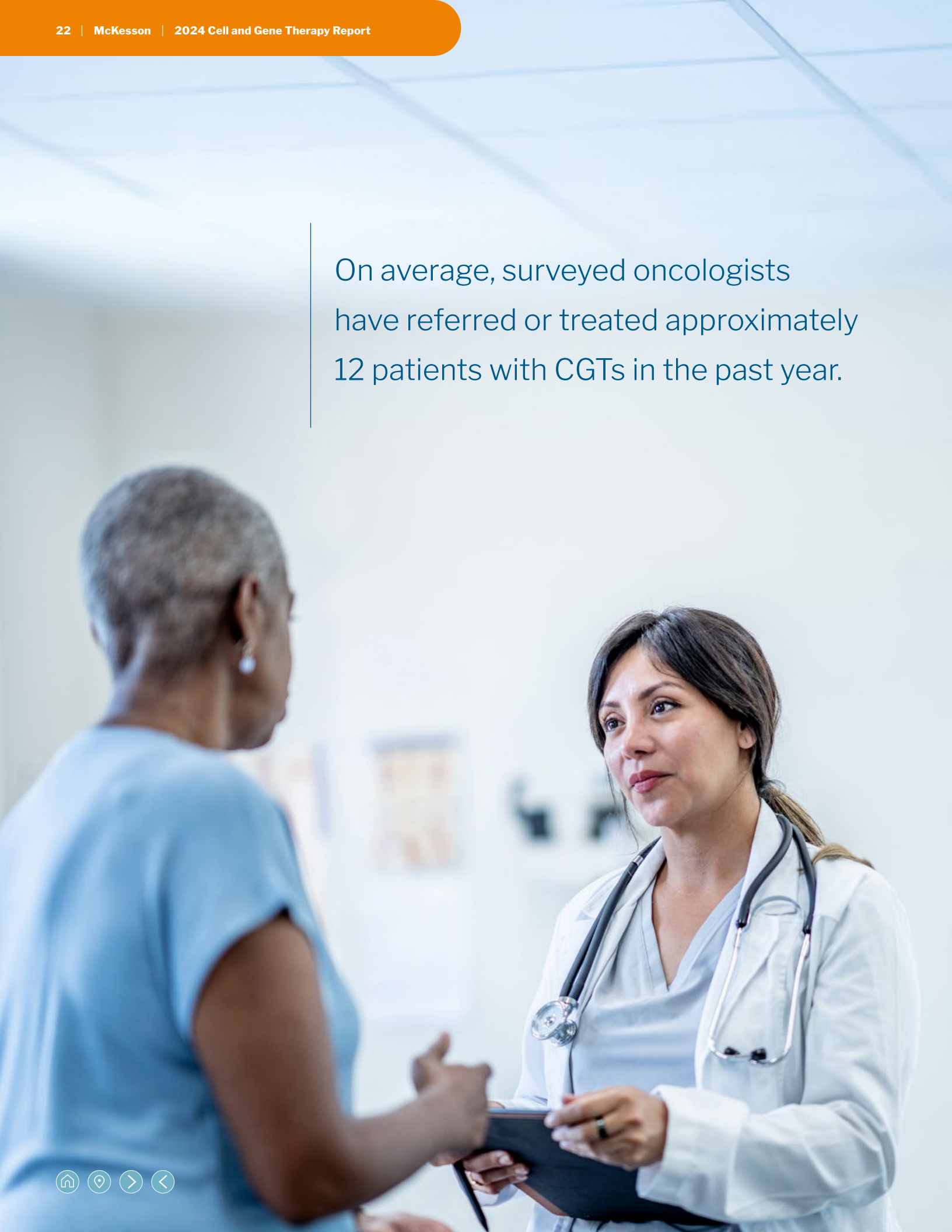
Base: Total Users (See chart)

Note: Total may not sum to 100% due to rounding

Oncologists overwhelmingly agree that what excites them most about CGTs is the potential for cures.

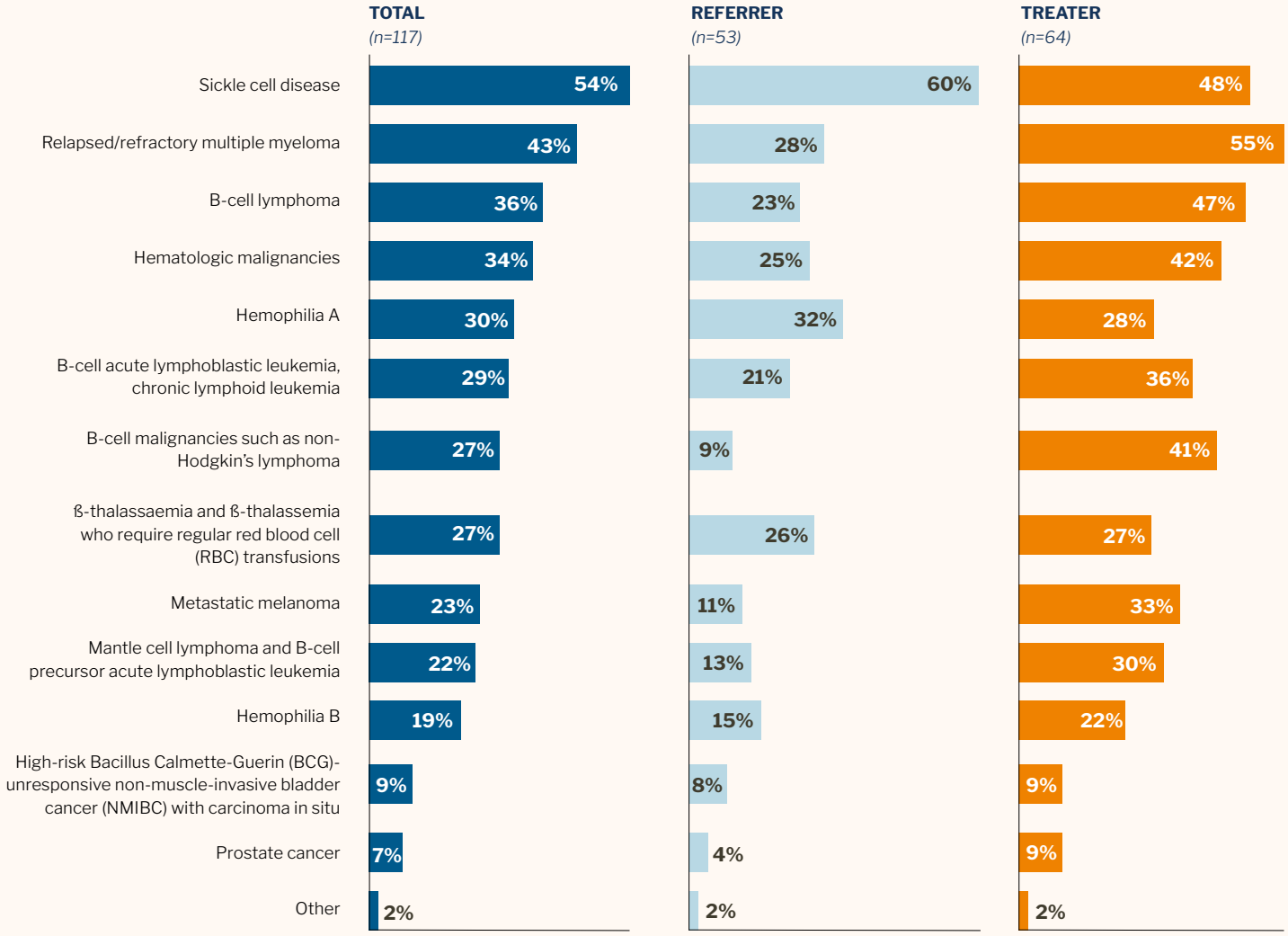


On average, surveyed oncologists have referred or treated approximately 12 patients with CGTs in the past year.



Although sickle cell gene therapies are still relatively new, many oncologists are referring patients for treatment.

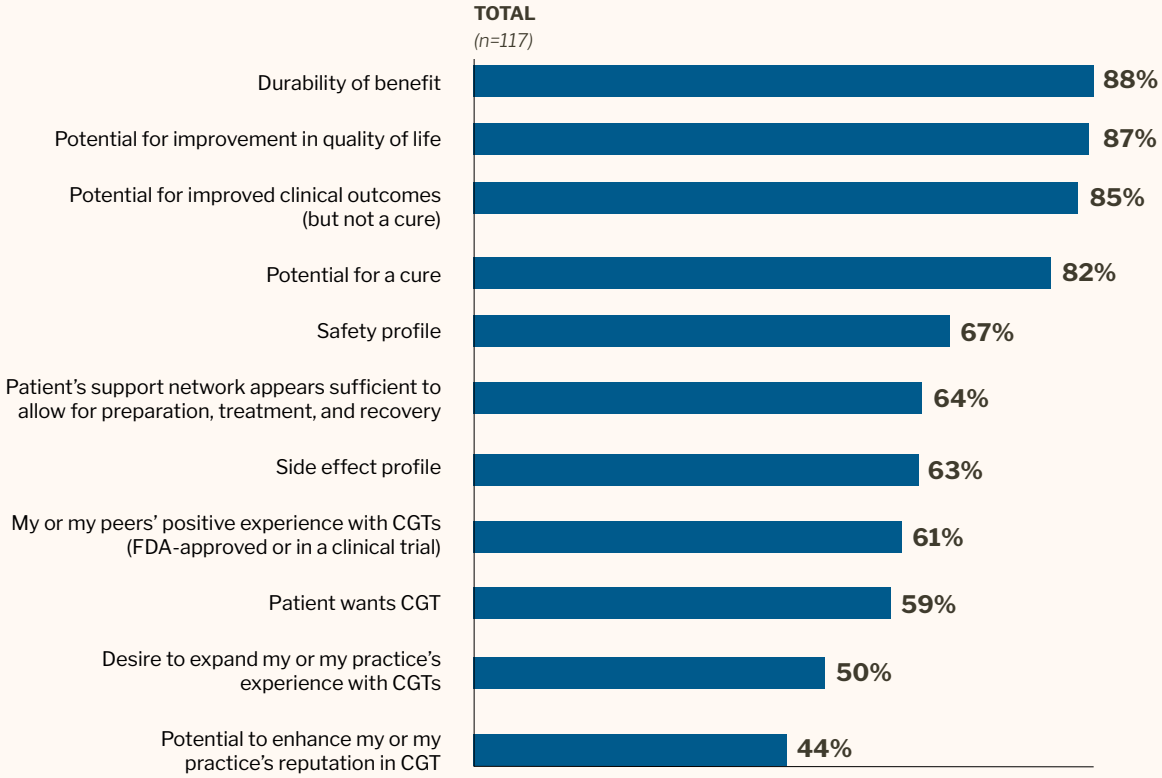
For which of the following conditions have you referred patients for CGTs or treated patients with CGTs? Select all that apply.



Base: Total Users (See chart)

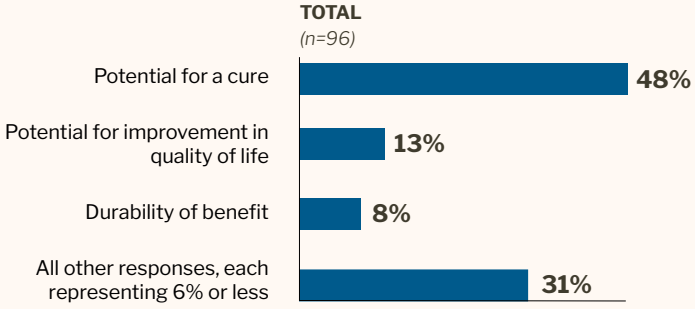
Potential to cure disease is the biggest driver of oncologists' decision to prescribe CGTs to patients.

Thinking of patients you have referred/treated with any type of CGT, how influential was each of the following in driving your decision to refer/treat with a CGT? Select all that apply.



Base: Total Users (See chart)

Of the items you considered extremely influential in your decision to refer/treat, what was the single most influential?



Base: Among those who selected >1 driver as extremely influential in previous question

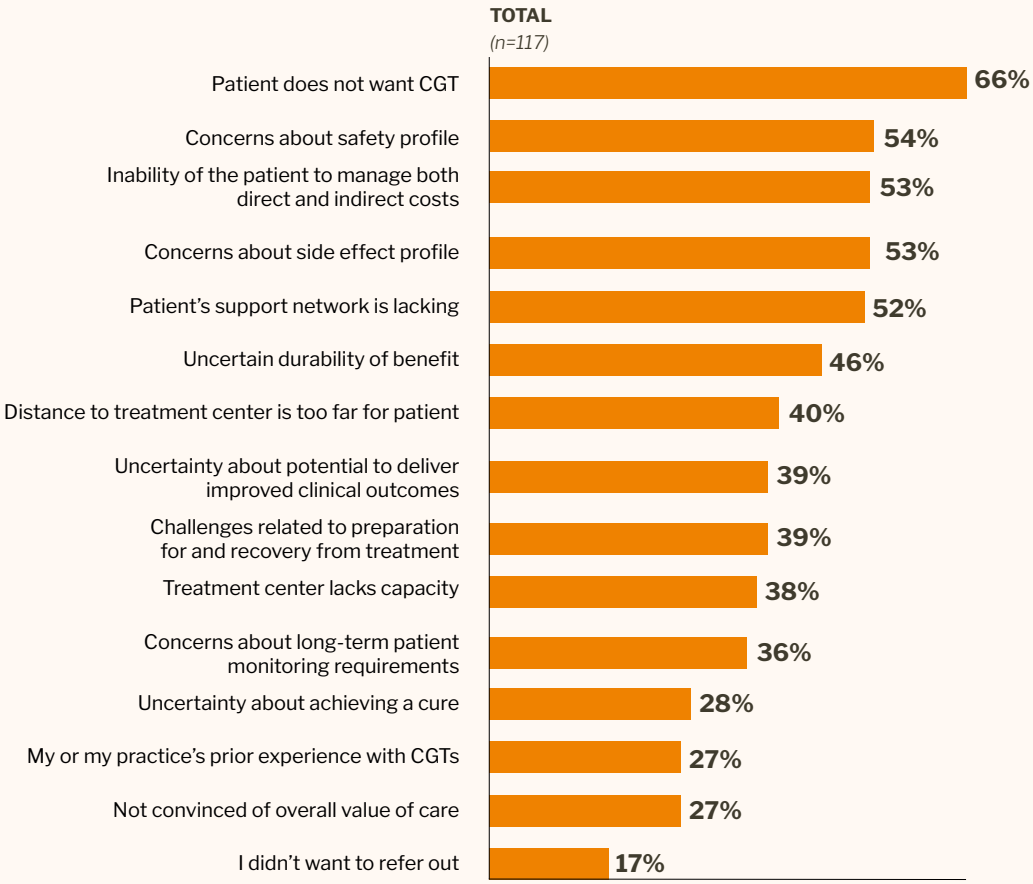
Note: Total may not sum to 100% due to rounding

Nearly half of oncologists say the potential for a cure was “extremely influential” in their decision to recommend a CGT to a patient.

Many oncologists see “durability of benefit” as a key reason to treat patients with CGTs, but almost half also cited “uncertain durability of benefit” as a potential barrier to treatment.

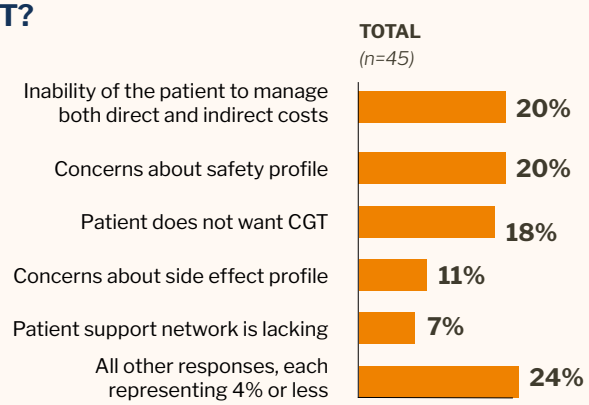
Safety, affordability, side effects, and patient preference are the biggest reasons oncologists don't prescribe CGTs.

Thinking of patients who fit the labeled indication of a CGT, but who you have not referred/treated with a CGT, how influential was each of the following barriers in your decision to not refer/treat? Select all that apply.



Base: Total Users (See chart)

Of the items you considered extremely influential in your decision NOT to refer/treat, which one if removed or resolved would be most likely to lead you to refer/treat with CGT?

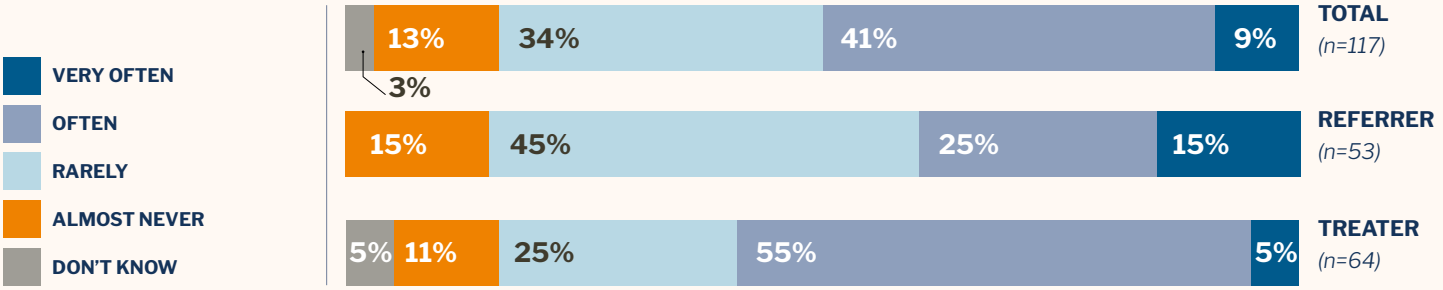


Base: Among those who selected >1 barrier as extremely influential in previous question.

Note: Total may not sum to 100% due to rounding

Half of patients aren't aware of CGT options for their disease, oncologists say, and newness of treatment is among their top concerns.

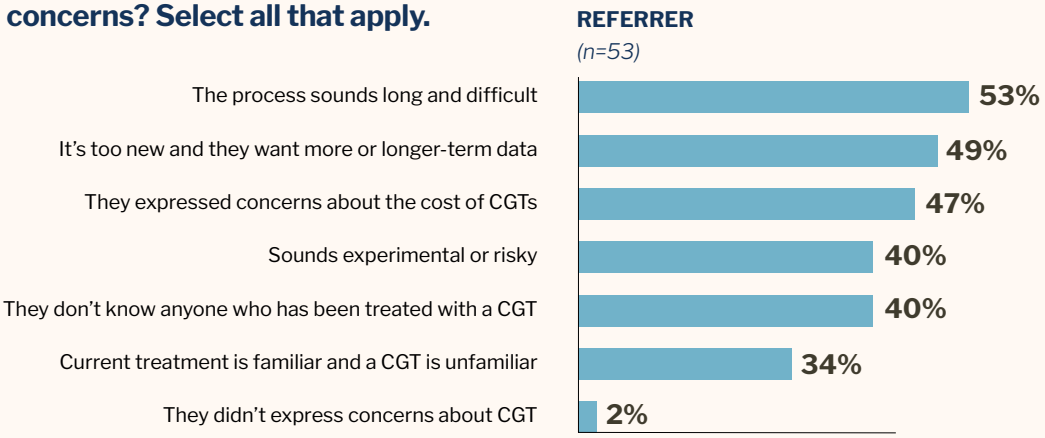
How often are your patients aware of a CGT if they have a condition for which a CGT is available?

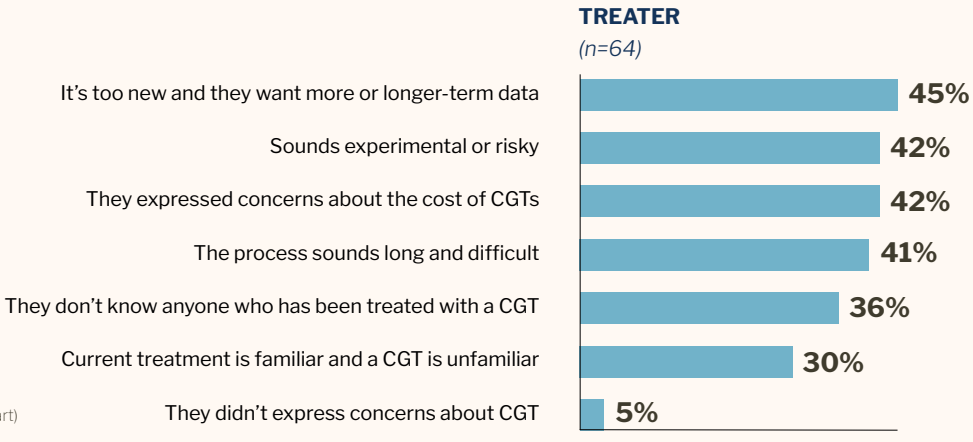


Base: Total Users (See chart)

Note: Total may not sum to 100% due to rounding

When you refer patients for CGT, what are their most common concerns? Select all that apply.

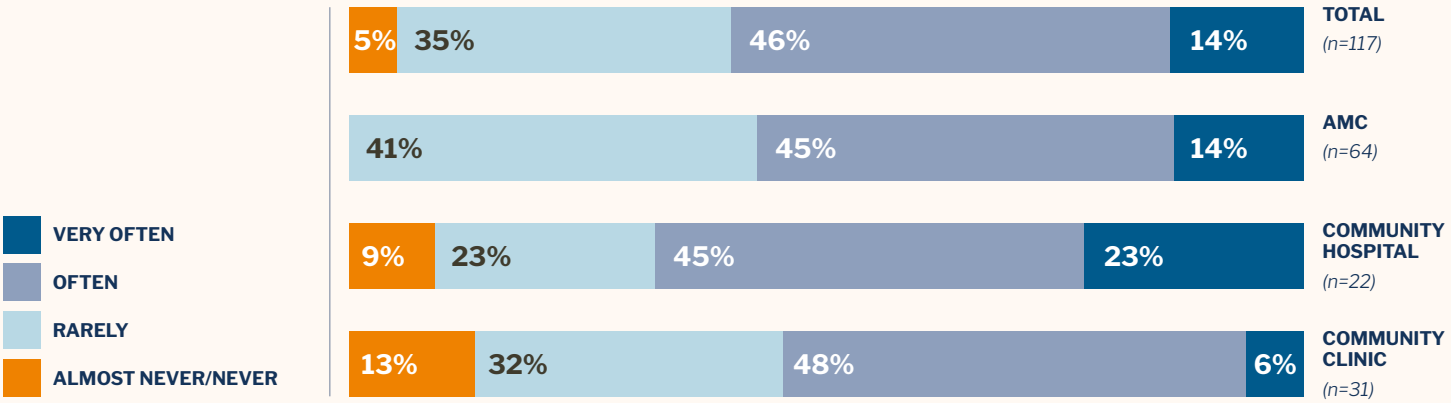




Base: Total Users (See chart)

Three out of five doctors say the patients they refer for CGTs often receive other therapies, and they cite insurance coverage and patient out-of-pocket costs as the top reasons.

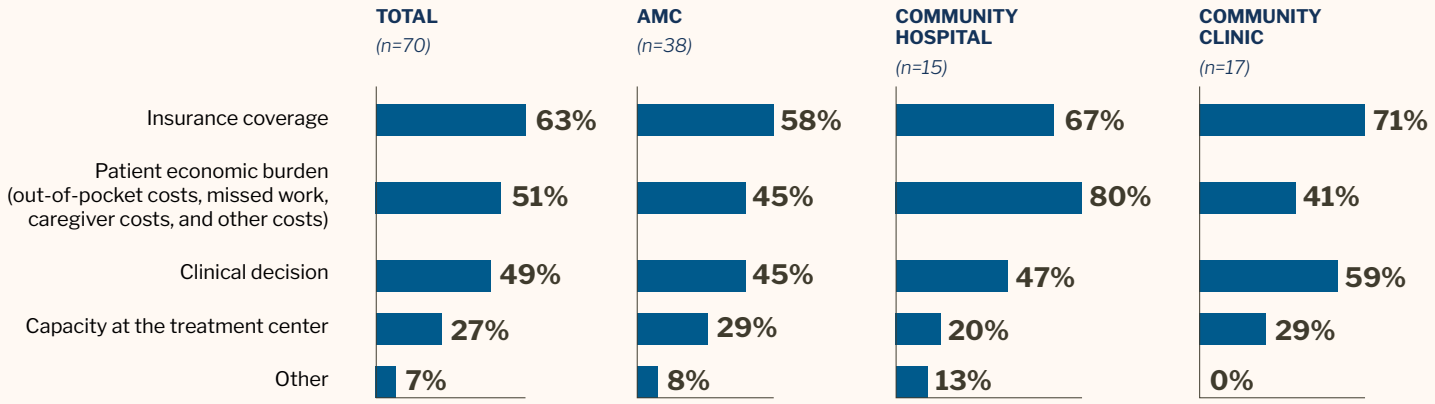
Based on your experience, how often are patients referred for a CGT but ultimately treated with another therapy?



Base: Total Users (See chart)

Note: Total may not sum to 100% due to rounding

What are the most common reasons patients who are referred for a CGT don't ultimately receive it? Select all that apply.

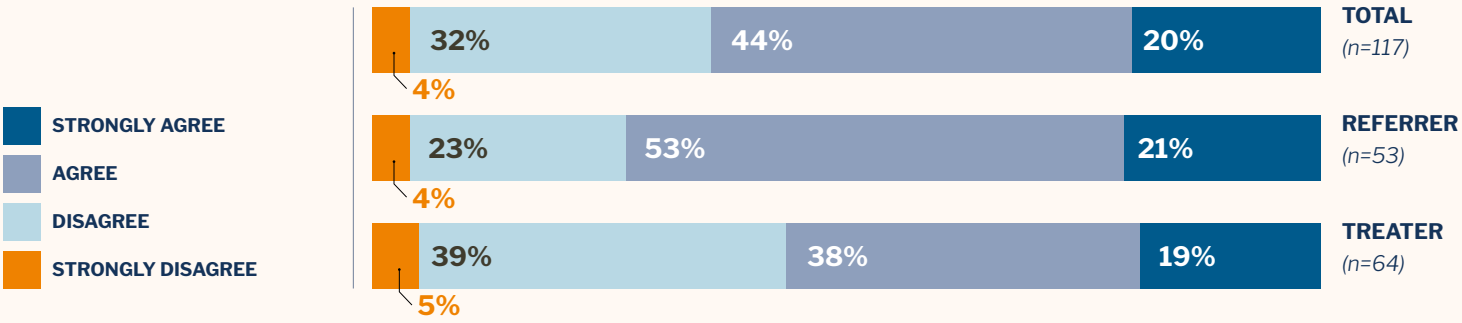


Base: Users who often/very often referred patients for CGT

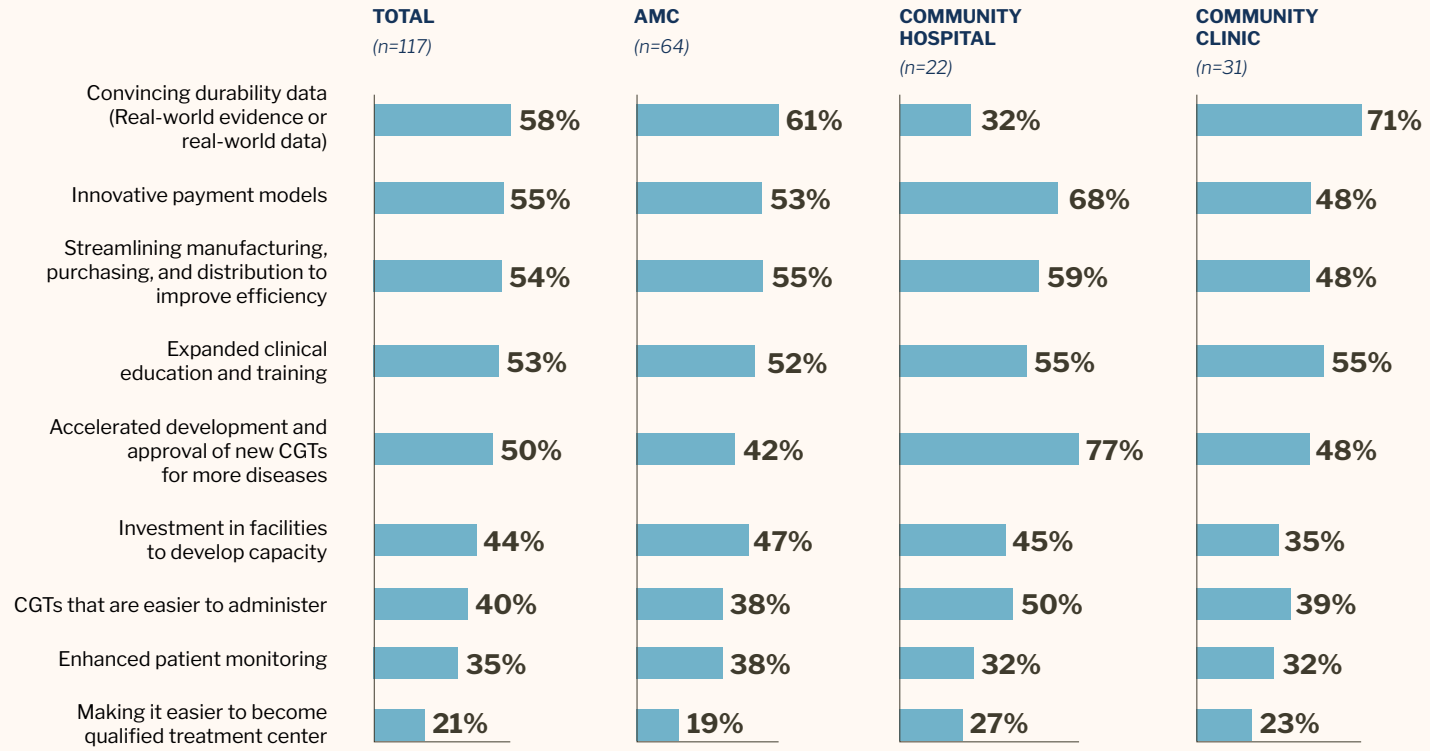
Two-thirds of oncologists say that CGTs are not easily accessible for their patients today, but they are split on changes necessary to increase access.

To what extent do you agree or disagree with the following?

CGTs are not easily accessible for my patients who meet the labeled indication.



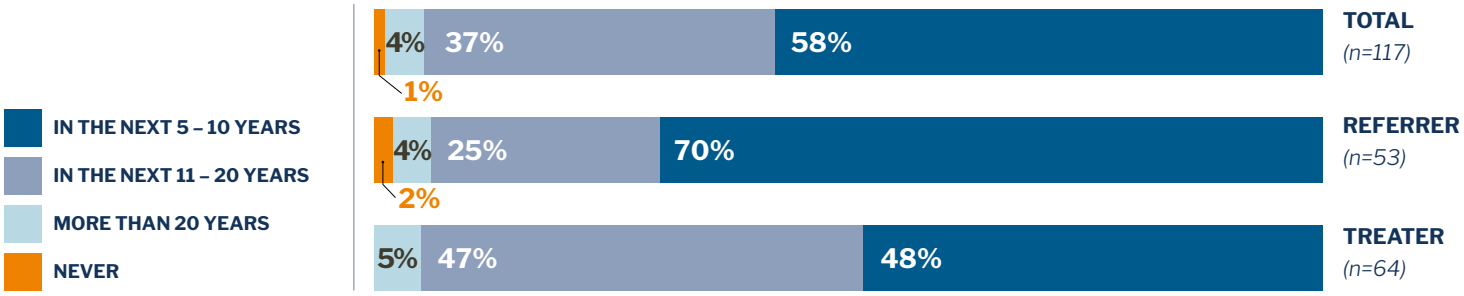
Thinking about CGTs across all care settings (and excluding the topic of overall costs), which of the following needs to be prioritized in the next decade to increase access to CGTs? Select all that apply.



Base: Total Users (See chart)

More than half of oncologists say CGTs will be widely accessible within 10 years.

In your opinion, when will CGTs become widely accessible for patients who meet the labeled indications?



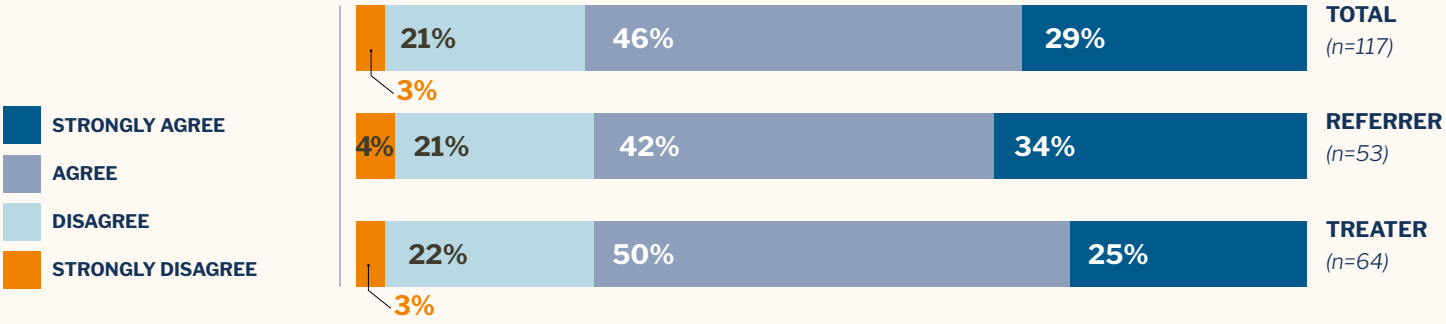
Base: Total Users (See chart)

Note: Total may not sum to 100% due to rounding

Overwhelmingly, oncologists see innovative payment models as a way to increase CGT adoption and address disparities.

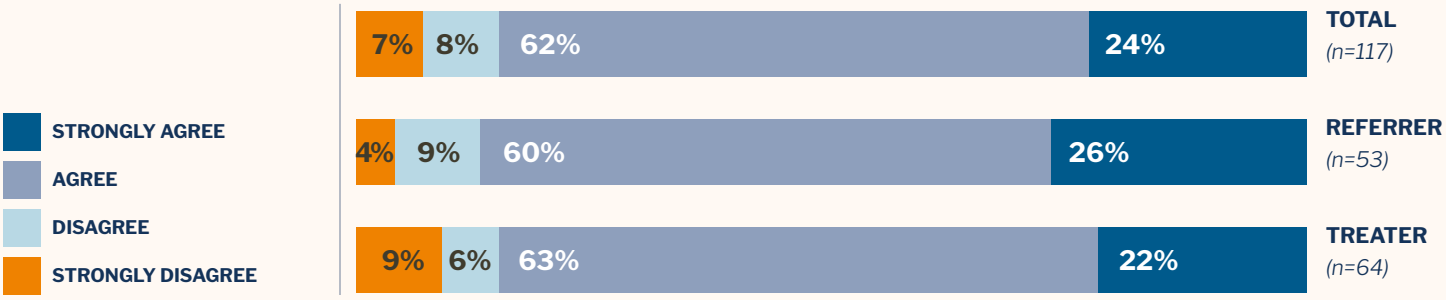
To what extent do you agree or disagree with the following statement?

The U.S. healthcare system is incompatible with high-cost CGTs.



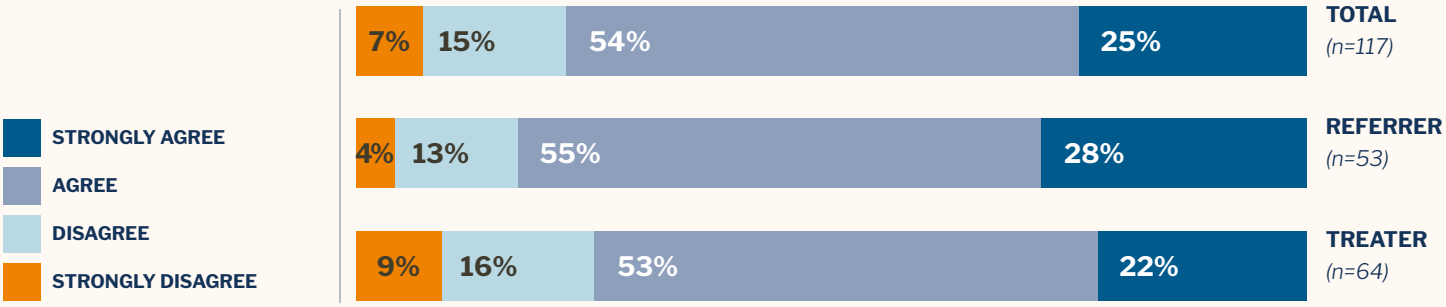
How much do you agree or disagree with the following statement:

Innovative payment models (such as value-based payment tied to successful outcomes) will lead to broader adoption of CGTs.



How much do you agree or disagree with the following statement:

Innovative payment models are likely to help alleviate disparities in access to treatment.



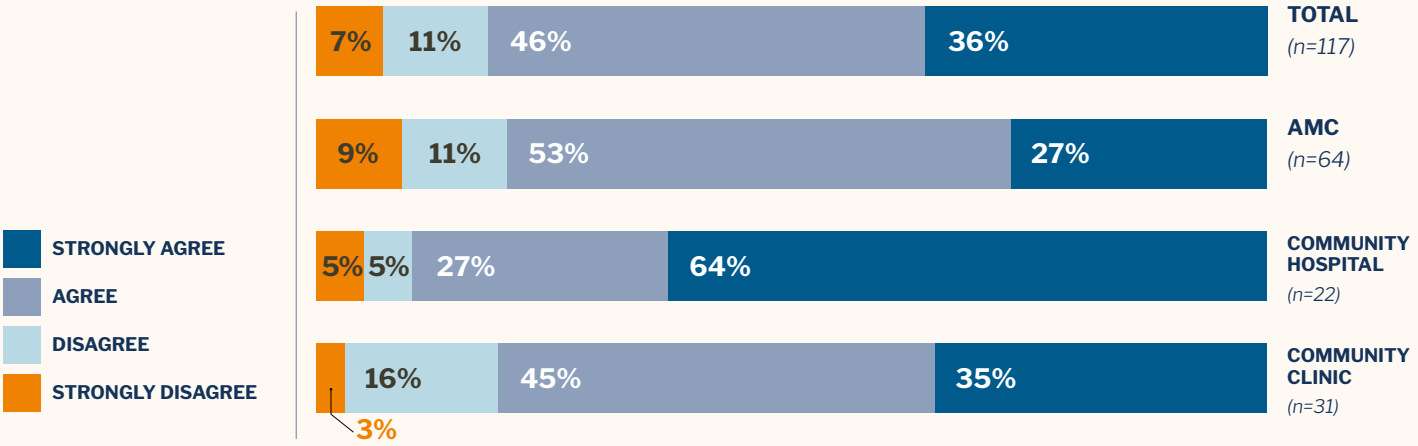
Base: Total Users (See chart)

Note: Total may not sum to 100% due to rounding

Moving CGTs into the community and expanding capacity at academic centers will help broaden patient access, oncologists say.

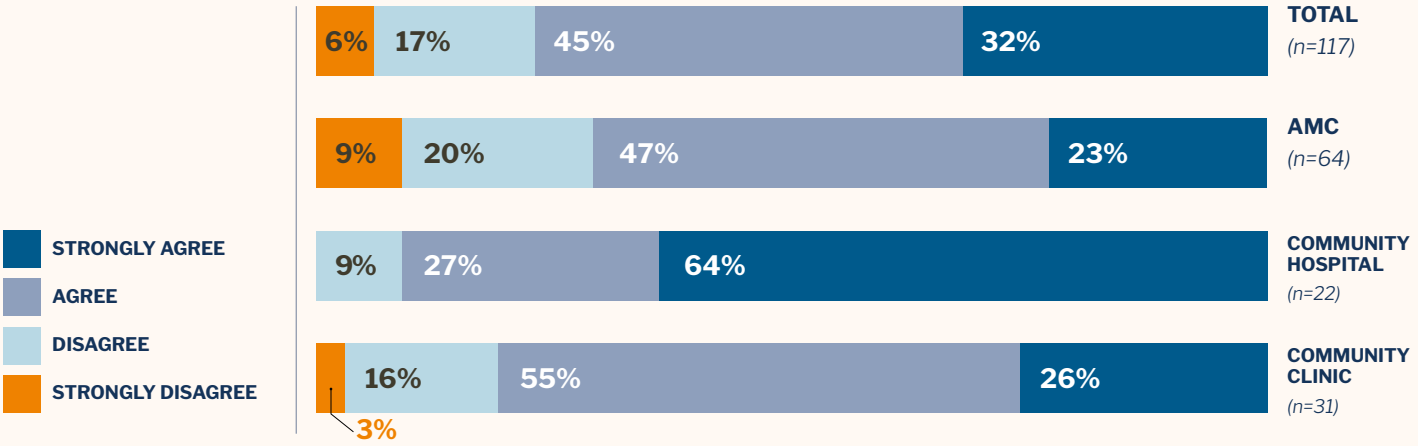
To what extent do you agree or disagree with the following statement?

Expanding CGTs into community hospitals is necessary to ensure broader access to patients.



How much do you agree or disagree with the following statement:

Expanding CGTs into community clinics is necessary to ensure broader access to patients.

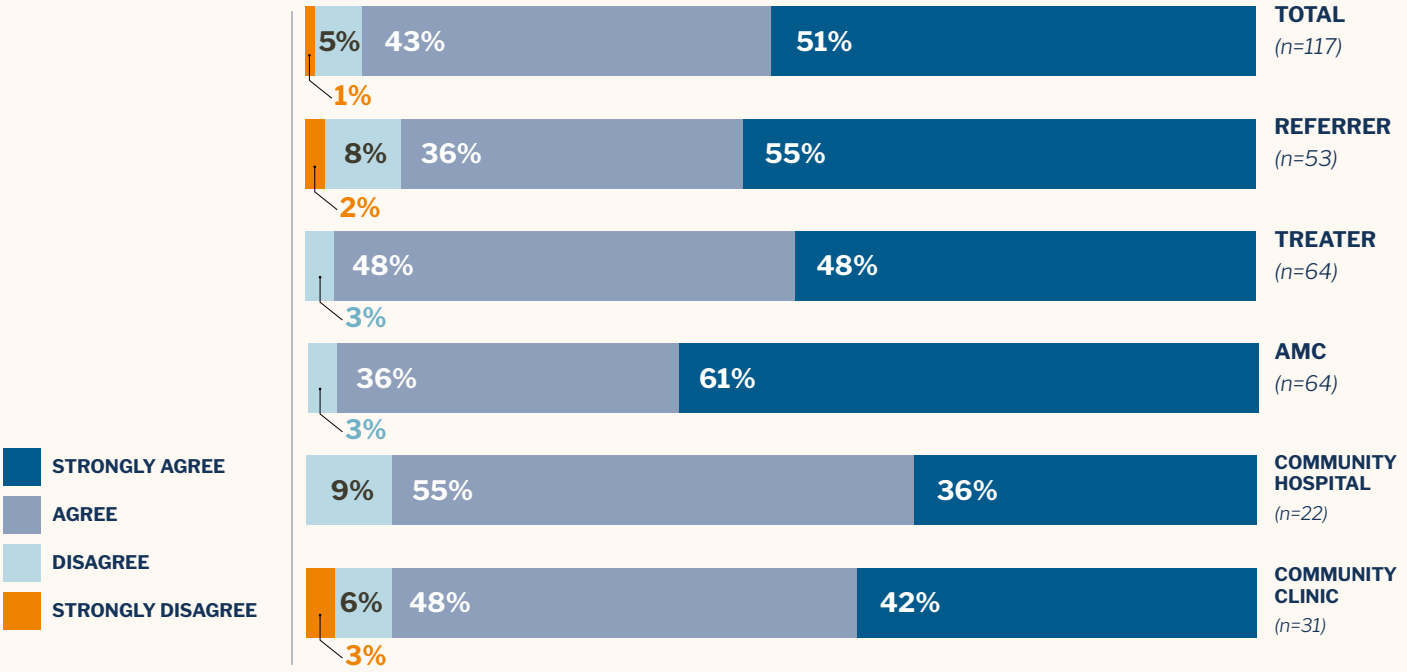


Base: Total Users (See chart)

Note: Total may not sum to 100% due to rounding

How much do you agree or disagree with the following statement:

Expanding the capacity of CGT programs at academic medical centers is necessary to ensure broader access to patients.

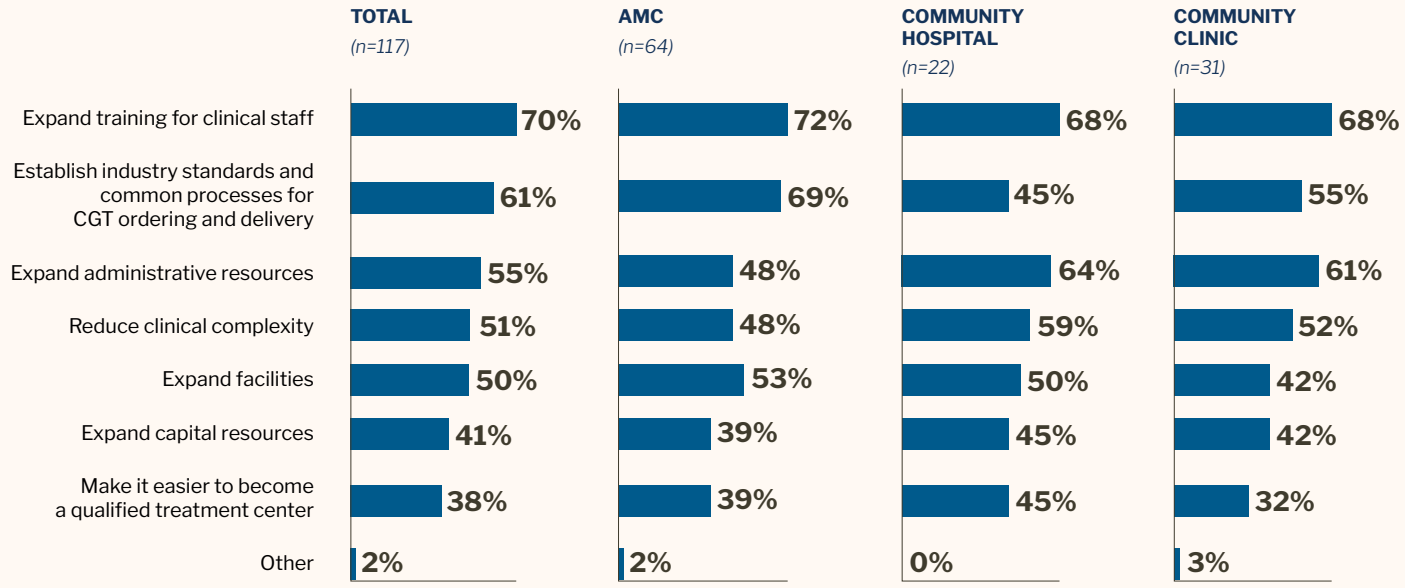


Base: Total Users (See chart)

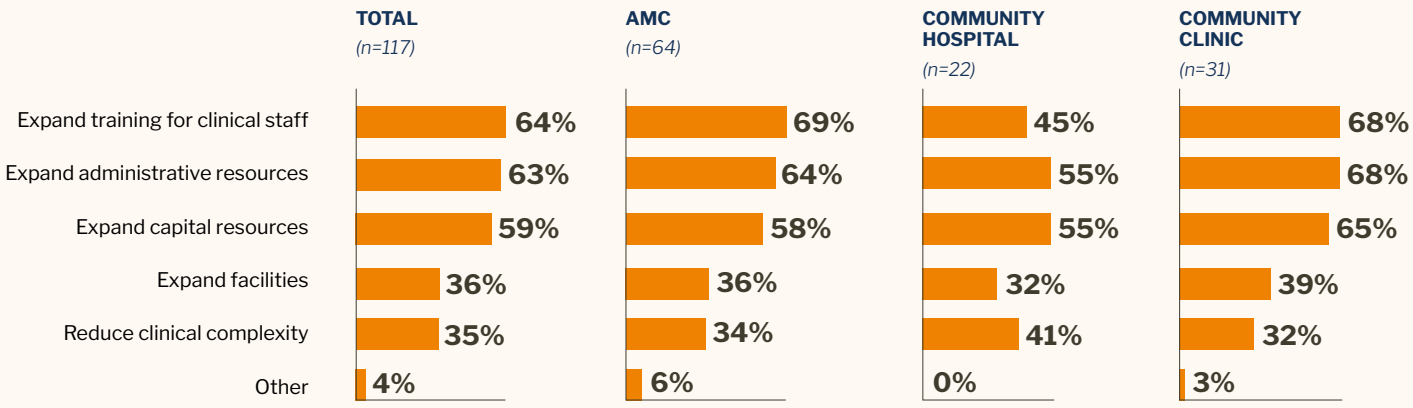
Note: Total may not sum to 100% due to rounding

Additional clinical training, increased resources, and established processes and standards are necessary to expand CGT programs.

Which of the following solutions are needed to ensure CGTs can be expanded in community sites-of-care? Select all that apply.



Which of the following solutions are needed so the capacity of CGT programs can be expanded at academic medical centers? Select all that apply.



Base: Total Users (See chart)

Expert perspective: ROBB RICHARDS

‘Hub and spoke’ model may enable access to broader CGT care.

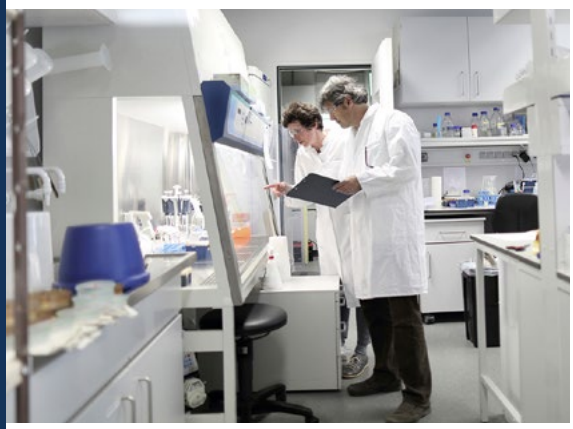
“These therapies are getting approvals for earlier lines of use, which means larger populations of patients are going to become eligible for these treatments. So I certainly hope [the healthcare industry] prepares for that wave that’s coming.”

— Robb Richards

Corporate Director of Cell Therapy and Transplant University of Pennsylvania

A massive wave of new cell and gene therapies is approaching, and Robb Richards is trying to prepare the healthcare industry for the swell as best as possible.

Richards, Corporate Director of Cell Therapy and Transplant at University of Pennsylvania, has worked in oncology for two decades, and has seen many changes in treatment options during the course of his career. Penn Medicine is also at the forefront of medical research, having pioneered the first human trial of chimeric antigen receptor CAR T therapies and holding one of the highest number of patents and FDA approvals of any academic medical center in the nation.



From his position as a leader at one of the most active academic medical centers in cell and gene therapy, Richards understands the challenges facing academic medical centers that are trying to expand their offerings to meet the growing demand for these innovative medicines. To accommodate this paradigm shift, there will need to be new standards, new practices, new technologies, and new ways of doing business.

“These therapies are getting approvals for earlier lines of use, which means larger populations of patients are going to become eligible for these treatments,” Richards said. “So I certainly hope [the healthcare industry] prepares for that wave that’s coming.”

At the forefront of this wave are CAR T therapies. First approved by the FDA in 2017, six drugs are currently available in the U.S. for a range of cancer indications, including blood cancers such as lymphomas, some forms of leukemia, and multiple myeloma—with that list of potential indications only expected to grow. The potential enhanced efficacy of these therapies has generated excitement among healthcare providers and patients, but the complex manufacturing and administration of these therapies, as well as capacity issues at sites of care, have limited their use to date.

That hasn’t stymied drug developers: approximately 1,000 CAR T therapies are in development, with 97% focused on treating cancer, according to the American Society of Gene and Cell Therapy Q2 2024[1] report. And with recent studies indicating these drugs may be helpful in other conditions, such as autoimmune diseases, more research is on the way.

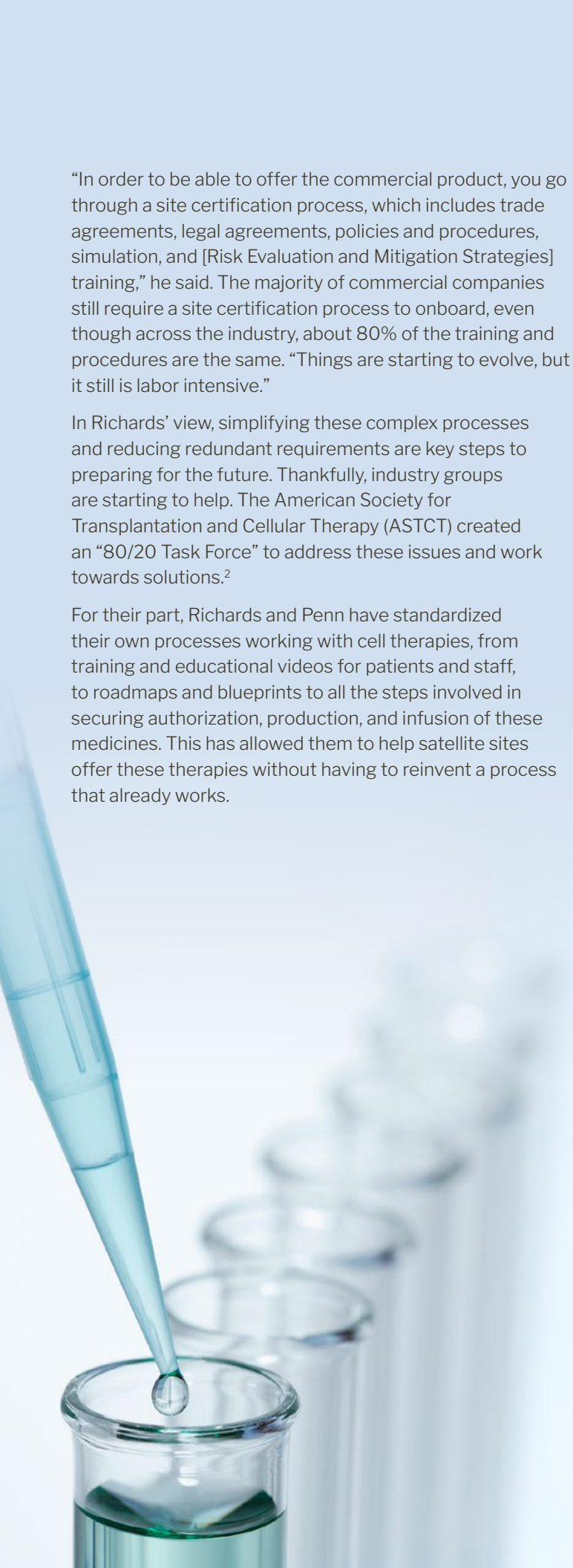
“The existing infrastructure is insufficient to manage the growing number of patients requiring these therapies,” Richards said. “Now the question is, how do we organize and standardize as we go forward?”

When he arrived at Penn Medicine in 2016, the hospital was performing around 250 bone marrow transplants a year. Now, they are performing 600 procedures, encompassing CAR T therapies, bone marrow transplants, and research cell therapies. Getting there wasn’t easy—Richards recalls having to train 700 staff members between Thanksgiving and Christmas one year to prepare for the surge of procedures and new treatments and ambiguous training guidelines.

"In order to be able to offer the commercial product, you go through a site certification process, which includes trade agreements, legal agreements, policies and procedures, simulation, and [Risk Evaluation and Mitigation Strategies] training," he said. The majority of commercial companies still require a site certification process to onboard, even though across the industry, about 80% of the training and procedures are the same. "Things are starting to evolve, but it still is labor intensive."

In Richards' view, simplifying these complex processes and reducing redundant requirements are key steps to preparing for the future. Thankfully, industry groups are starting to help. The American Society for Transplantation and Cellular Therapy (ASTCT) created an "80/20 Task Force" to address these issues and work towards solutions.[2]

For their part, Richards and Penn have standardized their own processes working with cell therapies, from training and educational videos for patients and staff, to roadmaps and blueprints to all the steps involved in securing authorization, production, and infusion of these medicines. This has allowed them to help satellite sites offer these therapies without having to reinvent a process that already works.



"What I found to be most powerful is for people to understand the episode of care broadly by making it visual," he said. So his team created process maps that laid out the entire episode of care, including everybody's responsibility from beginning to end. These blueprints help to understand the delivery of care and mitigate risk that can occur when administering CGTs. "It really calmed the waters with the C-suite folks about their concerns about delivering and treating patients with a half-a-million-dollar therapy that they've never done before."

The broader solution, however, for helping more people access CGTs, is greater collaboration across the spectrum of healthcare. The model Richards envisions is one where the large academic medical centers focus on newer treatments, including early stage clinical trials, to work out the clinical value and potential adverse events, and let community-based healthcare systems take more established therapies and later stage clinical trials. He calls this the hub and spoke model.

"Centralizing treatment through this model increases accessibility and allows more patients to benefit from advanced therapies without having to travel to large academic centers," Richards said.

This model not only enhances patient access but also helps distribute the burden of care more evenly across the healthcare system, ensuring that high-quality treatments are available to a broader population. However, this type of shift in where and how patients receive CGTs, and participate in clinical trials, will require substantial work among all the parties involved, which not all external stakeholders are ready to embrace. Still, Richards sees it as the best way to ensure patients receive the best treatment options.

"Collaboration, education, and standardization are key to overcoming the barriers and leveraging the opportunities in expanding cell and gene therapies to a broader patient population," Richards said.

Expert perspective: MARK TRUSHEIM

The price we pay: Innovative approaches to paying for cures.

In an era of revolutionary biological advancements, where a single dose of a cell or gene therapy can replace years of medical treatments, one key question remains: how do we most appropriately pay for these medicines?

This topic is one that Mark Trusheim, Strategic Director of NEWDIGS (New Drug Development Paradigms), at Tufts Medical Center, has lived and breathed for years. NEWDIGS, a "think and do" tank, launched in 2009 to help the broader healthcare system—payers, patients, providers, manufacturers, and policymakers—catch up with the pace of scientific innovation.

"The downstream system has to be as inventive and creative as the upstream science if patients are going to gain access and benefit from these exciting therapies," Trusheim said.

Trusheim advocates a rethinking of the current approach to paying for CGTs up front and in full, especially as these complex medicines can provide years of medical benefits with a single dose. Just as a consumer wouldn't purchase a home by putting the cost on their credit card, Trusheim says the healthcare industry needs to consider innovative payment models that look at the long-term value these medicines provide patients as well as the long-term savings they will generate when compared to traditional medical treatments.

For certain genetic conditions, such as sickle cell disease (SCD), gene therapies can offer much greater value to patients with a single dose when compared to the cost of a lifetime of treatments. The Institute for Clinical and Economic Review (ICER), an independent nonprofit research institute that analyzes the effectiveness and value of drugs, estimated the health benefit price benchmark of two recently approved gene therapies for SCD ranged from $1.35 million to $2.05 million—approximately the net price Medicaid might expect after 23.1% mandatory discounts off the commercial market average manufacturer price (AMP). In its report,[1] ICER said these medicines "are likely to substantially improve quality and length of life among patients."

The question the industry needs to answer, as new CGTs for more common conditions, such as heart disease or diabetes make their way through the development pipeline, is how to change our healthcare system to account for treatments that are given once instead of indefinitely over a lifetime. A study of the economic cost of diabetes in the U.S. pegged the figure at $412 billion in 2022,[2] or an average annual medical expenditure of $19,736 per patient. "The enormous economic toll of diabetes continues to burden society through direct medical and indirect costs," the study concluded.

Comparing such costs to the benefit of a lifetime cure with a single-dose gene therapy can be difficult, considering the issue of patient adherence to classic therapies, which can lead to more expensive care.

"What is the real-world outcomes difference?" Trusheim asked. "Because the current standard of care is never administered perfectly and adhered to perfectly. And so the value delivered is inherently less, thus increasing the comparative value of gene therapies, which do not depend upon patient adherence."

Trusheim suggested several payment models that could help the healthcare system digest higher upfront costs of CGTs, such as value-based contracts

"The downstream system has to be as inventive and creative as the upstream science if patients are going to gain access and benefit from these exciting therapies."

— Mark Trusheim

Strategic Director of NEWDIGS (New Drug Development Paradigms), at Tufts Medical Center

and performance-based annuities. Another innovative payment system could include a national pooling of people with rare conditions, to help distribute the costs of CGTs across a broader base and help payers and healthcare systems stay financially solvent.

The U.S. Centers for Medicare and Medicaid Services (CMS) is taking up its own initiative to help negotiate payment plans, called the Cell and Gene Therapy Access Model. The program, which launches in 2025 for SCD gene therapies, allows CMS to negotiate outcomes-based agreements on behalf of state Medicaid programs that choose to participate, potentially standardizing access and payment for these therapies across states and reducing the state plan administrative burdens.

This is a great step toward building new models that help ensure patients will be able to receive cell and gene therapies, Trusheim said.

"I'm hoping we'll continue to build on this confluence of exciting science, smart investment in and development of transformative therapies, and innovative payment approaches to provide patient access, affordability, and incentives for more," he said. "We can create a phenomenal positive cycle for benefiting from cell and gene therapies."

Map of CGT qualified treatment center locations

Expanding into ‘CGT deserts’: Ensuring equitable access to state-of-the-art therapies

As cell and gene therapies (CGTs) continue to transform treatment for a growing number of conditions, a critical challenge has emerged: ensuring equitable access to these potentially life-changing therapies. When discussing access, we often think about socioeconomic issues, but for CGTs another roadblock to care has arisen: geography.

Today, the vast majority of CGT treatment is concentrated at large academic medical centers in major metropolitan areas. This isn't surprising when one considers the complexity of CGT administration. Hospitals providing these personalized medicines must have special facilities, equipment, and highly trained staff to manage the complicated treatment process, including cell collection, infusion, and post-treatment care and monitoring. While major academic medical centers generally have the resources necessary to meet these requirements, smaller community-based hospitals and practices may struggle with the required investment.

For most Americans who don't live near a major academic medical center, these innovative therapies aren't readily available, creating vast "CGT deserts." And while traveling a long distance may not seem like a big hurdle to access a potentially life-saving medication, the required month-long stay near the hospital—with a caregiver—is often too disruptive or burdensome for many patients and their families to shoulder. A study from earlier this year noted that the likelihood of receiving a CAR T-cell therapy was reduced by 40% when patients lived two to four hours from their nearest treatment center.[1]

Although the centralization of CGT expertise in urban academic medical centers was a natural starting point for these complex therapies, as CGTs move from treating rare diseases to more common conditions with larger patient populations, the current infrastructure of care will become increasingly unsustainable and inequitable.

Expanding CGT access into community hospitals and clinics is necessary to support patient care, but it is fraught with challenges that range from insufficient resources and regulatory barriers to insurance roadblocks.

In this section of the report, we dive deeper into these challenges and discuss possible solutions with two pioneers in the field.

Dr. Gary Simmons of Virginia Oncology Associates, who has performed more than 20 CAR T procedures in the outpatient setting, shares his experiences building a CGT practice within a community clinic and provides advice for others looking to do the same.

Rocky Billups, Vice President of Operations, HCA Healthcare/Sarah Cannon Cancer Network (SCCN)—one of the nation's largest providers of cell therapies and stem cell transplants—provides his perspective on the requisites to move CGT care into the community, and what role institutions like SCCN need to play.

We also sought to understand the geographic challenge through data. This section features a map developed by McKesson (along with a link to an interactive tool) to visualize where qualified cell and gene treatment centers across the U.S. are located, and where care deserts exist. It illustrates a fact many doctors and patients already know: that while use of CGTs is expanding, many patients still live hours from their nearest care facility—putting advanced care out of reach.

On the map: Locations of qualified treatment centers (QTCs) for cell and gene therapy across the U.S.

As many as 60 million Americans[2] live in rural areas with limited access to CGT qualified treatment centers

Our research into qualified cell and gene treatment centers identified a total of 512 named centers within 272 parent institutions. A total of 135 of the QTCs are pediatric-focused institutions, which reflects the substantial proportion of CGTs that are indicated for children.

The QTCs we identified are located in 109 metropolitan regions. While it is unsurprising to see that CGT care is concentrated in densely populated urban areas, the map clearly illustrates the challenge facing patients who live in more rural areas.

This geography issue is not uncommon—advanced care is centered in urban areas across nearly all facets of medicine. However, CGT patients face a unique burden because they must stay near a treatment center for weeks or even months following a procedure.

Data Sources
All data sourced by Health Market Experts LLC as of July 2024. Sources included the American Society of Gene and Cell Therapy (ASGCT), the Foundation for the Accreditation of Cellular Therapy (FACT), BMT Infonet, and product websites of numerous cell and gene therapies.

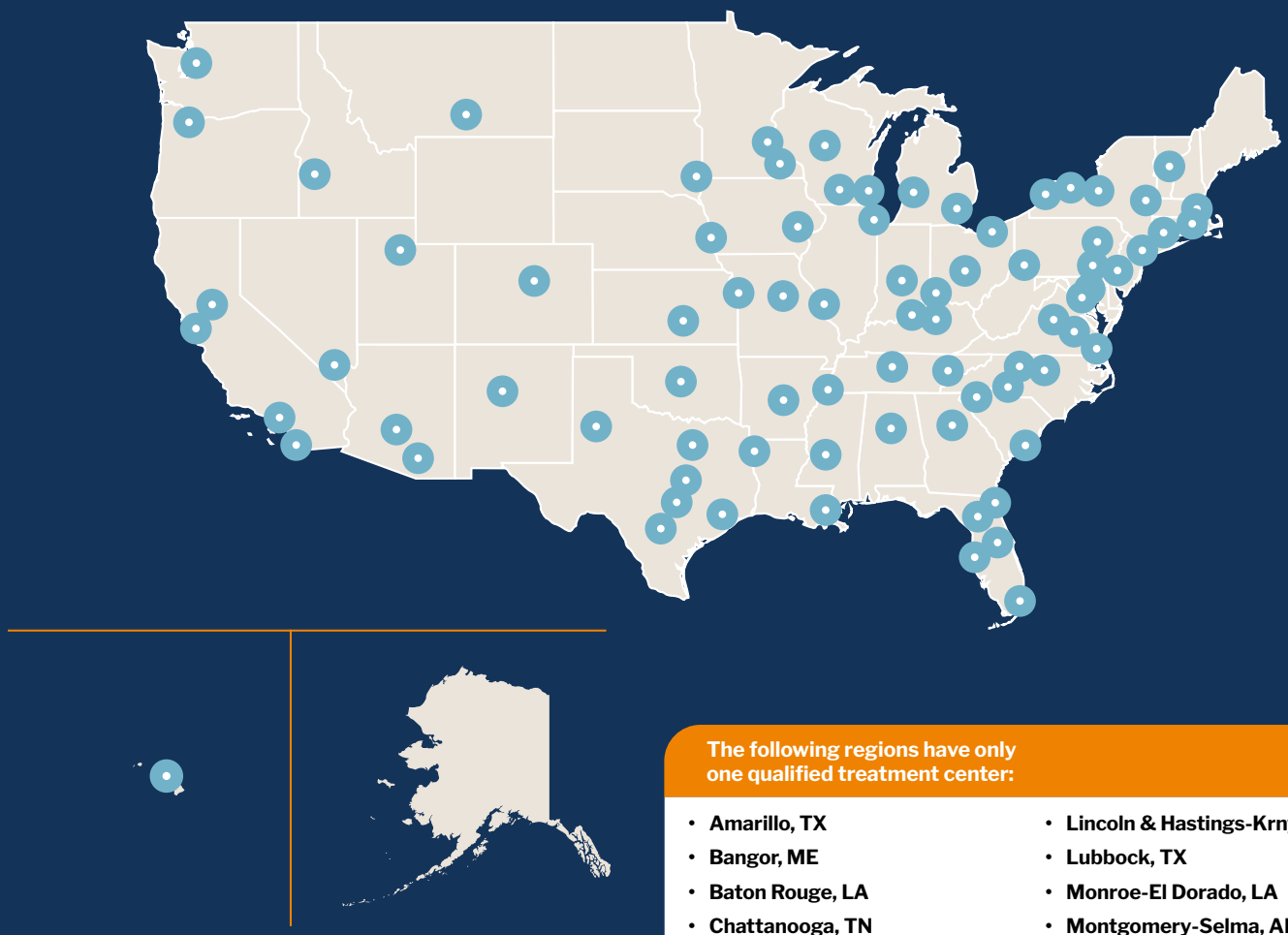
Each dot represents a region or metropolitan area with one or more Qualified Treatment Centers (QTCs)

Methodology
We organized the list of qualified treatment centers by city, state, and ZIP code, and then segmented the list using Designated Market Area (DMA) data, which defines distinct markets or metropolitan areas across the U.S. using ZIP codes.

To view our interactive map of CGT qualified treatment centers across the U.S., visit **https://inspirogene.com/thought-leadership/qtcmap/**

Cell therapy QTCs

While availability of CAR T cell therapy is expanding, treatment centers are still concentrated on the eastern side of the U.S. and on the West Coast



The following regions have only one qualified treatment center:

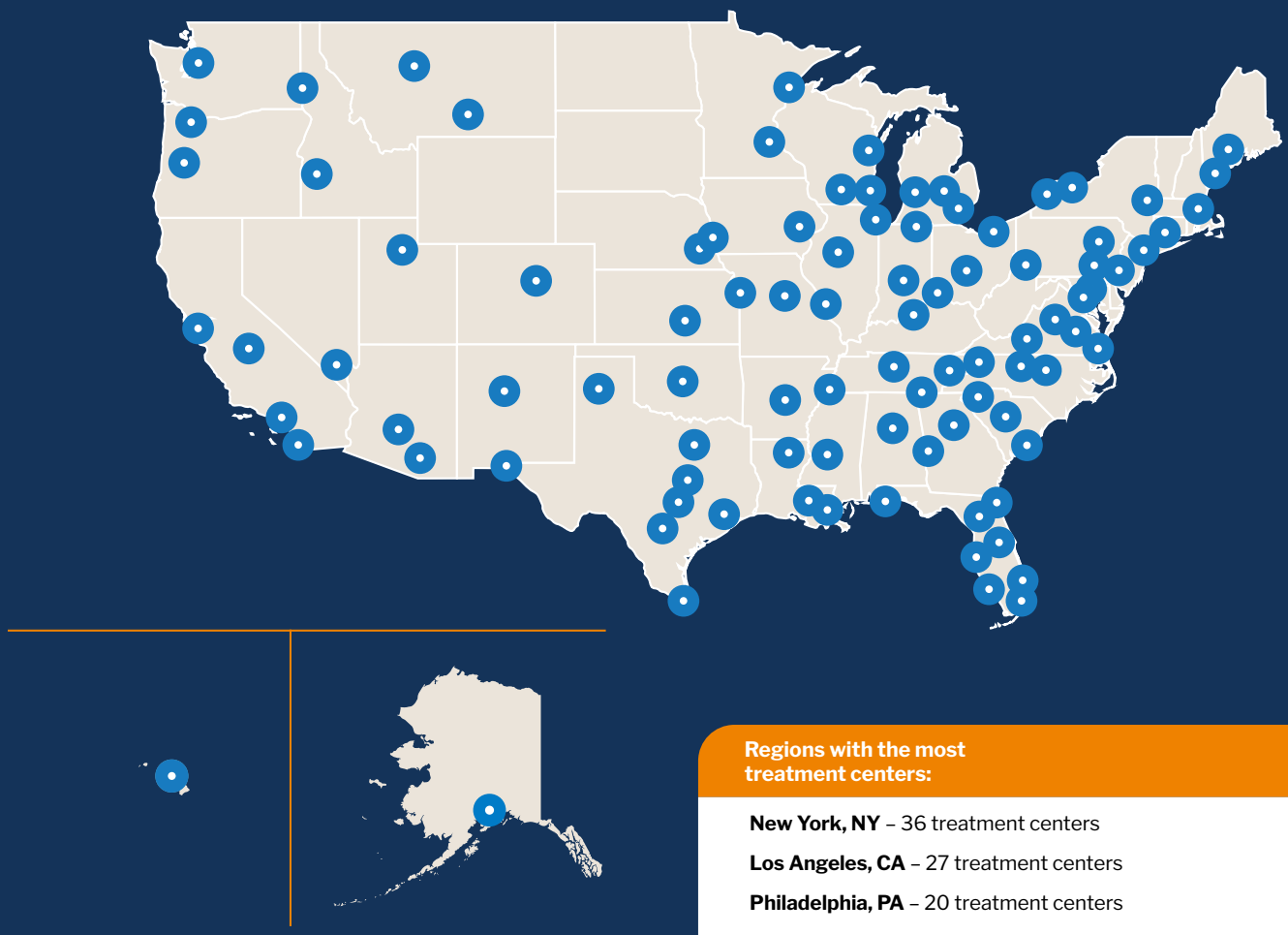
- Amarillo, TX
- Bangor, ME
- Baton Rouge, LA
- Chattanooga, TN
- Duluth, MN
- El Paso, TX
- Eugene, OR
- Flint-Saginaw-Bay City, MI
- Great Falls, MT
- Harlingen-Weslaco-Brownsville-McAllen, TX
- Kansas City, MO
- La Crosse-Eau Claire, WI
- Lexington, KY
- Lincoln & Hastings-Krny, NE
- Lubbock, TX
- Monroe-El Dorado, LA
- Montgomery-Selma, AL
- Peoria-Bloomington, IL
- Portland, ME
- Providence, RI
- Roanoke-Lynchburg, VA
- Rochester, MN
- Shreveport, LA
- South Bend-Elkhart, IN
- Spokane, WA
- Syracuse, NY
- Tri-Cities, TN-VA

Data Sources
All data sourced by Health Market Experts LLC as of July 2024. Sources included the American Society of Gene and Cell Therapy (ASGCT), the Foundation for the Accreditation of Cellular Therapy (FACT), BMT Infonet, and product websites of numerous cell and gene therapies.

Gene therapy QTCs

Although access remains limited nationwide, some gene therapies have begun extending care into regional hospitals and clinics

Each dot represents a region or metropolitan area with one or more Qualified Treatment Centers (QTCs)



Regions with the most treatment centers:

- **New York, NY** – 36 treatment centers
- **Los Angeles, CA** – 27 treatment centers
- **Philadelphia, PA** – 20 treatment centers
- **Chicago, IL** – 16 treatment centers
- **Boston, MA (Manchester, NH)** – 15 treatment centers
- **Atlanta, GA** – 12 treatment centers
- **Detroit, MI** – 12 treatment centers
- **Houston, TX** – 12 treatment centers
- **Miami-Fort Lauderdale, FL** – 12 treatment centers
- **Nashville, TN** – 11 treatment centers
- **San Francisco-Oakland-San Jose, CA** – 11 treatment centers

Methodology
We organized the list of qualified treatment centers by city, state, and ZIP code, and then segmented the list using Designated Market Area (DMA) data, which defines distinct markets or metropolitan areas across the U.S. using ZIP codes.

To view our interactive map of CGT qualified treatment centers across the U.S., visit **https://inspirogene.com/thought-leadership/qtcmap/**

Expert perspective: GARY SIMMONS, DO, MSHA

Bringing cell and gene therapies into community practice: a pioneer shares his playbook.

> More programs in the community means more access to patients.
>
> **— Gary Simmons, DO, MSHA**
> *Oncologist/Cell Therapist, Virginia Oncology Associates*

Gary Simmons is nothing if not tenacious. The hematologist oncologist and father of five knows a thing or two about endurance, having completed several 100-mile ultra-marathons. It's that unrelenting drive that has helped him break down barriers to bring new cell and gene therapy treatments to patients at Virginia Oncology Associates (VOA), a large community oncology practice in Virginia. Simmons and the VOA team are early leaders in a growing movement to transition more cell and gene care into the community practice setting.

Today, most CGTs are administered in large health systems and academic medical centers, typically located in metropolitan areas—which leaves patients living in smaller communities with limited access to experts and new treatments. Simmons, a leader in community transplant and cellular therapy at VOA, has taken steps to help ensure more patients—who may not live close to these medical centers—have access to the latest treatments closer to home.

He credits the organization's partners' and administrators' prior work building a robust infrastructure for stem cell transplants for enabling the practice to earn certification as an approved treatment center for CAR T therapy. To date, VOA, which is part of The US Oncology Network, has performed in excess of 20 CAR T procedures in the outpatient setting and is participating in dozens of clinical trials involving other cell therapies, making it one of the more active community oncology practices in the country.

"I never want to be in a position where I can't provide a therapy for my patients. Recently, a study showed patients more than 120 minutes from an authorized center are 40% less likely to get CAR T-cells," Simmons said. "We believe we can deliver these therapies quickly, safely, and less expensively than traditional large academic centers."

Still, the journey hasn't been easy. The requirements for providing CAR T therapies are numerous, involving specialized training, dedicated facilities, and timely reimbursement. Simmons said addressing these barriers as a community practice requires coordinated support from healthcare providers, policymakers, drugmakers, and insurers to ensure that more patients can benefit from these advanced treatments.

"I'm hoping we can start breaking down some of those barriers," Simmons said. Without more community practices offering these treatments, patients "will settle for potentially inferior therapies due to lack of access, which is what this comes down to ultimately. More programs in the community means more access to patients."

One of the biggest hurdles Simmons has had to overcome in treating patients with CAR T at VOA is resistance from payers. Many insurance companies, in Simmons' experience, require patients receive such treatments at academic medical centers, which are viewed as having more experience and more ability to invest in CGT than most community-based practices.

The concerns aren't without merit—CAR Ts, while potentially curative, can also cause severe immune system reactions weeks after treatment, which may require hospitalization. For this reason, patients are often required to stay for a month near the hospital where they receive treatment with a caregiver, for emergencies. But many times, patients and their caregivers can't leave their families, jobs, and responsibilities behind for such an extended period of time.

VOA has developed its team, added resources and proved it can handle administering CAR Ts in the community setting, but Simmons said payers still deny coverage for some patients, insisting that these patients travel for treatment at large health systems, often hundreds of miles away—despite the data showing that distance is a barrier to CAR T treatment.[1]

"So we appeal it and continue to push, until eventually the insurer relents, or the patient decides to go for a less optimal treatment option," Simmons said.

Simmons says more community practices need to be included in future plans for CGTs because—while 85% of patients with cancer are treated in the community[2]—cell and gene treatments aren't reaching the majority of those patients.

For other community practices that are looking to expand into CGT, Simmons has the following advice:

- **Leverage expertise:** Pharmaceutical companies have onboarded programs for years and can provide advice and guidance to help prepare a community practice, including insights into payers, government agencies, and regulators to help establish a cell therapy treatment center.
- **Obtain accreditation:** The Foundation for the Accreditation of Cellular Therapy (FACT) designation can be difficult to earn due to its rigorous requirements, but can be crucial for performing certain types of cell therapy. FACT accreditation is not required at the moment, but many payers will look for accredited programs.
- **Invest in staff training:** Running a CGT center requires specialized education for physicians, nurses, and other support staff. Community practices should offer resources to ensure staff are up to date on their training for these therapies.
- **Develop a broad network:** Establish relationships with community oncologists, other state-based local healthcare practices, as well as large academic medical centers to ensure patients get the right treatment if adverse events arise.

Simmons thinks other community practices can be part of the expansion of CGTs with the right leadership and commitment.

At VOA, "the physician-run leadership has been tremendously supportive of CGTs and forward thinking," he said. "And cell therapy is something we should all be really looking deeply into in our programs because it's not going away. At the end of the day, it's in the best interest of the patients that need these programs in their communities."

Expert perspective: ROCKY BILLUPS

Scaling the healthcare industry to meet growing demand for cell and gene therapies.

As physicians and patients eagerly await new cell and gene therapies with the potential to save lives, the hospitals that conduct clinical trials and administer approved therapies are exploring the best ways to build scale across the healthcare industry to meet the expected demand.

This is a challenge that Rocky Billups, Vice President of Operations, HCA Healthcare/Sarah Cannon Cancer Network (SCCN), thinks about often. Sarah Cannon comprises 180 hospitals, including more than 70 Commission on Cancer accredited hospitals. The organization's Transplant and Cellular Therapy Network is the largest provider of hematological cancer treatment in the U.S., administering approximately 1,600 stem cell transplants and cell therapies in 2023 alone.

With more than 4,000 CGTs in development,[1] the healthcare industry will need to explore new ways of working to ensure patients will be able to access these medicines—particularly as they are used in diseases with larger patient populations including diabetes and autoimmune diseases. SCCN is forging a path forward to help address capacity concerns by exploring new treatment settings, establishing unique partnerships with other stakeholders, and investing in training and education programs.

"Right now, we've got this small population of blood cancer patients," Billups said. "But once we figure out how to do it right in solid tumors and then in autoimmune disease, there is not enough capacity within our hospitals across the United States to meet this demand."

Billups suggests that one solution to freeing up capacity, and by extension expanding access to more community care centers, is rethinking how providers monitor patients after a drug is administered. Some CGTs, such as CAR T-cell therapies, can cause a severe immune reaction, prompting hospitals to require patients to remain in or near an inpatient facility for several weeks.

However, SCCN found it could safely monitor some patients remotely, using wearable devices and a dedicated team to monitor and triage any issues that occurred. As a result of this program, the network was able to transition 75% of patients treated with CAR T-cell therapies to an outpatient setting, saving more than 1,200 hospital bed days.[2]

Approximately 20% of patients were able to avoid the hospital altogether, not only decreasing the burden for patients, but also saving resources for others with more critical needs. While this approach won't work for every patient, if some CAR T-cell therapy can be administered in this way, more community oncologists will be able to provide the therapy, helping expand access.

Another key barrier to scaling cell and gene therapy is the shortage of physicians, nurses, and staff with the specialized expertise to administer these medicines, manage cell collection, and provide care for patients' unique needs. SCCN recognized that to address the staffing shortage, they would need to "grow their own" specialized team. That's one of the reasons HCA Healthcare acquired Galen College of Nursing[3] and invested more than $300 million[4] to expand training programs and open new locations near their hospitals.

"We've partnered with them to develop an oncology curriculum and electives, to really start getting nurses interested in this area as they go through school," Billups said.

Billups says they are also developing fellowships to train physicians, and recently graduated their first fellow. But the investments of one institution won't be enough—other hospitals will need to make similar moves to address the staffing gaps.

Reducing complexity is also key to scale. SCCN has streamlined how CAR T-cell therapies are ordered and administered across all centers, making it easier to onboard new staff and expand operations. They hold regular meetings among physicians to sync up on best practices and ensure the use of uniform eligibility criteria for patients.

"We all talk to each other about ways can we bring down the barriers to access for patients. Our focus is on doing what's right for the patients and making it safe."

— Rocky Billups

Vice President of Operations, HCA Healthcare/Sarah Cannon Cancer Network

"The policy procedures are the same, the clinical pathways the same," Billups said. "So that's why I'm confident we'll open up at least five new centers in the next two to three years."

Billups sees a need to help oncology practices prepare during the next few years as more CGTs shift to the community setting. That's why SCCN shares their expertise and resources with medical oncology groups outside of their official network.

"We are partnering with medical oncology groups in some of the markets where we have hospitals to say 'Let's work together to figure this out,'" Billups said. "We are happy to coordinate the care for patients to determine the best site of care for them."

Collaborations of this type are just one example of how stakeholders are working together to prepare for the surge of CGTs expected to come to market in coming years.

"We all talk to each other about ways we can bring down the barriers to access for patients," Billups said. "Our focus is on doing what's right for the patients and making it safe."

A call-to-action for CGT stakeholders

What's next for cell and gene therapies: addressing challenges to advance the state of the industry

Over the course of this report, we have presented data and expert opinions that have made a strong case for the significant role cell and gene therapies (CGTs) will play in advancing health outcomes across a wide range of conditions. The clinicians and industry experts we spoke with agree this new era of medicine will be unprecedented in improving patient outcomes. Still, challenges related to manufacturing, clinical and commercial infrastructure, reimbursement, and administration will need to be resolved before this future becomes a reality. Below, we share key takeaways discussed in this report and offer some potential solutions to advance medicine and better serve patients in great need.

CGTs have far-reaching potential, but face challenges

Since their debut in the past decade, CGTs have changed health outcomes for many patients across a range of diseases and conditions. Genetic conditions, such as sickle cell disease and spinal muscular atrophy now have multiple disease-modifying therapeutic options. In oncology, CAR T therapies have reshaped the treatment landscape for several forms of blood cancers, including lymphomas, leukemia, and multiple myeloma.

Our survey of oncologists showed near universal agreement that these treatments are "among the most important medical innovations of our time," especially given their curative potential.

New therapies continue to emerge. In just the past year, two gene therapies for sickle cell disease were approved, as well as the first approvals of CGT therapies in solid tumors—AMTAGVI (lifileucel),[1] a cell therapy for melanoma; and TECELRA (afamitresgene autoleucel),[2] a T-cell receptor (TCR) gene therapy for unresectable or metastatic synovial sarcoma.

Currently hundreds of CGTs are in late-stage clinical trials,[3] including drugs for diseases with broader patient populations, such as diabetes, cardiovascular disease, autoimmune disorders, and osteoarthritis. A number of therapies already approved as second-line or later treatments for cancer are also being tested against first-line therapies, which could lead to their broader use.

> **"Logistics requirements are not always clear across this industry today. As a result, manufacturers are increasingly reliant on experienced logistics partners who not only have the necessary cold-chain resources and technology, but also the expertise in cell and gene."**
>
> **— Joe DePinto**
> *Head of Cell, Gene, and Advanced Therapies*
> *McKesson*

While these developments have raised expectations for future improvement in patient outcomes, the commercial landscape for many of these products is fraught with challenges.

In hemophilia, the FDA approved three disease-modifying therapies since 2022, offering patients a durable treatment to help avoid frequent hospitalizations and other significant medical issues from the condition. However, sales of these medicines lag expectations[4] due to several factors, including patient satisfaction with their existing treatment options and uncertainty about the long-term durability of these new medicines.[5]

Hemophilia is not the only therapeutic area to experience these trends—uptake of new CGTs is slow across many therapeutic areas, leading some companies to re-evaluate their investments in CGT. Since 2021, more than 50 gene therapy programs have been abandoned or deprioritized because they weren't viewed as commercially viable.[6] At the same time, venture capitalists are pulling back on investments in the CGT space.

Continued on following page

According to Nature, venture financings in CGT to date in 2024 are "down steeply, reflecting clinical, manufacturing, and commercial hurdles."[7]

Overcoming the challenges facing CGTs and paving the way to commercial success is critical to ensuring that these life-saving therapies reach patients. Today, the primary hurdles include how we manufacture CGTs, how we pay for them, how and where we administer them, and the logistics of the supply chain. The next section of the report focuses on key solutions that, with support from stakeholders across the industry, may help to address the obstacles facing CGT and accelerate broader adoption.

Innovation is needed to scale manufacturing

As promising as the CGT pipeline is, the industry still needs to address significant manufacturing challenges that limit wide scale production if these medicines are to reach patients. The complex, labor-intensive processes involved in producing CGTs—which require the modification of genetic material—make it difficult to scale up, while maintaining quality and consistency. This limitation restricts production capacity and results in long waiting times for patients.[8] While CGT manufacturing has progressed, more advancements are needed.

CAR T producers have made strides in expanding capacity, while reducing production times. Gilead, for instance, was able to reduce the manufacturing process for its CAR T-cell therapy, YESCARTA (axicabtagene ciloleucel), to an industry-leading 14 days from 16 days after the FDA approved a manufacturing change earlier this year.[9]

Some companies are working on new and improved methods to reduce production costs. Ori Biotech launched a manufacturing platform, IRO, which automates the most manually intensive part of scaling up CGTs from candidate selection to product formulation, including activation, transduction, expansion, and harvesting cells. This method reduces labor needs by as much as 70% and costs by as much as half,[10] and allows digital synchronization through the cloud to distributed production sites.

This type of platform can help companies shorten their time to clinic and seamlessly transition to commercial-scale manufacturing. More importantly, Ori Biotech's system can be deployed at sites closer to patients, further reducing the time needed to transport cells and therapies between manufacturer and hospital.

The CGT industry needs to continue investing in manufacturing innovation, from technologies to improve production, scale, and efficacy, as well as new models that involve decentralizing and automating CGT manufacturing. By placing smaller, digitally connected manufacturing hubs closer to major hospitals, biopharma companies can reduce transportation times and logistics hurdles that currently complicate the process of collecting a patient's cells, shipping them to the manufacturer to be modified, and then shipping the drug back to the hospital.

"As an industry we need to focus on product viability, alongside safety and efficacy early in pre-clinical development, to ensure these incredible products are able to make it to all patients who need them," said Jason C. Foster, CEO, Ori Biotech.

Through his work with CGT manufacturers at McKesson, Joe DePinto, Head of Cell, Gene, and Advanced Therapies, says continued innovation in technology is key to scaling manufacturing to meet greater demand and reducing the complexities of the supply chain.

"A lot of companies use a centralized distribution model where all production is regulated within one contract manufacturer or a single company-owned manufacturing site," DePinto said. "But we need to explore decentralized models, and in my mind, there's zero doubt that there has to be a digitization component to the manufacturing process in order for it to scale up to meet the demand."

Shared standards and streamlined processes will alleviate burdens on care sites

One challenge consistently mentioned by CGT experts in this report was the need to simplify and standardize administrative and operational processes for cell and gene sites of care. Becoming a qualified treatment center for CGTs is a considerable burden for clinical sites—even those already certified for other CGTs. Each manufacturer has unique requirements, resulting in a complex qualification process, which can take months to complete.

> **"As an industry we need to focus on product viability, alongside safety and efficacy early in pre-clinical development, to ensure these incredible products are able to make it to all patients who need them."**
>
> **— Jason C. Foster**
> *CEO, Ori Biotech*

Efforts to streamline these processes are under way. The American Society for Transplantation and Cellular Therapy (ASTCT) launched the "80/20 Task Force" to standardize 80% of manufacturers' requirements.[11] This initiative aims to alleviate the administrative burden, speed up the qualification process, and enhance consistency across manufacturers.

The Foundation for the Accreditation of Cellular Therapy (FACT) plays a pivotal role, providing rigorous accreditation necessary for many clinical sites. FACT accreditation requires comprehensive documentation, on-site inspections, and continuous compliance with high standards in clinical care, laboratory practices, and quality management.[12]

But industry associations alone cannot solve the challenge—reducing complexity will require collaboration among all of the stakeholders.

On the manufacturer side, companies need to agree to joint safety training standards required by hospitals to offer one of their CGT products. The current need for hospital staff to train separately for each drug, when a majority of the training is the same, drains resources that smaller institutions don't have. By simplifying the process and making it more "plug and play," hospitals will be able to train staff once on protocols for administering these therapies and offer more CGTs.

Health systems also have an important role to play in streamlining CGT processes. Penn Medicine is taking steps to tackle standardization within their network of hospitals by documenting processes around the three pillars of their program—clinical, financial, and operational—to ensure consistency among their teams across different sites of care. Robb Richards, Corporate Director of Cell Therapy and Transplant, credits these 'blueprints' with helping them to successfully expand their CGT program into community sites.

"What we ended up doing was developing process maps where we laid out the entire episode of care, everybody's responsibility from beginning to end, all the way through 30 days post infusion, inpatient and outpatient," said Richards.

Developing a standardized framework for delivering CGT has not only eased the burden on the sites of care themselves, it has also led to more productive discussions with manufacturers and payers about expanding care to community hospitals by showing specifically where the decision points are and how the hospitals mitigate risk, while still providing high-quality care.

While this framework has enabled Penn to scale its CGT program, Richards acknowledges that scaling the industry as a whole will require a broader solution and adds that, "All of the stakeholders are going to have to have skin in the game" to achieve critical mass and widespread standardization.

Standardization and transparency are needed in the CGT supply chain, says DePinto. CGT manufacturers must

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manage a complex set of variables including temperature control, shelf life, packaging, transportation, chain of identity, and chain of custody, when it comes to moving a drug from cell collection, to manufacturing to patient infusion.

"Logistics requirements are not always clear across this industry today. As a result, manufacturers are increasingly reliant on experienced logistics partners who not only have the necessary cold-chain resources and technology, but also, expertise in cell and gene," DePinto said.

Medical centers must take the lead in bringing care to the community

The current landscape, in which CGT care is largely centered at major academic medical centers in major metropolitan areas, has created vast "CGT deserts," leaving large portions of patients unable to access treatments.

Expanding care into the community setting will require academic medical centers, which already have expertise in CGT, to partner with community sites of care. Some collaboration is already happening. Richards describes an approach where major academic medical centers would focus on early-stage clinical trials for new treatments, and develop a framework for dealing with any adverse events that may arise, and then helping transition treatment to community hospitals.

"Once we know how to deliver care for these therapies safely at major academic centers, that's the moment we should be moving them into the community setting with this framework in place to mitigate risk," Richards said. "That would allow us to create additional capacity at academic sites to treat patients and keep moving the needle of science and medicine forward with newer therapies."

Academic medical centers can also play an essential role in facilitating discussions with CGT manufacturers and payers about moving care into the community, potentially removing a significant barrier.

"I have (pharma) companies in position willing to offer therapies in the community, and I have payers willing to pay for those therapies in the community. So you're trying to orchestrate the symphony in such a way that you can get them to play in concert," said Richards.

Another strategy to help share resources and learnings involves holding outreach clinics in communities that do not have the capabilities to offer these therapies, said Rocky Billups, Vice President of Operations at HCA Healthcare/Sarah Cannon Cancer Network (SCCN). Some patients may be hesitant to receive a new treatment at an unfamiliar facility that is located in another city or state. To overcome this, SCCN physicians and staff travel to remote sites to strengthen relationships with physicians and see patients in the community where they live. This also allows them to provide information about the latest CGTs to patients.

"I have (pharma) companies in position willing to offer therapies in the community, and I have payers willing to pay for those therapies in the community. So you're trying to orchestrate the symphony in such a way that you can get them to play in concert."

— Robb Richards

Corporate Director of Cell Therapy and Transplant University of Pennsylvania

These types of collaborations among academic centers, community practices, and clinicians will go a long way toward helping expand access, said DePinto.

"As we're starting to see expansion into community hospitals, we're also starting to see community clinics working together to identify the best treatment and site of care options for patients," DePinto said. "This is wonderful to see."

Investment in training future clinicians is critical

One simple reality that has become apparent to the hospitals and treatment centers wanting to offer CGTs is the shortage of specially trained nurses and doctors needed to administer these therapies. As demand for these medicines grows, investments in clinical education and training by hospitals and large practice groups are essential.

"A lack of nursing can be a barrier to offering cell and gene therapies," said Gary Simmons, who leads the transplant and cellular therapy team at Virginia Oncology Associates. "A hospital has to have nurses to administer chemotherapy and sub-specialized nurses to administer cell therapy, to look for cell therapy toxicities, to do home monitoring, and to be a coordinator for that patient."

Beyond nursing staff, administrative employees are needed to secure the therapy manufacturing slot from the drugmaker, schedule treatments, work with insurers, track patient test results and data required by the FDA, and coordinate ancillary care for patients during their weeks' long stay.[13]

More investment in medical education for current CGT clinicians and staff, as well as training for future practitioners is necessary. Already, collaborations are happening among hospitals, advocacy groups, drug manufacturers, and state agencies to funnel more resources for this purpose. The University of Florida College of Medicine, for example, is partnering with several groups to expand its Regional Center for Development of Advanced Therapeutics[14] to "advance these innovative faculty ideas across the academic health center and to train the next generation of clinician-scientists in advanced therapeutics."

Similarly, SCCN took steps to increase the number of CGT-trained nurses with its acquisition of Galen College of Nursing[15] and investment of more than $300 million[16] to increase training programs and open new locations near their hospitals.

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Educating the workforce on manufacturing CGTs is also a necessity. In North Carolina, the Research Triangle Region has attracted more than 600 life sciences companies and has become a hub for gene therapy research, as well as manufacturing.[17] To help local employers find the workers they desperately need, North Carolina State University developed special programs at its Biomanufacturing Training and Education Center for hands-on training in the manufacturing and analysis of adeno-associated virus (AAV),[18] a key ingredient for gene therapies. The program is so successful, it is being replicated by universities in other states.

While these examples are encouraging, many more programs like these are needed to support the expected demand as new CGTs are approved. States, universities, health systems, industry groups, and drugmakers all need to fund training programs to ensure patients are able to receive these medicines in the future.

Innovative payment models must be embraced to expand access

Managing the high cost of one-time CGTs remains a serious challenge in a healthcare system that is designed for pay-as-you-go therapies for chronic conditions. Addressing this challenge will require both innovation and collaboration across the ecosystem, not just the industry.

Over the past decade, healthcare stakeholders—including manufacturers, payers, and policymakers—have explored the use of outcomes-based payment models to help reduce the financial risks associated with these therapies.

When Novartis launched KYMRIAH (tisagenlecleucel) in 2017,[19] it initially offered a warranty program that guaranteed the product's performance for some indications—if patients did not respond within the first month, Novartis would refund the cost, providing a safety net for patients and payers.[20, 21]

Spark Therapeutics introduced an annuity-based payment model for LUXTURNA (voretigene neparvovec-rzyl), a gene therapy for inherited blindness. Payers could opt for a single upfront payment or a series of annual payments over five years, tied to the patient's visual acuity outcomes.

These are two examples, but there are many more. Rules issued by CMS in 2020[22] provided guidance for structuring alternative payment models to pay for high-cost advanced therapies, clearing the way for broader use of innovative payment plans going forward.

Mark Trusheim, Strategic Director of NEWDIGS at Tufts Medical Center, recommends that stakeholders need more creativity when it comes to thinking about how to pay for CGTs, especially when long-term outcomes aren't known up front. One idea is exploring public-private insurer partnerships to spread risk and create pools large enough to absorb the costs associated with CGTs.

"We have to demonstrate the financial sustainability not just for one of the stakeholders, but that the system works across all of them," Trusheim said. "A system that is affordable for providers, reduces financial toxicity for patients, and generates enough returns to attract investors and developers to continue funding research for new therapies."

Several states and federal agencies are investigating methods and models to cover costs of these therapies and alleviating the burden of treatments, including transportation, lodging, and follow-up visits. An assessment of state Medicaid programs found that many are exploring value- or outcomes-based agreements, with three states—Arizona, Massachusetts, and Michigan—structuring contracts with drug manufacturers for gene therapies that provide rebates if certain clinical outcomes are not met.[21]

The best path forward for paying for CGTs is yet to be determined, but with different innovative approaches by manufacturers, states, and CMS, the industry should continue to work with different stakeholders to structure agreements that best serve patients.[23]

Final thoughts

The CGT industry is at a pivotal moment, poised for growth but facing significant challenges. From manufacturing bottlenecks to the burden on clinical sites and the need for patient education, many areas need significant attention before broader adoption of these medicines becomes a reality.

With investment in innovative manufacturing technologies, efforts to streamline clinical site qualifications, and expansion of CGT education and training, the industry is making strides toward overcoming these hurdles. By embracing innovative payment models and partnering to broaden care into the community, the industry is building a foundation for sustainable growth.

We hope this report has been both eye opening and inspiring, and leads to productive conversations and collaboration among all CGT stakeholders. By working together to overcome existing hurdles facing CGTs, we can ensure that these innovative treatments reach the patients who need them most.

"More approvals are coming," DePinto said. "This is an exciting time to be working in cell and gene therapies."

Expert contributors

Joe DePinto
Head of Cell, Gene, and Advanced Therapies
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Joe DePinto has spent the past 30+ years focused on bringing life-saving specialty drugs to market, helping patients with complex diseases to access advanced care and helping to develop future leaders in the pharmaceutical industry. As Head of Cell, Gene, and Advanced Therapies at McKesson, he has responsibilities over working with teams and customers to find new and better solutions to today's cell and gene value chain challenges while looking toward the development and identification of strategies that can help meet tomorrow's opportunities. Joe's past roles have included leadership positions at top pharmaceutical companies including Johnson & Johnson, Lilly, Sunesis, and Dendreon. His core leadership competencies include driving all aspects of strategy, drug development, investor relations, and commercialization with multiple global launches. Joe holds a bachelor's in Marketing and an MBA with an emphasis on Pharmaceutical/Chemical Studies.

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Ellen Moore currently leads GlobalData's Cell and Gene Therapy Consulting team. She has 20+ years of experience in pharmaceuticals, both in industry and as a consultant. For the past 12 years, Ellen has worked across many biopharmaceutical segments and has assisted clients with issues such as strategic opportunity evaluations, portfolio prioritization, new product planning, and strategic partnering. Her work has supported the capital raises of dozens of companies totalling hundreds of millions of dollars as well as the successful sale of small biotech companies to large pharma players. Ellen started her career with roles in Marketing, Corporate Development, and New Product Planning at Elan Pharmaceuticals. Ellen earned an MBA from the Anderson School at UCLA and a Bachelor of Science in Mathematics from Chestnut Hill College.

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John Bishai is a Managing Director within the Global Healthcare group, based in New York. Previously, John was a Managing Director within the Global Healthcare group at Wells Fargo. Before joining Wells Fargo, John spent over three years in Biotech Equity Research at J.P. Morgan and William Blair where he covered large and mid-cap biotech companies. Prior to coming to Wall Street, he served four years at the Food and Drug Administration focused on drug approvals and safety surveillance. John holds a Ph.D. in physiology from Johns Hopkins University, a M.B.A. from the University of Virginia, and a B.A. from the University of California, Los Angeles.

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Robb Richards has over 20 years of experience in oncology, first with a private practice in Southern New Jersey and more recently the University of Pennsylvania Health System. He has served in different roles throughout his healthcare career: IT Manager for the Center for Cancer and Hematologic Disease in Cherry Hill, Division Chief Operating Officer of Regional Cancer Care Associates (RCCA) in Cherry Hill, New Jersey, and RCCA corporate VP and Chief Information Officer. He unofficially joined Penn's Cell Therapy and Transplant program (CTT) in 2016 and was the lead in overseeing operationalizing/implementation of CAR T-cell therapy for commercial use. He is currently the Corporate Director of The Center for Cell Therapy and Transplant program at Penn Medicine, with oversight of both commercial and research work and its expansion into community hospitals within the Penn system. Robb received his BS in Information Technology from Drexel University and both his MS in Informatics and MBA from St Joseph's University.

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Mark's research focuses on the economics of biomedical innovation, especially precision financing for patient access, precision medicine, adaptive pathways, platform trials, biosimilars, and digital health advances. Mark has served as a Special Government Employee for the FDA's Office of the Commissioner. Mark is also President of Co-Bio Consulting, LLC. His career has spanned policy as the President of the Massachusetts Biotechnology Council, diagnostics as founder of Cantata Labs, genomics as President of Cereon Genomics, eHealth as Vice President of Monsanto Health Solutions, managed care marketing at Searle Pharmaceuticals, and big data at Kenan Systems. He holds degrees in Chemistry from Stanford University and Management from MIT.

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Dr. Gary L. Simmons is an oncologist at Virginia Oncology Associates in Norfolk. Dr. Simmons received his medical degree from the University of New England College of Osteopathic Medicine in Biddeford, ME and completed his residency in Internal Medicine and subspecialty training in Hematology/Oncology at Virginia Commonwealth University in Richmond, VA. He is board-certified in internal medicine, hematology, and oncology. Dr. Simmons is recognized for his leadership in the development of several CAR T-cell programs in Virginia. In addition to performing numerous CAR T-cell procedures, he is also active in cell therapy research and frequently publishes papers, and gives local, regional, and national talks. He co-authored the BMTinfonet CAR T-cell handbook for patients in 2022. He has an extensive publication record of abstracts and papers and has presented his work at the American Society of Transplant Cellular Therapy and the American Society of Hematology. He currently serves as the Medical Director of McKesson Cell and Gene Therapy, as well as on the board of FACT consulting.

Rocky Billups
Vice President of Operations, HCA Healthcare/Sarah Cannon Cancer Network

Rocky Billups has been with HCA Healthcare for 28 years and joined HCA Healthcare/Sarah Cannon Cancer Network (SCCN) in 2011. He has more than 30 years of experience in hospital operations, clinical practice and developing oncology programs. Rocky is responsible for the development and implementation of SCCN's strategy for the delivery of a comprehensive and integrated blood cancer service line in the United States. He was instrumental in the development of the Sarah Cannon Transplant and Cellular Therapy Network in 2011 and facilitated the opening of Sarah Cannon Research Institute – UK, the first phase I oncology research facility in the private sector and accredited by the Care Quality Commission. Rocky received his diploma in nursing from St. Mary's School of Nursing in Huntington, WA and his Bachelor's and Master's degrees in Health Services Administration from Regis University in Denver, CO. He is a member of the American Society of Clinical Oncology, the American Society of Hematology, the American Society of Blood and Marrow Transplantation, and the Association of Community Cancer Centers.

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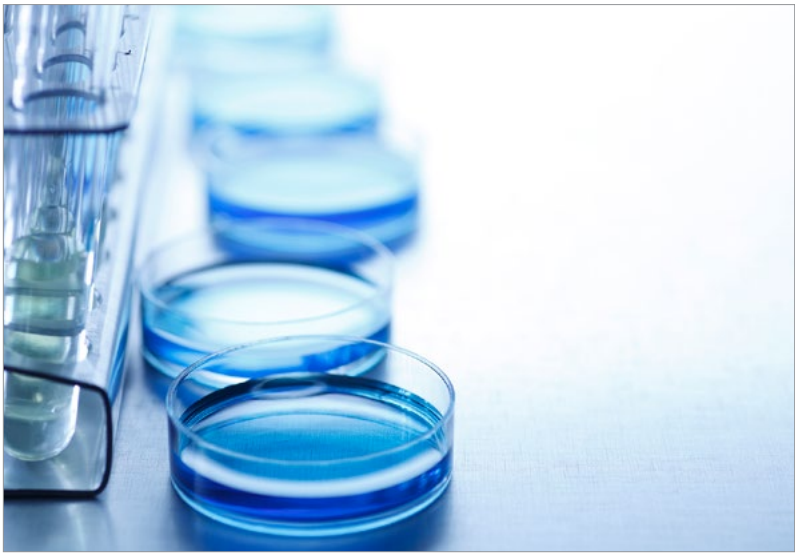

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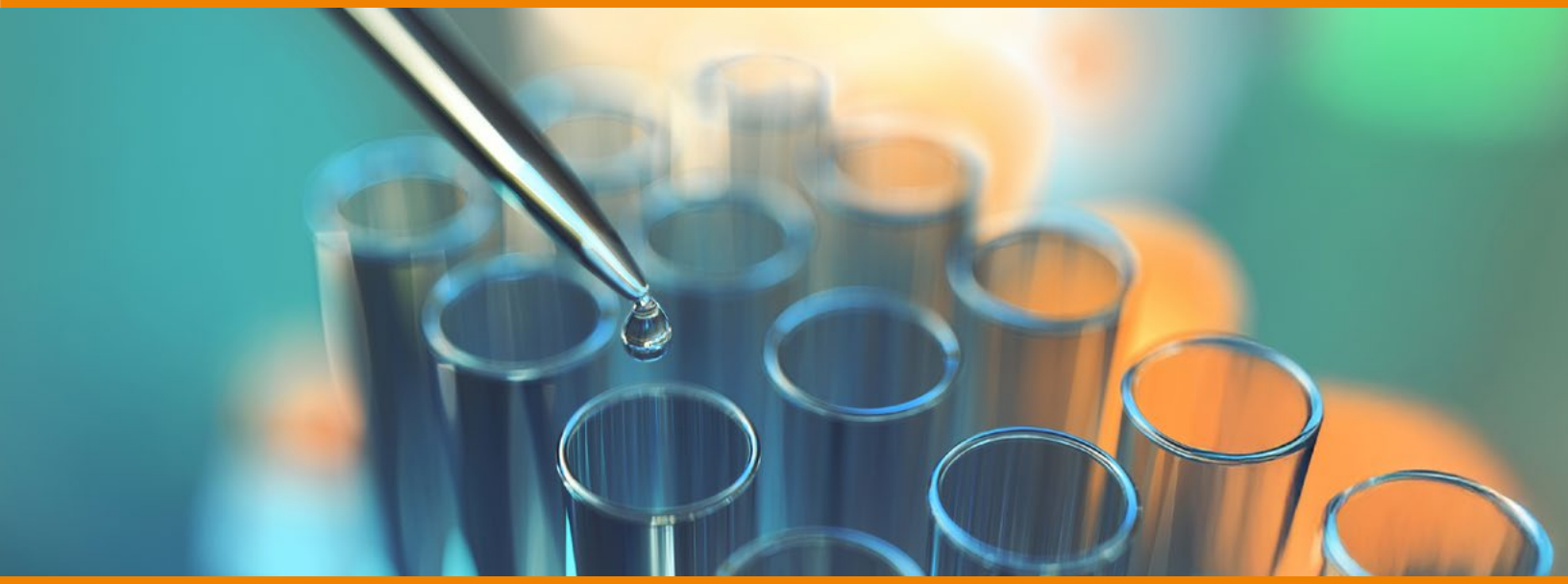

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InspiroGene by McKesson ("InspiroGene") is a dedicated business unit focused solely on supporting the commercialization of cell and gene therapies (CGTs). At InspiroGene, we turn CGT innovation into a reality. We offer flexible, sustainable solutions to help you navigate CGT commercialization to achieve long-term success. As your ally, we're dedicated to transforming patient care and driving better health outcomes. Learn more about the InspiroGene advantage at **InspiroGene.com**.

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