cell & gene

Guest Column | October 2, 2023

Part 1: Mobilizing Community Hospitals To Improve Patient Access To Cell And Gene Therapies

By Dr. Sanjay Srivastava, Managing Director, Cell & Gene therapy lead; Rahul Mirchandani, Senior Analyst, Life Sciences at Accenture Life Sciences, and Joe De Pinto, Head of Cell, Gene & Advanced Therapies at McKesson Pharmaceutical Solutions and Services

First in a two-part series

Case for Change

Today we're seeing several successes in cell and gene therapy (CGT) clinical trials. There are 32 CGT products commercially available in the United States, contributing to measurable progress in the fight against cancer and other diseases, with stem-cell research leading these efforts[1]. By some estimates, there were more than 1,000 CGT clinical trials globally across a range of modalities, targeting a variety of diseases across therapeutic areas. These potentially curative therapies are causing significant ripple effects up and down the social, clinical, and economic spectrum. We are seeing vast improvements in disease prevention, diagnosis, and management, with the promise to improve patient outcomes and provide significant long-term societal benefits.

The market remains in the early phases of commercialization with many products progressing quickly through clinical development and towards global approval. From a research and development perspective, the promise of cell and gene therapy has been primarily focused on hematologic malignancies and rare diseases to date. Research continues in these areas as well as tackling therapeutic areas with larger patient populations such as Lupus, Sickle Cell Anemia, and solid tumors. A recent review of Biopharma pipelines shows significant pre-launch activity is underway with 15-20 approvals anticipated a year from now until 2025, as reported by the US FDA[2]. Cell and gene therapies currently make up approximately 20 % of biopharma pipelines with a trend of increased investment. (McKesson analysis).

However, the rise of success and projected growth in the research arena brings many challenges. Chief among them: A constrained and ill-equipped healthcare delivery system that was not designed to handle complex therapeutics. The patient, product, and value chains for CGTs are far different than traditional healthcare delivery and requires new and specialized sets of capabilities at hospitals. Consequently, commercially available CGTs are primarily administered at limited number of tier 1 FACT[1]-accredited transplant centers and Academic Medical Centers (AMCs) in the US, as well as similarly equipped centers globally.

Figure 1. Major accredited treatment center coverage for a typical CAR-T therapy in US – the Midwest and Central regions are a patient access desert, and centers in the Northeast are unable to serve enough patients.

Availability of CGTs to these select treatment centers limits access to these therapies, especially for patients who don't live within the vicinity of an accredited center (**Figure 1**). Both patient and payors incur economic and social burden to provide access to remote patients, as some patients require travel support to reach the closest center, and patients on Medicare/Medicaid have even fewer options to cross state lines.

The growing pipeline of commercially approved therapies, in addition to earlier line approvals of existing therapies, is straining the current CGT delivery model. Qualified treatment centers are beginning to face capacity issues in administering cell therapies, and operational overhead may require them to limit the number of therapies they can offer as well as clinical trials they can collaborate in (**Figure 2**). Recent analysis by Accenture found that HCPs recommend expanding the HCP network to include community hospitals. The assumption is that it will not only improve patient access but could also provide commercially viable options for treatment centers. The

excess patient load can be transferred to community hospitals and, as the safety profiles of CGTs continue to improve, physicians can administer therapies in an outpatient setting. Outpatient treatment alleviates capacity constraints and provides academic institutions to leverage Medicare/Medicaid 340B programs and stretch its resources.

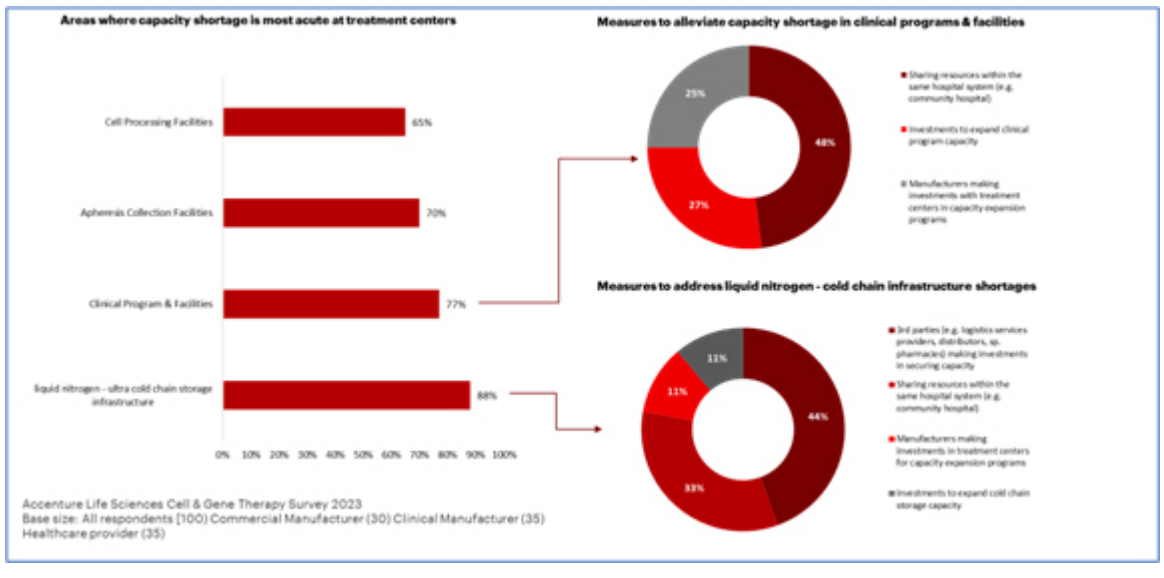

Figure 2. Treatment centers are encountering capacity shortages; There is a need to deploy robust measures to overcome the issues.

A Dose of Reality

Community hospitals (e.g. non-federal acute care) provide a viable option as an extension of AMCs to alleviate qualified treatment center capacity and patient access challenges. This shift could catalyze cross-industry investment and robust participation, paving the way for a future where community hospitals play a pivotal role.

While this is a compelling vision, several conditions must be met before community hospitals can be considered to alleviate capacity bottlenecks. CGT developers must address the supply challenges and community hospitals must develop a deep understanding of how to safely administer treatments to patients. Administration of CGTs is complex; successful delivery of CGTs requires therapy-specific operational capabilities, staff training and education, and compliance with regulatory requirements. CGT developers have typically launched therapies by converting their clinical trials sites at AMCs and extended the HCP network with similarly capable medical centers. When designing the HCP network, industry has taken a conservative approach and limited the number of centers where CGTs can be administered. It takes enormous effort to onboard and qualify a treatment center and maintaining a vast network can be cumbersome too. Regardless of designing a broad HCP network or a narrow network, CGT developers have avoided including community hospitals due to several considerations listed below.

1. **Patient Access and Identification:** Multi-specialty committee is often required to identify the right patient and determine patient eligibility. The committee needs to have experience to make treatment decisions based on therapy dependent biomarkers.
2. **Infrastructure:** On-site pharmacy / cell therapy unit at community hospitals were not designed to be equipped for handling, storing, and maintaining products that require products with ultra-cold (e.g., -150-degree freezer, cold chain), thawing, and final prep before infusion. The centers need key staff trained and responsible for coordinating transportation of time and temperature sensitive drugs with manufacturers and ecosystem partners.
3. **Capabilities:** Patient care and toxicity management capabilities with trained physicians and staff. Ideally, need an integrated EMR to support smooth outpatient and inpatient communication.
4. **Capacity:** Capacities to conduct long-term patient follow-up for up to 15 years, while maintaining regulatory compliance.
5. **Finances:** Ability to handle cash flow pressure due to the high upfront payment associated with high price tag. The current price range of commercially available CGT products is approximately $350,000 to $3,200,000 WAC per patient. This does not take into consideration any preconditioning or supportive care agents that may be needed alongside of these therapeutic interventions. Sites of care have done total cost of care analysis taking into consideration direct and indirect costs to deliver these innovative therapies.

Looking to the Future

As manufacturers prepare for product launch, they will need to consider ways to successfully operationalize and scale current cell therapy treatment models beyond—and within—the accredited transplant centers. While transplant centers will continue to be a priority, as we look to expand the reach of CGT treatments into community hospitals, it is important to address the above listed barriers and enable administration of these therapies at community hospitals. **The expansion of CGT into community hospitals depends on addressing several key issues. A clear understanding of these questions and steps that can be taken to solve them is critical to the effective and continued growth of the space.**

Key Questions

- *Which community hospitals that are not close to AMCs will reduce travel distance for the most patients?*
- *How can we ensure access across socioeconomic and cultural backgrounds and continue to prioritize diversity to ensure the greatest possible reach?*
- *What must have infrastructure needs to be clinically available to address the needs of the treating HCP and the patients? (e.g., Cryostorage, cell lab equipment, etc.)*
- *What capabilities, SOPs, and capacity is needed to satisfy the patient demand as it continues to grow?*

- *Is the community hospital equipped to address the clinical delivery of the product both inpatient and outpatient?*
- *What staff training and audit processes will be part of the site certification to ensure accurate and safe administration of these therapies?*
- *What investments are needed to equip community hospital to offer CGTs?*
- *Are community hospitals equipped to address the serious adverse events associated with some CGTs like CRS and ICANs? Or are they located within the vicinity of a center that provides such services?*
- *Can the site appropriately negotiate appropriate payment rates and assure reimbursement for these products?*
- *A clear understanding of the health provider economics is important as these products are often given 1 time at a high dollar expense causing a cost density issue. What are the net cost recovery models for both inpatient and outpatient use?*

The next piece in this series will address some ways we can overcome the challenges and answer key questions outlined here to make a lasting, positive impact and improve patient access to CGTs.

References:

1. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products
2. https://www.evernorth.com/articles/the-history-and-future-of-gene-therapy

[1]Foundation for the Accreditation of Cellular Therapy, a non-profit organization that inspects, accredits, and promotes cell and gene therapies globally.