BARRIERS TO CLINICAL TRIAL IMPLEMENTATION AND COMMERCIAL SITE READINESS FOR CELL AND GENE THERAPIES IN RARE DISEASES

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BACKGROUND

- Cell and gene therapies (CGTs), offer transformative potential for patients with cancer and rare diseases
- Unlike conventional therapies, CGTs require specialized infrastructure and operational capabilities, including product tracking, handling and storage, and intensive post-treatment monitoring
- When investigational therapies obtain regulatory approval, authorized treatment centers encounter significant challenges in transitioning from research-focused clinical trial processes to commercial care delivery
- Dedicated research coordinators, grant-funded trial support, and investigational drug services are often withdrawn, leaving clinical care teams to absorb significant administrative and logistical burdens
- Commercial delivery introduces substantial financial risk, including multi-million-dollar exposure, complex prior authorization workflows, coverage delays, and prolonged reimbursement delays
- As these therapies move from investigational to commercial availability, understanding the factors that influence site readiness is imperative to mitigate delays in patient access to these life-saving treatments

PURPOSE

- To identify key operational, financial, and logistical barriers that delay treatment center readiness for CGT clinical trials and subsequent commercial delivery in rare diseases
- To evaluate whether clinical trial participation influences site readiness and accelerates the transition to commercial availability

METHODS

- In February 2026, a web-based survey was fielded to multidisciplinary CGT provider experts and clinical leaders
- Survey topics included determinants of trial participation, barriers to implementation, and challenges encountered during transition to commercial delivery
- Results are summarized using descriptive statistics

RESULTS

DEMOGRAPHICS

A total of 16 CGT experts participated in this study. Collectively, this cohort has a mean 15.4 years of clinical experience, spend 72% of work time on CGT activities, and have experience in various advanced therapy modalities, Table 1.

Table 1. Participant Demographics and Characteristics

Primary professional role	
Physician (hematology or medical oncology)	31.3%
Pharmacy leadership	25.0%
Research – clinical trials	12.5%
Operations or administration leadership	31.3%
Advanced therapy modality experience	
Autologous cell therapy (e.g., CAR T-cell)	93.8%
Allogeneic cell therapy	81.3%
Ex vivo gene therapy (e.g., LVV, gene-modified cell therapy)	62.5%
In vivo gene therapy (AAV, LNP delivery)	43.8%
Other advanced modalities	31.3%
CGT care experience, mean (range)	15.4 (2-30) years
Work time dedicated to CGT activities, mean (range)	72.0% (10%-100%)

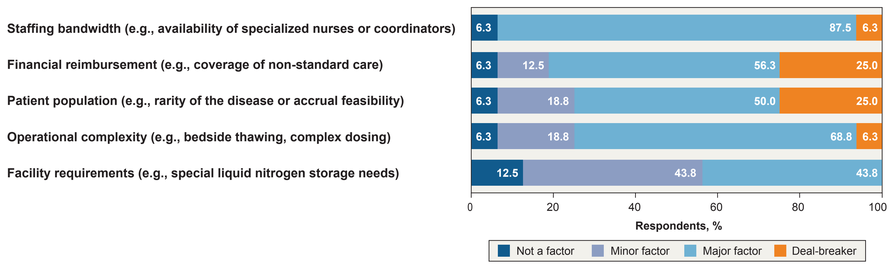
N=16

AAV, adeno-associated viral; CAR, chimeric antigen receptor; LNP, lipid nanoparticle; LVV, lentiviral vector.

KEY FINDINGS

Staffing bandwidth was the most important determinant of clinical trial participation with 87.5% of respondents identifying it as a major factor. Financial reimbursement and patient population size were also frequently cited as important considerations.

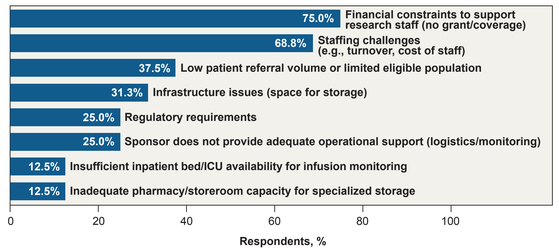
Figure 1. Determinants of Clinical Trial Participation



Survey question: When evaluating a new clinical trial, how much might the following factors influence a treatment site's decision to participate?

Figure 2. Major Barriers to Clinical Trial Implementation

Financial and staffing constraints emerged as the leading barriers to trial implementation.

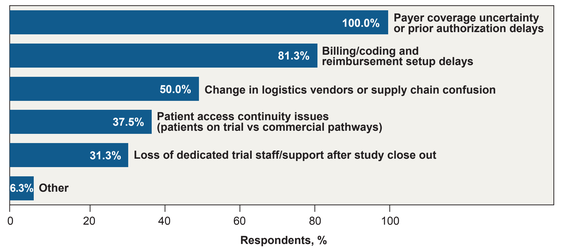


Survey question: Which of these staffing or operational barriers most often create significant challenges for trial implementation and participation at treatment centers? *Please select up to 3.*

ICU, intensive care unit.

Figure 3. Clinical Trial to Commercial Availability Transition Barriers

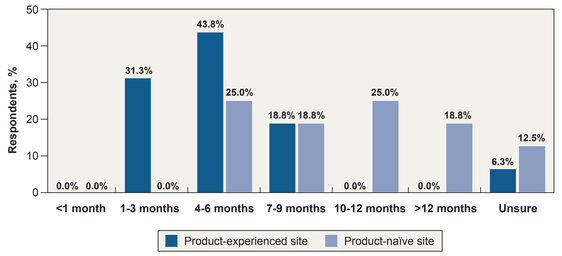
The transition to commercial delivery was most often hindered by payer coverage, prior authorization, and reimbursement-related barriers.



Survey question: Which of the following transition issues have you observed when a therapy moves from clinical trial to commercial availability? *Please select all that apply.*

Figure 4. Site Readiness Lead Time: Product-experienced vs Product-naïve Sites

Clinical trial participation may accelerate commercial readiness, while product-naive sites more often require >10 months to achieve readiness.



Survey question: What is a typical lead time for a site to be operationally ready to deliver a newly-approved product commercially? Product-experienced is defined as the site participated in the product's clinical trial and product-naïve is defined as the site did not participate in the product's clinical trial.

CONCLUSIONS

- Our data show that many barriers, including staffing capacity, financial sustainability, and payer processes, continue to delay timely patient access to CGTs
- Clinical trial participation may accelerate commercial readiness by providing sites an opportunity to develop the infrastructure, care pathways, and institutional expertise needed to support future CGT delivery
- However, even experienced trial sites often encounter reimbursement, payer, and operational challenges when transitioning therapies from clinical development to commercial availability
- Sites without prior trial experience may face additional barriers, as they must establish these capabilities while simultaneously preparing for commercial implementation
- Proactive commercialization planning during the trial phase and earlier engagement of key stakeholders are critical to reducing time to treatment and ensuring access to CGTs for patients with rare diseases

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