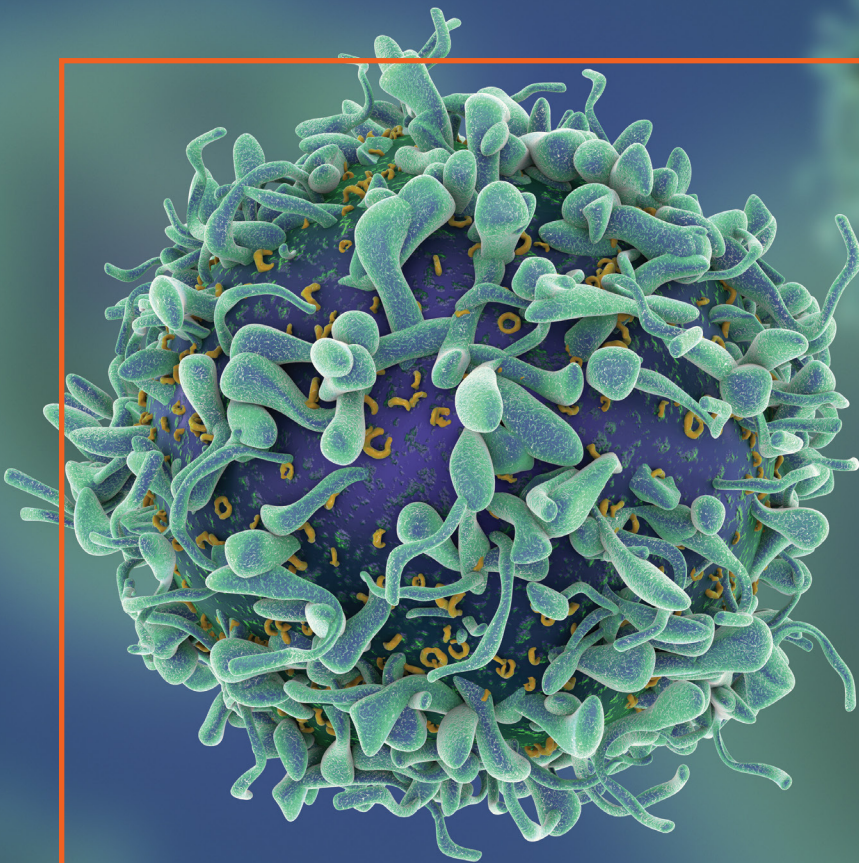


# Elevating Cell & Gene Therapy Clinical Trials: Strategic Alliances Between Biopharma and CROs

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## Pioneering Advances in Cell & Gene Therapies

The journey into the realm of cell & gene therapies marks an unprecedented era in medical innovation, promising transformative breakthroughs in addressing complex diseases. The sector is projected to hit around USD 82.24 billion by 2032, growing at a CAGR of 18.3% from 2023 to 2032,<sup>1</sup> with novel therapies seeking to remedy previously unmet needs of patients, particularly in oncology and rare diseases. The sector is now reaching a greater level of maturity as cell and gene therapies begin to become more commonplace in the pharma landscape, with five new therapies receiving FDA approval in 2023 and over 1,800 clinical trials underway across the globe. According to ARM (Alliance for Regenerative Medicine), orphan gene therapies are 3.5 times more likely to be approved once entering Phase I trials than average drugs included in BIO's Global Trends in R&D 2023 Report.

Close collaboration between industry and regulators such as the FDA is helping to tackle some of the issues and challenges currently associated with developing cell & gene therapies — reducing clinical development timelines, harmonizing global regulatory requirements and addressing safety concerns. Work is also underway to develop new payment models, helping to make these innovative new therapies more affordable for healthcare providers, and for some rare disorders, more cost effective than the current standards of care.

In this paper, we share insights into the necessary collaboration and partnership between biopharma entities and contract research organizations (CROs), highlighting their pivotal role in propelling these revolutionary therapies from inception to clinical implementation.

## Sponsors' Perspectives: Navigating the Complexities of Therapy Development

Biotechnology and pharmaceutical sponsors navigate a multifaceted landscape filled with formidable challenges that span financial limitations, the dynamic interplay of adaptive strategies amid regulatory flux and the ceaseless competition prevailing within the therapeutic domain. This intricate milieu demands a convergence of astute foresight, unwavering resilience and an agile approach to align the trajectory of therapeutic innovation with the evolving demands of the industry.

**Financial Constraints and Strategic Navigation:** The path toward therapeutic breakthroughs can potentially span decades and is often impeded by financial constraints. These constraints pose formidable barriers, impacting research and development budgets, and often necessitating prudent allocation of resources. Amidst this, sponsors are compelled to curate astute strategies that balance fiscal prudence with innovation, seeking optimal avenues to leverage available funds for maximal impact.

**Regulatory Flexibility and Adaptable Strategies:** Evolving regulatory landscapes constitute an ever-shifting terrain, demanding not just compliance but the ability to fluidly

adapt to emerging guidelines and standards, and sponsors face the arduous challenge of staying abreast of regulatory shifts. Employing flexible and adaptive strategies can aid in aligning and maintaining compliance, continuously recalibrating approaches to match evolving frameworks without compromising the pace and integrity of therapy development. Prioritizing safety and continuous patient monitoring remains paramount, particularly given the mandated collection of approximately 15 years of post-trial data, a unique aspect for these relatively new cell & gene therapies compared to more traditional standards of care. Sponsors must consider how this requirement for extended data collection may impact patients and devise strategies to alleviate any associated burdens while maintaining data quality and regulatory compliance.

**Establishing successful partnerships with CROs is not merely a choice but an indispensable strategy.**

**Competitive Landscape and Strategic Alliances:** The relentless competitive ecosystem within the pharmaceutical industry necessitates a perpetual quest for differentiation. Standing out amidst a sea of competitors mandates innovative approaches and the establishment of strategic alliances to fortify positions in the market. Collaboration with CROs emerges as a highly advantageous approach, offering a strategic edge through productive partnerships, leveraging specialized expertise and combining resources for accelerated therapy development.

## Strategic Partnerships With CROs: Optimal Approach To Drive Success

Establishing successful partnerships with CROs is not merely a choice but an indispensable strategy. These alliances transcend transactional collaborations, embodying a shared vision and a collective commitment toward therapeutic triumph and patient welfare. The cultivation of such symbiotic relationships necessitates a combination of specialized expertise, adept navigation through regulatory intricacies and the creation of transparent communication channels that serve as the cornerstone of seamless collaboration.

> **Cultivating Specialized Expertise:** Biotechnology and pharmaceutical sponsors recognize the inherent value of leveraging the specialized prowess inherent in experienced and credible CROs; the combination of CRO expertise, spanning therapeutic domains and intricate trial methodologies, proves instrumental in navigating the complexities of therapy development, expediting timelines and mitigating unforeseen hurdles that could impede progress.





> **Navigating Evolving Regulatory Landscapes:**

Regulatory compliance stands as a significant variable, marked by continual evolution and paradigm shifts. The symbiotic alliance between sponsors and CROs necessitates a cohesive strategy, ensuring the alignment of trial protocols and methodologies with the ever-evolving regulatory benchmarks. The adept navigation of regulatory landscapes demands a collaborative ethos, where CROs play an integral role in interpreting guidelines and adapting trial methodologies in real time — for example, ensuring patient identification and enrollment appropriately reflects the diversity of the patient population, or producing compliant and quality protocols or other documentation that adheres to regulatory guidelines.

- > **Transparent Communication Channels:** Transparency forms the foundation of successful collaborations between sponsors and CROs. Establishing clear and open lines of communication fosters an environment conducive to synergy, enabling seamless exchange of insights, addressing challenges proactively and aligning strategic trajectories toward mutual objectives. Transparent communication channels serve as catalysts for innovation, enabling dynamic course corrections and strategic pivots when warranted. Good communication is also paramount to helping ensure smooth and effective project management, mitigating potential delays and maintaining positive relations with regulatory bodies to help address any concerns during the submission and approvals process.

## Multi-Stakeholder Perspectives on Cell & Gene Therapy

- > **The Patient Experience: Balancing Promises and Realities:** For patients, novel therapies can often epitomize a beacon of hope, offering potential curative avenues, amplified survival rates and tailored treatments. Yet, the realization of these promises contends with logistical complexities inherent in therapy administration, treatment costs limiting access and the restricted availability of specialized centers alongside establishing trust between patients and trial investigators. Harmonizing trial designs with patient-centric needs and amplifying access pathways is paramount for fostering healthcare delivery, ensuring that the transformative potential of these therapies reaches those in need.
- > **Sites and Investigators: Bolstering the Frontlines of Therapeutic Innovation:** Clinical sites serve as the frontline of therapeutic innovation but grapple with mounting trial burdens, logistical intricacies and acute staffing shortages. Cultivating a proficient and well-trained workforce emerges as an indispensable necessity for the efficacious administration of these groundbreaking therapies. Strengthening streamlined processes, instituting robust training protocols and reinforcing infrastructure stand pivotal to managing the escalating demands intrinsic to these specialized trials.

> **Medical Monitoring: Fortifying Patient Well-Being Beyond Treatment:**

Post-treatment patient vigilance emerges as an indomitable necessity, mandating meticulous and sustained monitoring over extended periods. Rigorous postmarketing surveillance, coupled with unwavering adherence to stringent regulatory benchmarks, serve as the bedrock for continual assessment and enhancement in therapy administration. This relentless vigilance aims to minimize adverse events and maximize therapeutic efficacy, thereby ensuring optimal patient well-being throughout their treatment journey.

> **Manufacturing Dynamics: Innovations Shaping Therapy Delivery:**

Quality, efficacy and titer are vital considerations in conjunction with the technology platform when determining how to efficiently scale-up production and manufacturing to best serve the target patient population. Technology platforms such as mRNA mean personalized medicine could soon become more familiar and routine, offering potential hope for vaccines against certain cancers and rare diseases. Whether small or large scale manufacturing, an array of logistical hurdles and supply chain complexities must be overcome. These difficulties are particularly pronounced in the case of autologous cell therapy products and ex vivo therapies involving the extraction, manipulation or modification of cells outside the patient's body before being reintroduced into the patient. The operational intricacies involved in managing and tracing individual patient or donor samples from the clinic to the manufacturing site and back present an unparalleled set of logistical challenges. Ensuring each product is meticulously tracked to its respective patient is essential for maintaining a seamless chain of custody and traceability throughout the entire manufacturing process. The tightly scheduled manufacturing processes in CGT add additional strain to the supply chain, intensifying the pressure. Any delays in delivering the therapy can directly impact the health outcomes of the patient. Close collaboration and supply chain management between the site, CDMO and CRO is necessary to help alleviate vulnerabilities, assuring the secure and timely delivery of therapeutic agents into the clinical realm. There are both open and closed production systems.

The figure below illustrates the CAR-T therapy process, starting with the patient donating cells at the hospital in an open production system, a procedure known as leukapheresis, which must be repeated if not done correctly. The cells are then taken to the engineering facility where they must be kept in a cold chain of custody during transport, which may take several weeks. During this period, to ensure that their blood cells do not attack the infused cells, the patient undergoes chemotherapy, leaving them vulnerable to infection, requiring a sterile environment. If supply chain issues occur, this can reduce the viability of the altered cells that return from the processing plant, meaning the entire process has to be repeated once the patient has recovered and regenerated enough white cells.



## CART - JOURNEY OF THE CELLS - COLLECTION TO INFUSION

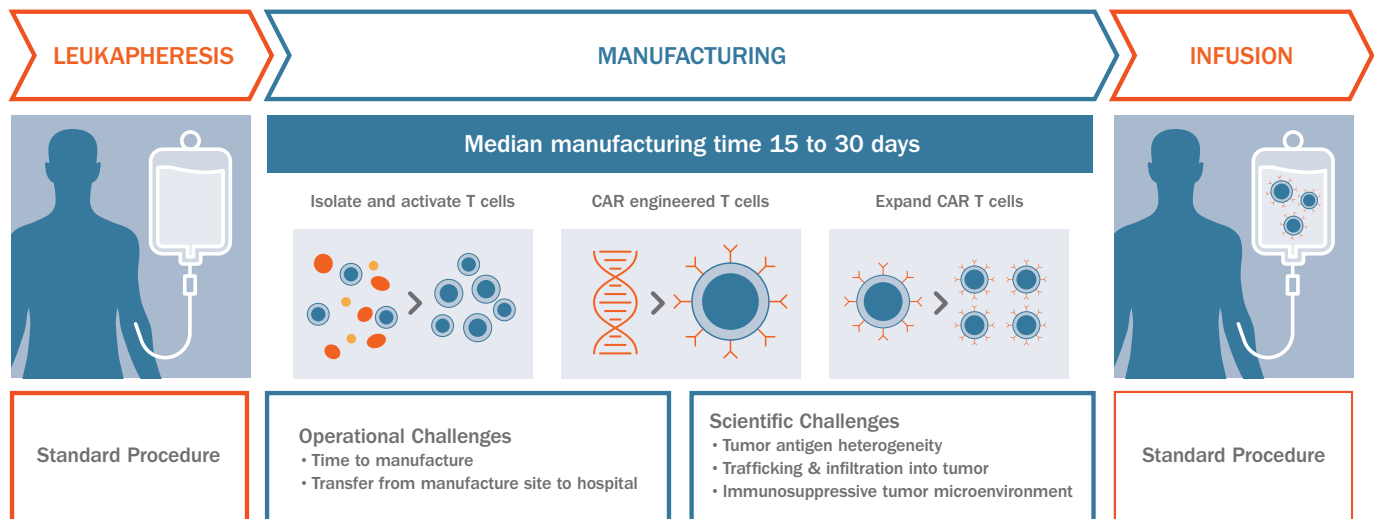


Figure 1: The journey of the patients' cells: From collection to infusion

### Regulatory Navigation in Cell & Gene Therapy: Charting a Path to Approval

Navigating the intricate regulatory landscapes combined with cell & gene therapies necessitates collaboration to establish harmonized frameworks for streamlined approvals and global accessibility. Addressing quality assessment intricacies, navigating the nuances of manufacturing regulations and accommodating global variations in approval processes emerge as pivotal requisites for the seamless transition from product development to commercialization. Establishing robust communication channels and cultivating relationships with regulatory bodies is imperative. This strategic approach is vital for articulating trial results effectively, highlighting the benefits and value to patients and expediting regulatory approval whenever feasible.

### Paving Future Avenues Through Better Biopharma-CRO Collaborations

The evolution of cell & gene therapies toward diverse therapeutic domains underscores the imperative of addressing multifaceted challenges encompassing clinical intricacies, regulatory nuances, funding gaps, manufacturing complexities and barriers to accessibility. The symbiotic partnership between biopharma companies and CROs is pivotal in unleashing the transformative capabilities of cell & gene therapies. Collaborative efforts between biopharma entities and experienced CROs play a crucial role in addressing challenges, creating innovative solutions and sparking a revolution in healthcare delivery, catalyzing therapy development and expanding patient access. This paves a path toward a future where personalized and effective therapies seamlessly reach the hands of patients worldwide, reshaping modern medicine.

**CONNECT WITH OUR EXPERTS TO LEARN MORE ABOUT HOW WE CAN COLLABORATE WITH YOU ON YOUR NEXT CELL & GENE THERAPY TRIAL:**

**Contact Us**

### Reference

<sup>1</sup> [www.precedenceresearch.com](https://www.precedenceresearch.com). (n.d.). Cell and Gene Therapy Market Size To Hit USD 93.78 Bn By 2030. [online] Available at: <https://www.precedenceresearch.com/cell-and-gene-therapy-market>.

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