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Article 1:

Commercializing your first cell or gene therapy (CGT): Five best practices for planning

Cell and gene therapies (CGTs) are transforming the treatment of serious diseases, offering unprecedented potential to improve outcomes, address health issues with a single dose, and even deliver cures for diseases once thought untreatable. However, the path from scientific breakthrough to successful commercialization is fraught with challenges, even for CGTs backed by the most compelling science and clinical results.

Unlike traditional therapies that follow standardized commercialization processes, there is no set roadmap for the commercialization of CGTs. Their complex, patient-specific nature presents biopharma companies with equally complicated commercialization challenges related to everything from patient identification and logistics to market access and reimbursement and beyond. A unique patient or product journey is created with the development of every dose of a CGT, which means that every commercialization plan must also be customized.

If you're developing your first CGT product, here are five key commercialization practices to keep top-of-mind.

1. Start commercialization planning early.

The complexity of CGT commercialization can cause slower-than-expected adoption, particularly when the planning process begins too late in the development cycle. For example, identifying qualified patients for CGTs can require extended time because patients must undergo specialized screening processes for genetic biomarkers. Commercialization planning must also

account for complex manufacturing and value chain processes, which sometimes can require months between the initiation of the prescription and the administration of the therapy. Most notably, the high cost density presented by CGTs creates new reimbursement challenges that require considerable, advance collaboration with payers and other stakeholders. Ideally, developers should recruit and fill the right roles, like head of commercialization, early in the development process, to ensure these foundational steps to commercialization are in place during Phase I/II studies, or well before your therapy's pivotal trial.

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- Unlike most biologics, the commercialization process for CGTs isn't just "rinse and repeat." There is no one-size-fits-all template due to the personalized nature of each therapy. The patient, product and reimbursement journeys for every CGT are unique and complex, and factors such as cell vs. gene, autologous vs. allogeneic, in-vivo vs. ex-

vivo, and liquid vs. solid tumor will lead to different market access needs. It's essential to understand the intricacies, complexities and interconnectedness of these journeys, so you can implement commercialization strategies that are tailored to your therapy and patient population, in a way that supports optimal access.

- 3. Become an expert on your therapy's site-of-care dynamics.** Will patients receive your CGT therapy on an inpatient or outpatient basis? Will you launch with only a few sites of care, or will you need your therapy to be broadly accessible at many sites? Will it be administered in large, academic hospitals, or at community hospitals or clinics, or both? What are the benefits and drawbacks of each model? Today, the vast majority of CGT treatment is delivered at large, academic medical centers in major metropolitan areas, but there is a growing movement to expand access into community settings to reach broader patient populations. Intimately understanding these site-of-care dynamics and where your therapy needs to be delivered to reach your patient population is key to accelerating adoption of your product.
- 4. Think broadly about your competitive landscape.** Contrary to popular assumption, having strong clinical data showing efficacy and safety doesn't always mean a therapy will be commercially successful. Less expensive, more easily accessible therapies may still pose a competitive threat, even if they are less effective. That's why your commercialization plan must take into consideration how to differentiate your product not only from

direct competitors with similar mechanisms of action, but also indirect competitors like antibody-drug conjugates, bispecifics and RNA-derived advanced therapies.

- 5. Don't underestimate the challenge of reimbursement: It's currently one of the most significant obstacles to expanding CGT access.** In the world of CGTs, where a single dose can replace a lifetime of medical treatments, the most essential question remains: how do we most appropriately pay for these medicines? Our healthcare reimbursement system is primarily designed to accommodate pay-as-you-go therapies for chronic conditions, which are reimbursed in much smaller individual sums over the course of years and even decades. That's why, even for CGTs that are curative, managing high, one-time costs remains the most significant challenge to commercial success. Understanding (and helping develop) innovative payment models, like value-based contracts and performance-based annuities, is a critical step to helping the healthcare system digest the high, front-loaded costs of CGTs.

CGTs represent a new era in healthcare. The successful commercialization of these innovative treatments will require developers to think, plan, collaborate, innovate and troubleshoot in new ways. It will also require developers to start early, and reach out to experienced partners, like InspiroGene, who can help bridge the gap between innovation and reality. In the next installments in this series, I'll dive deeper into how each of these best practices can set your therapy up for commercialization success.

Author's note: Joe DePinto is Head of Cell, Gene and Advanced Therapy at McKesson, where he leads InspiroGene, the company's dedicated business focused solely on supporting the commercialization of cell and gene therapies (CGTs). At InspiroGene, we turn offer flexible, sustainable solutions to help manufacturers, payers, and providers navigate the complex CGT commercialization landscape. As an enduring ally, we're dedicated to transforming patient care and driving better health outcomes. Learn more about the InspiroGene advantage at [InspiroGene.com](https://www.inspirogene.com).