# CELL AND GENE THERAPIES

## THE CATALYST FOR NOVEL COMMERCIAL STRATEGIES

By Maya Khurana and Alfred Chon

Cell and gene therapies (CGT) are disrupting the traditional pharmaceutical business model because they diverge from traditional revenue streams that rely on recurrent dosing, requiring a larger upfront sum for a one-time treatment. These innovative therapies are altering care pathways, and healthcare commercial models are not equipped to capture the potential value of CGT.

To address this, pharmaceutical companies should rethink their business models and develop appropriate capabilities to support the implementation of system solutions that encourage innovation and improve patient access. At a recent Huron roundtable and a series of follow-up conversations, participants (including treatment developers, healthcare providers, payors and policymakers) noted that, regardless of how companies adapt their approaches to CGT, they must partner differently with payors by evolving across four dimensions: value, culture, pricing and relationships.

## Unique Challenges for CGT Developers

Not all developers have the necessary organizational strengths and capabilities to take on the challenge of reengineering their business models to enable commercial success, and the two developer archetypes (pure and mixed players) each have their own unique challenges.

- **Pure play: Technology focused strictly on CGT with singular or multiple therapeutic areas of focus**

  They have entrepreneurial spirits, greater appetites for risk share, and lean structures that enable agility, but they lack the scale and resources necessary to develop commercialization capabilities, enter therapeutic areas with strong incumbents, and continue investing in their research and development (R&D) pipelines. To make real headway with CGT and scale up their commercial strategies, pure players often need incumbents to provide the resources to help them develop their assets.

- **Mixed play: Multiple technology focus (monoclonal antibodies, small molecules, etc.) along with multiple therapeutic areas of focus**

  They have the resources and scale to build the systems and solutions needed to encourage CGT adoption, but they often face challenges of integration with legacy portfolios and outmoded organizational mindsets, especially because their CGT portfolios are usually built via licensing and acquisitions. Even with the right strategy, mixed players can struggle to execute effectively if they are unable to foster a culture of innovation that mimics those of their pure play peers.

# 4 Dimensions of CGT Commercial Model Evolution

The following recommendations are geared toward mixed players that have the resources and scale to make a significant impact in CGT development. These four dimensions, if given appropriate focus, can help these companies to differentiate themselves in this market.

## CULTURE

**Challenge:** Functional silos, misaligned incentives and compliance concerns are major barriers to implementing any creative solutions for CGT adoption. That is why fostering a culture of innovation and collaboration is a significant challenge for larger mixed players that are often bound by the weight of legacy portfolios and traditional mindsets.

**Solution:** In order to rise above these hurdles, mixed players must adopt a startup mindset that enables innovation and strategic risk-taking (often inherent in pure play companies). At the same time, leaders must also unequivocally endorse this change and incentivize corresponding behaviors. This, combined with their scale and resources, will position these companies to achieve long-term success with CGT adoption.

## RELATIONSHIPS

**Challenge:** Pharmaceutical developers need to change the way they come to the negotiation table by engaging in good faith to repair the eroded trust with payors and increase their comfort with novel CGT treatments.

**Solution:** Sustainable adoption will require pharmaceutical companies and payors to be flexible, transparent and collaborative to find compromises that are win-wins for all involved.

## VALUE

**Challenge:** Adjacent to pricing and contracting considerations, the ability to clearly articulate the value proposition of CGT is of paramount importance to success for pharmaceutical developers. The value criteria for CGT amounts to a 180-degree shift from a focus on volume to an emphasis on outcomes and total cost of care.

**Solution:** The pharmaceutical industry can help payors to develop frameworks that quantify indirect outcomes that go beyond traditional economic valuation guidelines to highlight the societal effects and long-term cost savings these treatments can deliver (workforce considerations, societal effects, etc.)

PRICING

**Challenge:** With very few CGT products on the market to date, a fundamental shift is required in terms of how payors manage one-time treatments. Cell and gene patients may require additional interventions to truly maximize the clinical outcomes of a particular therapy, which requires moving beyond asset-level contracting and toward more outcomes-based models. For more on possible contracting options, see the Novel CGT Contracting Options chart. The administrative resources needed to monitor CGT patients and track outcomes are often cited as barriers to pricing innovations for these unique therapies. At the same time, "best price" reporting requirements can hinder developers' willingness to innovate their pricing strategies (e.g., pay-over-time or outcomes-based methods), particularly in the U.S.

**Solution:** To encourage adoption, pharmaceutical developers must engage differently with payors to create unique contracting options (see Novel CGT Contracting Options chart) to suit population- and portfolio-level deals. Pharmaceutical developers can build the foundations of risk share with payors by adding transparency into the process, articulating their value and acting as a credible partner.

## Novel CGT Contracting Options

| | | |
|---|---|---|
| **Portfolio Level** | **PORTFOLIO OUTCOME GUARANTEE**<br>Payment made if patient meets desired health outcomes using any combination of products offered in a portfolio<br><br>**SUBSCRIPTION MODEL**<br>Fixed payment made for each patient to access the portfolio; no health outcomes guaranteed | **TOTAL CARVE-OUT**<br>Vertical integration with care delivery; total costs of the entire patient population taken out of the health system; pharmaceutical developers provide products and infrastructure for care delivery |
| **Product Level** | **WARRANTY MODEL**<br>Payment made if patient meets desired health outcomes using a particular product | **POPULATION WARRANTY MODEL**<br>Payment made if the eligible pool of patients meets a desired health outcome, calculated by taking an average of health outcomes across the population<br><br>**LEASE MODEL**<br>Fixed payments made by the payor each month for all patients to access the therapy |
| | **Patient Level** | **Population Level** |

= contracting options for health system management

# The Path Forward

CGT has the potential to upend traditional healthcare delivery. But even the most effective novel treatments are only as valuable as they are accessible. For most patients, that means payor coverage.

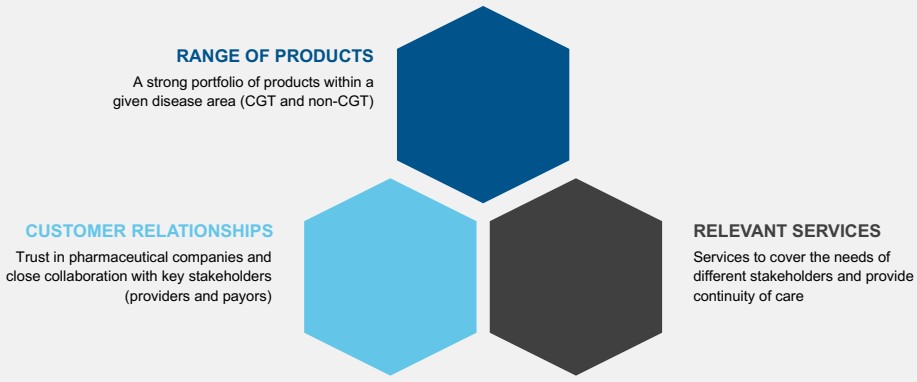
To continue to move the needle on CGT adoption, pharmaceutical developers must evolve their approaches to pricing and market access by repairing their relationships with payors and collaborating to ensure the value of their products is captured. That means rethinking the pharmaceutical industry's long-standing — and in the case of CGT, archaic — approaches to market access as well as payors' conventional understanding of treatment valuation.

## An Innovative Business Model Alternative: The Health Management System

One of the more innovative alternative business models some pharmaceutical companies are considering is the health management system. A health management play requires an appropriate range of products and services as well as strong relationships with a variety of stakeholders. Companies looking to build this model will also need supportive capabilities and partnerships (i.e., driving patient identification and eligibility or investing in digital platforms to collect real-world evidence).

Many of the components of medtech business models mirror those of the health management services model. Medical device companies (e.g., Fresenius Medical Care, Medtronic) use wraparound services as a key differentiator to build customer loyalty and ensure positive outcomes. Likewise, a health management model will rely heavily on patient follow-up and outcomes collection.

This approach may not work for all disease areas or all companies, but certain disease areas, such as hemophilia, could be good candidates to pilot this approach if they have reasonable patient numbers and require a mixture of different treatment models and services for effective patient management.




Andrew Hobbs, managing partner at Axian Consulting Ltd., helped to moderate the roundtable on which this piece is based.

HURON

huronconsultinggroup.com



20-1811