Preparing Healthcare Systems for Broader CGT Access – Developing a Mutual Framework to Improve Outcomes

Nathanael Green, YanWen Chong, Liam Maddison & Stefan Focsa

Eradigm Consulting

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INTRODUCTION

Research aim: To explore the concerted motions that pharma and healthcare systems can action to unify and co-create win-win situations, opening cell & gene therapy access to more patients globally.

Background: Cell & gene therapies (CGTs) have heralded a new era of medicine, promising potentially curative treatments for patients with acute, chronic, and often fatal diseases. However, discrepancies between the high prices set by pharma and healthcare systems' willingness-to-pay has restricted access and somewhat dampened the broadening potential of CGTs. In the current healthcare environment, the majority of emerging CGTs will struggle to establish a foothold, as it is simply not designed to accommodate this step-change in innovation.

METHODOLOGY

- **Literature review** using key search terms to source articles evaluating the key drivers behind positive and negative CGT access decisions, from global healthcare/regulatory and pharmaceutical company perspectives.
- **Search terms** included ("CAR T" OR "CAR-T" OR "CGT" OR "cell and gene therapies" OR "gene therapies" OR "gene therapy") AND ("HTA" OR "access" OR "pricing" OR "evidence“ OR “reimbursement”).
- A search was also conducted on **documents** from various **HTA bodies in the EU4 + UK, Nordics, and the US (ICER).**
- **Articles detailing challenges and/or barriers relating to CGT access or pricing and reimbursement were analyzed**, where key themes were extracted and collated into a framework that can be utilized by both healthcare systems, regulatory agencies, and pharma companies.

RESULTS

We developed a framework with which pharma and healthcare systems can find mutual ground to meet their respective objectives and streamline processes

1) **Robust aligned evidence:** As CGTs demand strong evidence throughout their lifecycle to prove value to patients and payers, pharma companies can develop their evidence base by openly sharing clinical trial designs and future evidence generation plans from early stages.

2) **Unified value recognition:** Pharma could determine value based on the available evidence at launch with additional outcome-based contracts capturing incremental value, in addition to healthcare systems being more open in their value calculations.

3) **Tailored pricing models:** Innovation in pricing models requires flexibility from both pharma and payers, where a tailored pricing strategy would enable equitable access to novel therapies, ultimately benefitting the patient.

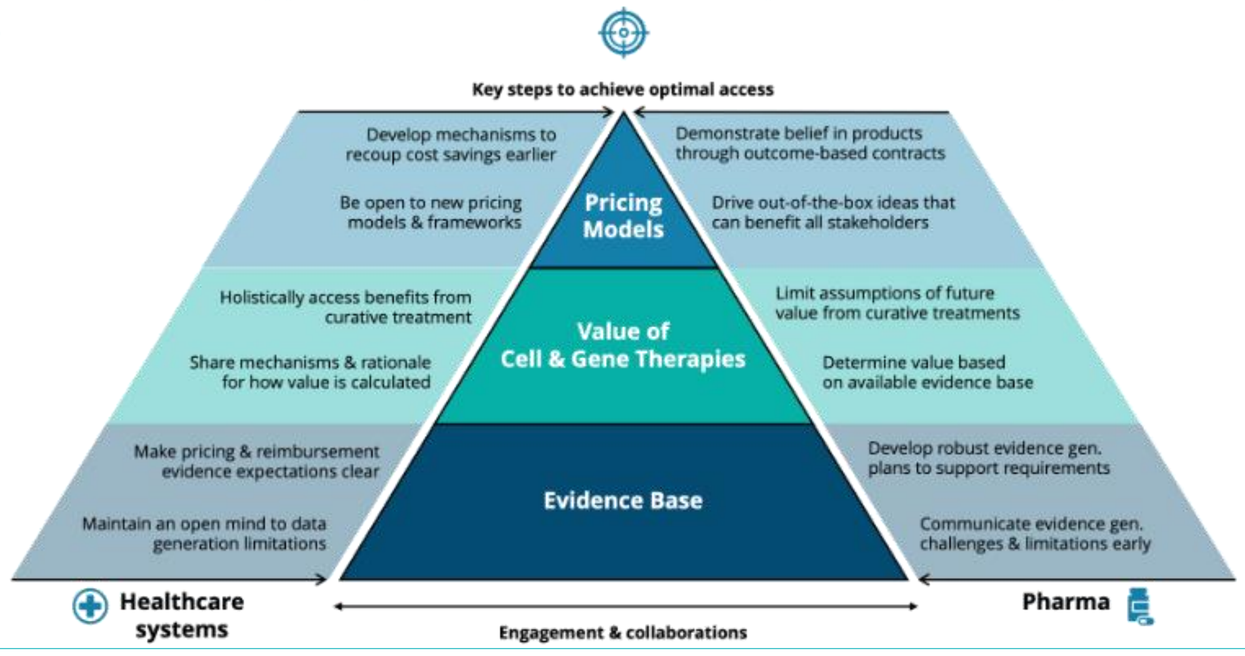
Figure 1: Examples of innovative pricing strategies used to facilitate access to CGTs

Asset	Pricing Model	Strengths	Weaknesses
Tisagenlecleucel	Deferred outcomes-based payments	Mitigates initial budget impact	Requires case-by-case reimbursement administration
Betibeglogene autotemcel	Outcomes-based annualized installments	Enables fair adjustment of payments by addressing data uncertainties	Requires commitment to payer future budget
Voretigene neparvovec	Third-party financing and contract administration	Supports short-term affordability at the hospital level	Hesitancy to use an additional intermediary who is also looking to monetize
Sofosbuvir / Velpatasvir; Glecaprevir / Pibrentasvir	Subscription model	Supports front-loading treatment and eradicating illness in a short time while maintaining a stable budget by spreading the cost over several years	Marketed as a "subscription" but structurally resembles a simple budget cap, does not incorporate any outcomes provisions

DISCUSSION

- Before these three key themes can streamline future CGT access, the current short-term price-focused mindset must be reformed, or it will continue to act as a barrier to innovation. Removing this roadblock will take time, but concerted efforts by pharma can catalyze this.
- Evolving evidence expectations, increasing collaboration between key stakeholders, and flexibility in adopting new pricing frameworks are vital steps to achieving access and unlocking the maximal value of CGTs.
- As CGTs broaden their applicability into more prevalent diseases, such as solid tumors and Alzheimer's disease, the time is now to find solutions to access barriers that exist today for CGTs.

Figure 2: Framework to support pharma and healthcare systems achieve improved access for new CGTs



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