PHP249: Are traditional managed entry agreements suitable for potentially, curative gene/cell therapies?

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Introduction

Potentially curative gene/cell therapies face clinical uncertainties about long-term safety and duration of effect. This is compounded by the financial risks in the asymmetry between significant upfront costs and promised life-long benefits. While current tools, such as managed entry agreements (MEAs), may be suitable to address one uncertainty at a time, tackling clinical and financial uncertainties together will show the limitations of existing contracts and models.

With an MEA, manufacturers face the binary choice of either being denied access or having to reduce their initial target price. This price reduction often occurs through confidential means to address the financial uncertainty in terms of cost effectiveness and/or budget impact that payers deem unacceptable. These financial MEA contracts have various modalities on several levels: unit price, patient eligibility or population cap. Clinical performance-based agreements are linked to a clinical measurement that in turn triggers payment, additional discounts or no payment. Response, survival, prevention or other parameters are potentially used, but data collection and interpretation create another loop of complexity. Innovation is paramount for MEAs to work in the context of gene and cell therapies.

Methodology

We polled 60 conference participants using an instant electronic voting system at BioEurope, 2018 in Amsterdam. The participants self-selected to attend and comprised a mix of industry executives, patient association representatives, R&D researchers, strategy consultants and financial investors.

Discussion and conclusions

Using traditional MEAs to address both the clinical and financial uncertainties associated with potentially curative therapies offered by gene/cell therapies could lead to unprecedented price level reduction to address concerns about cost effectiveness, affordability and budget impact in the context of significant long-term clinical uncertainties. While possible in the short-term, there is a tipping point when price will not be sufficiently attractive to sustain investment in new gene/cell therapies (Fig 1) as debated in poster #PMU75.

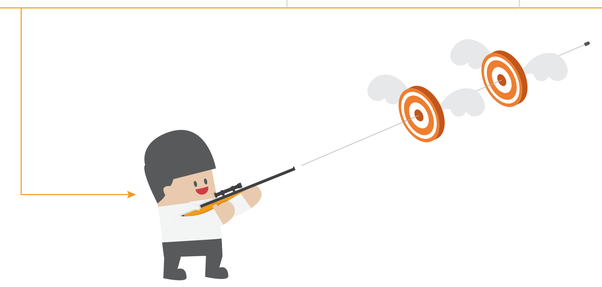
While innovation in science and medicine is providing new options to treat diseases and patients, innovation around funding and paying for these new interventions is not necessarily moving with the times.

Many participants noted (Fig 2) the limitations of the traditional bargaining discussion between manufacturers and payers that occurs at launch. New players in the financial and/or the actuarial industry could be considered, especially in Europe (Fig 3) to find a satisfying long-term solution, addressing not only access, but also the sustainability of the research ecosystem, small biotech, larger pharma and public payers. A new comprehensive approach, combining pricing strategy with policy initiatives, is needed upfront to ensure future pricing negotiations take place in an environment with sufficient funds available for advanced medicines access to patients.

One solution could be access to funding from third parties such as insurers, charities or financial institutions for securitised loans. New conceptual models are burgeoning and may reach the marketplace in the near future to ease the pressure on industry and payers and provide more sustainability.

Figure 1: MEA – Hitting two birds with one stone?

	MEA to address clinical risks	MEA to address financial risks
Drug with limited outcomes evidence at launch	✓	
Drug with "high price" at launch		✓
Potentially curative gene/cell therapy	✓	✓

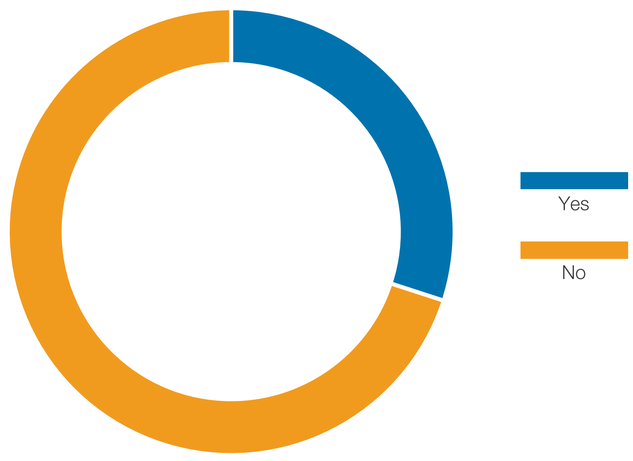


Over the last two decades increased adoption of generics, and more recently biosimilars, plus the efforts by health technology assessment (HTA) bodies to improve "value for money", have had an impact on the drug price index. As reported by the French CEPS, the cumulative drop in 16 years (2000–2016) has been 33%. Over the same period, price has been used as a key lever to manage market entry of new drugs.

R&D budgets have increased over the same period. R&D has adapted to pricing pressures by refocusing on more specialised, less prevalent conditions, and less chronic treatments, however these treatments are more costly to develop, produce and market.

Understanding when price may become too low to become attractive for R&D investment is critical to engage in areas such as gene and therapy discovery, development and commercialisation.

Figure 2: Can we still do the same tomorrow as yesterday?

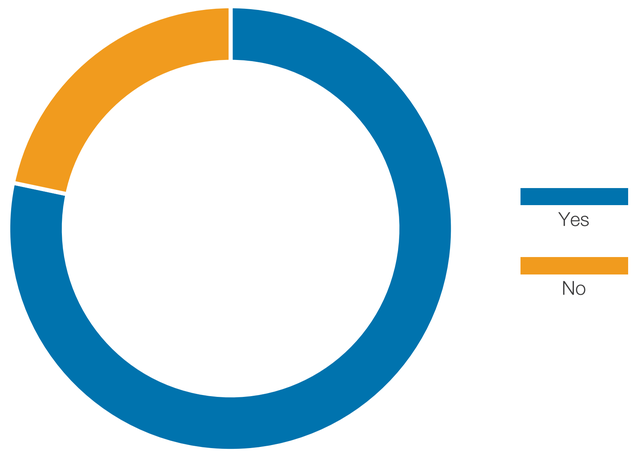


Can EU health systems cope with gene/cell therapy pricing and is it just a question of reassigning funds and negotiating contracts?

A majority of participants (70%) believe that obtaining access to gene/cell therapies using the same approach as in the past will not work. This is not simply a case of re-assigning funds or negotiating contracts because health systems cannot currently cope with this level of pricing.

From an industry perspective, multi-functional internal stakeholders need to consider product launch activities as well as policy activities to ensure the long-term sustainability of gene/cell therapy development and funding for future pipeline products.

Figure 3: The need to involve new stakeholders



Will engaging third parties (insurers, charities and financial institutions) solve the funding/affordability issues hampering access today?

Participants strongly believe (83%) third parties could help solve funding and affordability issues that currently hamper access to what are labelled as expensive medicines. To improve negotiation outcomes for high-cost therapies, all stakeholders must acknowledge that access to medicine (in general) and advanced medicine (in particular) is a problem multiple stakeholders can solve via innovative and mutually beneficial solutions, which otherwise could not be implemented with the traditional players who typically sit at the negotiating table.

References
Poster #PCP13 (Joe Nevins, Walter Colasante, Luka Jelcic; CRA); Poster #PMU75 (Walter Colasante, Bhavesh Patel; CRA)