PMU75: Is adopting a low price strategy to gain market access compatible with a viable gene/cell therapy business?

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Introduction

Will 2018 become known as the year when new cell and gene therapies began taking hold in the market? While their life-saving potential is clear in many instances (based on clinical evidence available at launch), there are many medical and technological uncertainties that long-term data and further research likely will address. The more fundamental question of who pays for therapies at a price that is financially attractive to industry and financially affordable for the buyer is harder to answer. How can the market set a fair price for drugs that addresses the sustainability of the research industry and the needs of healthcare systems and patients? Too high a price means healthcare systems and patients are unable to afford a drug, thereby denying patients access to life-saving medicines. At too low a price, the research industry, as we know it, could flounder. In this study, we assess the perceived impact of price on the sustainability and long-term viability of the industry behind the emerging innovative and potentially curative interventions along with associated negotiated rebates.

Methodology

Using an instant electronic voting system at World Orphan Drug Conference Washington, DC (US) in 2018, we polled 43 conference participants that self-selected to attend the market access session on gene/cell therapy. The group comprised industry executives, patient association representatives, R&D researchers, strategy consultants and financial investors.

Discussion and conclusions

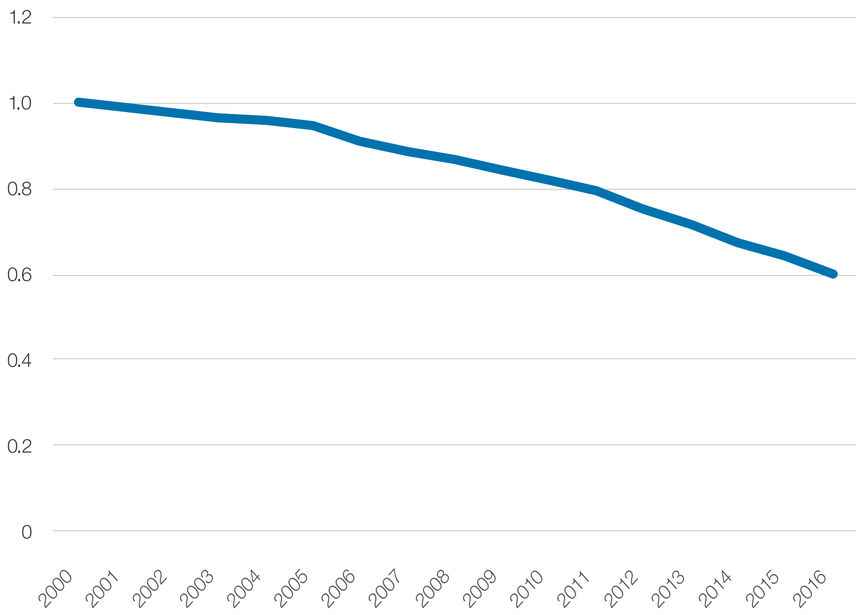
Accepting a low price with the goal to gain market access may be the simplest solution to ensure patient and health system access to an innovative treatment (Fig 1). Low price could, in theory, avoid the restrictions and/or delays that a higher price may otherwise trigger. Opinion is not unanimous: 77% of those polled perceive a potential long-term viability risk for gene/cell therapies (Fig 2) if negotiators simply bargain access at the expense of price.

Low epidemiology and incidence for diseases, potentially cured by gene/cell therapies, need to be aligned to the “right” price to be affordable and support sustainable investment in research and development. Stakeholders with influence on pricing and access decisions should refrain from using price as a bargaining tool, and seek new, likely complex and mutually beneficial, long-term solutions.

If HTA bodies conclude that prices are unjustified, there may be restrictions to usage, access delays, slow uptake, and consequently a failure by manufacturers to meet commercial goals. Changes to the P&R system are likely necessary where exceptions are made, or greater flexibility is allowed, for diseases with substantial medical need.

A third party, in addition to the manufacturer and payer, is seen as a possible solution for 52% of the audience in the US (Fig 3). In Europe, the constraints of the system and the nature of public health systems push a third party option even higher up the agenda (83%). Financial and risk management instruments could become part of the solution in some markets under certain circumstances to ensure clinical innovation and financial sustainability for all stakeholders.

Figure 1: Price of drugs indexed to year 2000

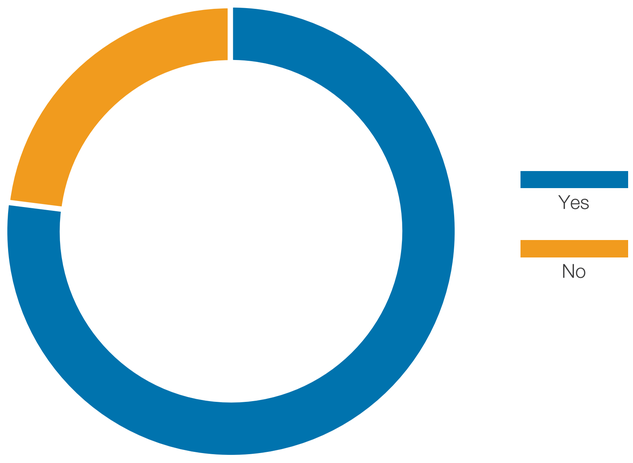


Over the last two decades, increased adoption of generics and more recently biosimilars plus efforts by HTA bodies to improve “value for money” have affected the drug price index (French CEPS[1]). The cumulative drop in 16 years is 33%. During this same period, price has been used as a key lever to manage market entry of new drugs.

R&D budgets have increased over the same time frame. R&D has adapted by developing for more specialised, less prevalent conditions, and less chronic treatments, which cost more to develop, produce and market.

Understanding when price may be too low to attract R&D investment is critical to engage in gene and therapy discovery, development and commercialisation.

Figure 2: Low prices vs. long-term viability



Will selling at a low price to manage market access, put the long-term viability of gene therapy at risk?

The majority of our audience (77%) see potential long-term risk for gene/cell therapy if originators prioritise access at the expense of price.

A minority (23%) of the audience do not see low prices as harming the long-term viability of cell and gene therapies, and may not be accepting the reality of financial risk in this space. Two months before the WOD conference, we asked a similar question at BioEurope, Amsterdam (PHP249) with 57% of those polled perceiving that viability would be at risk.

Figure 3: Can we still do the same tomorrow as yesterday?

Should a third party be involved in ensuring patient access for expensive products?

Opinion is divided. A small majority (52%) believes that the usual bargaining game between pharma and payers is not sustainable. There were 48% who still believe that there is no need to expand the debate to new stakeholders.

However, 83% percent of the audience two months prior to this congress (at BioEurope, Amsterdam, PHP249) thought a third party should be involved to reframe the issue and provide new solutions beyond the usual bargaining around price and access.

References

[1] Rapport Annuel 2016 Comite economque des produits de sante, https://solidarites-sante.gouv.fr/IMG/pdf/rapport_annuel_2016_medicaments_2.pdf, page 11; accessed October 2018.