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). 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