Determinants of the Optimal Route to Reimbursement for Orphan Medicinal Products (OMPs) in England

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Introduction

Orphan and ultra-orphan diseases are defined in the EU as having a prevalence of ≤5 in 10,000 and ≤1 in 50,000 patients respectively. As such, rare diseases pose significant additional challenges for stakeholders than more common diseases. The rarity of orphan diseases has meant that many biotech and pharmaceutical companies have been reluctant to develop orphan medicinal products (OMPs) due to economic reasons under normal marketing conditions.

Additionally, despite the recent increased focus to improve patient access to treatments for rare diseases, there is no route to reimbursement in England which caters particularly for OMPs. We have identified the different routes to market that OMPs have taken, and conducted a critical appraisal to assess which pathway is best suited to evaluate OMPs.

Methods

The routes to market for OMPs launched between January 2012 and June 2017 were evaluated via online searches on the NICE and NHSE websites, and analysis of responses to parliamentary questions. The assessments were reviewed and their outcomes, along with key elements ascertained, were utilised to help identify successful strategies.

Results

Analysis of the parliamentary questions showed that 28/60 OMPs submitted between 2013 and March 2017 were routinely commissioned. 17 OMPs were routed through a NICE health technology appraisal (HTA), 8 OMPs underwent a single technology appraisal (STA), 6 through the cancer drugs fund (CDF), and 3 through the highly specialised technologies (HST) programme. The remaining 11 OMPs underwent an NHSE commissioning policy (CP) (Figure 1).

Of the remaining 34 OMPs, 15 are currently under NICE review, 4 OMPs are on the NHSE programme, 4 OMPs are not routinely commissioned and 3 OMPs were not launched in the UK. Information on the remaining 8 OMPs was not available.

NICE has begun to consider more flexibility with companies such as accepting greater levels of uncertainty for OMPs. However, there has been little leeway in the £20,000 to £30,000 (or £50,000 for end-of-life treatments) incremental cost-effectiveness ratio (ICER) threshold.

An increasing trend seen in submissions is the inclusion of patient access schemes (PAS) and managed entry agreements (MEAs). Both schemes allow manufacturers to improve the cost-effectiveness of their product, with the added benefit that MEAs can more easily be confidential risk sharing schemes.

The majority of clinical policies will be considered in NHSE’s annual prioritisation round (Figure 2) which considers the incremental clinical benefit and cost of different developments and makes investment decisions based on the available resources. The high cost nature of orphan drugs means that OMPs will likely only ever be classified as priority level 3, 4 or 5.

Individual funding requests (IFRs) and clinically critically urgent (CCU) procedures remain extremely unpredictable and difficult options for patients to gain access to OMPs due to the strict criteria used and uncertainty around their future viability.

Conclusions

The OMPs coverage gap has been recognised for some time now yet there seems to be no orphan-specific route to reimbursement in England. There are a few routes to market that have been utilised by companies, however, there is no one specific route that stands out for OMPs. This situation is commonly referred to as the “doughnut hole”.

The ideal route to market would be through a NICE HST, an HTA process that takes into account wider considerations such as data limitations, complexity of the condition and service, patient numbers and limited opportunity to recoup investment.

NICE only conducts 3 HSTs a year. OMPs that have not been scoped into a NICE HST but are likely to meet NICE’s ICER threshold range, including the use of PAS/MEAs, could undergo an STA. The remaining OMPs, which are unlikely to meet NICE’s ICER thresholds, may be better suited to NHSE CPs (Figure 3).

References:
2. UK Parliamentary questions (PQ7797)
5. MAP BioPharma. Available at: https://www.mapbiopharma.com