A review and evaluation of successfully reimbursed orphan and non-orphan drugs in Ireland

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Background

In Ireland, health technology assessments (HTAs) are conducted by the National Centre for Pharmacoeconomics (NCPE)[1]. Since 2009, the NCPE has allowed recommendations to be made via Rapid Review (full HTA required or full HTA not required) in an effort to enable more appropriate resource prioritisation, although some manufacturers still gain reimbursement following price negotiations with Ireland's Health Service Executive (HSE). Previous reviews have studied number of drugs achieving NCPE reimbursement; however, the discrepancy between orphan and non-orphan European Medicines Agency (EMA)[2] drug status in terms of reimbursement success is an important consideration which has not yet been comprehensively studied.

Objectives

To analyse the number of drugs with non-orphan drug status successfully achieving NCPE reimbursement in 2022 compared to those with EMA orphan drug status, and to identify any impacts achieving orphan drug status might have on reimbursement success in Ireland.

Methods

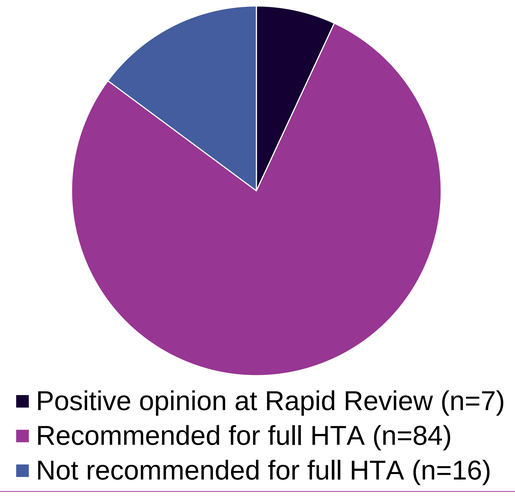
Rapid Review and full HTA outcomes were reviewed based on the decision date and whether full or partial reimbursement was achieved.[1] Results were tabulated and descriptive statistics were compiled. The list of authorised pharmaceuticals with orphan designations was obtained from the EMA website[2], while information regarding reimbursement status was retrieved from the NCPE website.

Results

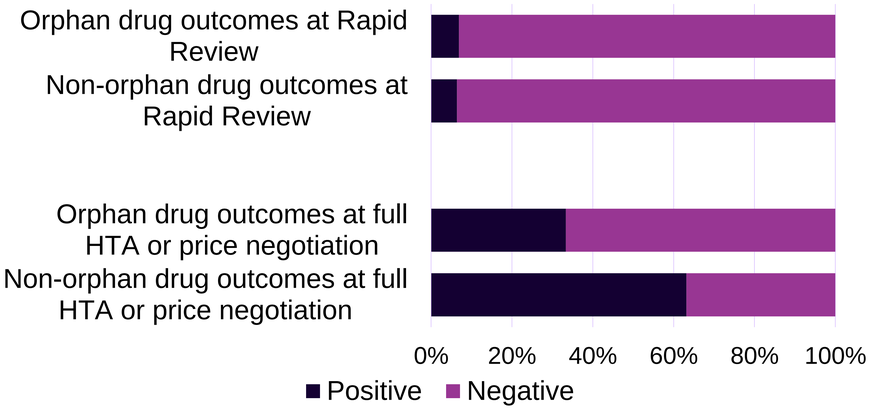
The NCPE received a total of 107 submissions (Rapid Review or full HTA) in 2022. 29 (27%) of these submissions were for EMA-designated orphan drugs. At Rapid Review, the initial stage of the NCPE assessment process, only 7 out of 107 submissions (7%) received a positive reimbursement recommendation, which was 7% (2/27) of orphan drugs and 6% (5/78) non-orphan drugs.

28 products which received a negative Rapid Review advanced to price negotiation or full HTA submission, and received a final decision. 15 products were approved at price negotiations or full HTA stage, with 33% (3/9) of submissions for Orphan drugs receiving a positive opinion, compared to 63% (12/19) of non-Orphan. 56 of the products that received a negative Rapid Review but were recommended for HTA are still working on their full submissions, while 16 were not recommended for Rapid Review.

Outcomes at Rapid Review (n=107)



Success rates of reimbursement for orphan versus non-orphan drugs at Rapid Review and full HTA



Conclusions

In Ireland in 2022, orphan drugs were approved for reimbursement at a lower rate than non-orphan drugs; the Rapid Review process had a negligible impact on whether an orphan drug was reimbursed. While there are several plausible explanations for this, it should be noted that the Irish reimbursement framework does not contain a process specifically designed to improve access to orphan drugs. As such, patients suffering from rare diseases in Ireland may experience setbacks as a consequence of the NCPE's 'one-size-fits-all' strategy.

Abbreviations: EMA, European Marketing Authorisation; HSE, Health Service Executive; HTA, Health Technology Assessment; NCPE, National Centre for Pharmacoeconomics.

References: 1. NCPE Website (www.ncpe.ie) 2. EMA Website (https://www.ema.europa.eu/en/human-regulatory/overview/orphan-designation-overview)

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