DOES ORPHAN DRUG STATUS CONFER ANY BENEFITS FOR PRODUCTS UNDERGOING HTA APPRAISAL BY NICE IN ENGLAND?

Legg K[1], Wilson C[1], Craddy P[2], Foxon G[1],
[1]Remap Consulting, Cheshire, United Kingdom, [2]Remap Consulting, Zug, Switzerland,

Introduction/objective

- Orphan drug status is granted by regulatory agencies for medicines proposing to target rare diseases with a high level of unmet need.
- At a regulatory level, incentives are offered, for example the European Medicines Agency (EMA) offers specialised scientific advice, fee reductions and ten years of market exclusivity.[1]
- Health Technology Assessment (HTA) agencies will often consider orphan status during their assessments.
- The purpose of this study is to compare the assessments of orphan and non-orphan drugs by the National Institute of Health and Care Excellence (NICE) since 2018 and determine if orphan status confers any benefit.
- This study looks at both single technology assessments (STAs) and Highly Specialised Technology (HST) evaluations. The HST pathway is an alternative assessment route for drugs very rare and serious diseases.
- Given the strict criteria for drugs to enter the HST pathway, all HST assessments concern drugs with orphan status, but there are also such drugs that are assessed via the standard STA route.

Methods

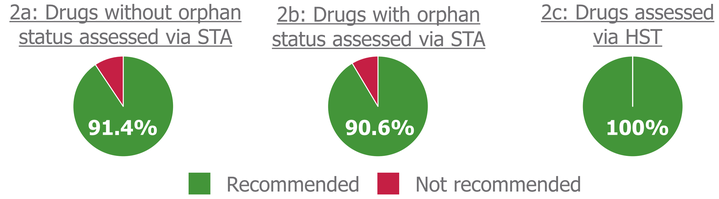
Results

- There were 163 STAs during the study period, of which 22% (N=35) had orphan drug status. Additionally there were 8 HSTs during the study period, 100% of which had orphan drug status.

Figure 1: STAs and HSTs Within the Study Period

- The success rate, defined as the proportion of drugs that were recommended by NICE (regardless of any restrictions), was determined.
- The overall STA success rate was 90.8%, with no significant difference between the non-orphan (Figure 2a) and orphan (Figure 2b) groups. In contrast, the HST success rate was 100% (Figure 2c).

Figure 2: Comparative success rates of drugs with and without orphan status in both STA and HST appraisals

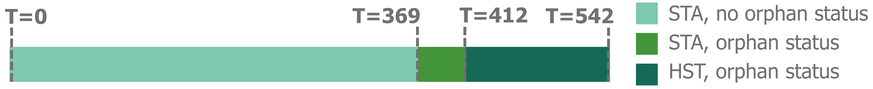


- However, whilst success rate did not differ between drugs with and without orphan status (at an STA level), the same cannot be said for use of financial agreements.
- Drugs with orphan status were more likely to require a financial agreement; either a patient access scheme, commercial access arrangement or managed access arrangement.
- 89% of STA drugs with orphan status, and 88% HSTs, required a financial agreement of some kind.
- Conversely, just 77% of drugs without orphan status required a financial agreement (Figure 3).

Figure 3: Proportion of financial agreements used in the positive assessment of drugs with and without orphan status, in STA and HST appraisals

- Furthermore, drugs with orphan status generally took ~10% longer to assess.
- The average assessment was 412 days for STAs drugs with orphan status and 542 days for HSTs, whilst drugs without orphan status took an average of just 369 days.

Figure 1: Appraisal length in days from final scope to final recommendation document



Discussion and conclusion

- Orphan drug status does not confer any benefit during a NICE assessment.
- In fact, whilst STAs of drugs with and without orphan status had a comparable success rate, those with orphan status were more likely to require a financial agreement, and took longer on average to be assessed. This could suggest the process is more complicated for drugs with orphan status.
- Other confounding factors such as smaller patient numbers leading to challenges in evidence generation, and higher price expectations, should also be considered. These can make it to harder to demonstrate cost effectiveness, which in turn could have complicated and stalled the appraisal process.
- The 100% success rate of HSTs is a clear exception to the lack of benefit conferred by orphan status, but with so few drugs meeting the strict criteria for the HST process, it is a minor one.

Abbreviations

*References: **1**. European Medicines Agency. Orphan Incentives. https://www.ema.europa.eu/en/human-regulatory/research-development/orphan-designation/orphan-incentives [Accessed 08/10/201].*