Trend analysis of technology appraisal decisions from NICE - what factors influence the likelihood of recommendations?


Objectives

The objective of the analysis was to compare trends of single technology appraisal (STA) versus multiple technology appraisal (MTA) recommendations for new technologies and indications conducted by NICE. We hypothesize that despite the methodological advantages of an MTA, due to the high cost and long duration of appraisals, NICE are increasingly favoring STAs over MTAs. Trend analysis was also conducted segregating products by European Union (EU) orphan designation status and by disease area to identify the specific challenges faced by companies planning an European launch of any new products or indications.

Methods

A validated, longitudinal MAP BioPharma database containing all technology appraisal guidance published by NICE since the formation of the organization was analyzed1,2. Analysis of products by disease area was conducted by classification into British National Formulary (BNF) categories. Reviews of technology appraisals, which overwrite earlier guidance, were not accounted for in the analysis.

Results

In 2000-2007, the ratio of guidance published for MTAs to STAs was 1.8:1 (68 versus 37). However, this ratio was reversed to 1:3.4 in 2008-2014, when guidance was published for 41 MTAs and 141 STAs (Figure 1). In 2009, 92% of STA guidance included positive or restricted recommendation (n=12), decreasing to a historical low of 54% in 2013 (n=28) and increasing to 86% in 2014 (n=21) (Figure 2).

NICE have evaluated a small number of orphans, mostly through STAs rather than MTAs (Figure 3). Excluding withdrawn appraisals, approximately 50% of recommendations for orphan products or indications were positive or restricted in 2011 and 2013 and 100% were positive or restricted in 2012 and 2014. Excluding withdrawn appraisals, the overall recommendation rate was significantly lower for orphan products than non-orphan products (55% versus 78%, p=0.0135).

Malignant disease and immunosuppression treatments were the most common STAs but had the second lowest recommendation rate (61%, n=75). All cardiovascular (n=20), endocrine (n=6), respiratory (n=3) and skin (n=4) products that have been evaluated through the NICE STA process have received a positive recommendation (Figure 4). Products most commonly withdrawn from the STA process were for obstetrics, gynecology and urinary-tract disorders (n=2), the endocrine system (n=3) and the central nervous system (n=4).

Conclusions

A decreasing proportion of appraisals include multiple technologies which is likely due to the complexity of scheduling and appraising multiple technologies at once. Recently NICE stated that all biosimilars will be appraised through MTAs so we expect in the future that the MTA process will be reserved primarily for biosimilars. There have been substantial variations in annual recommendations from STAs. This could be due to a genuine failure to demonstrate clinical or cost-effectiveness but may to some extent reflect the choice of appraisal committee and evidence review group involved. Initial findings from an additional analysis indicates there may be statistical differences in the recommendation rates between appraisal committees and evidence review groups.

A common misunderstanding of the UK market is that NICE is the key route to reimbursement. We have shown that NICE appraise a very small number of orphan products. Alternative routes to market are possible and should be considered, for example NHS England commissioning policies. Malignant disease and immunosuppression products continue to be the most common NICE appraisals and have a low success rate. However, many oncology products that are not recommended by NICE go on to be funded by the controversial Cancer Drugs Fund. This leads to questions regarding the validity of NICE assessment of oncology products and whether NICE resources are wasted when many treatments will be funded regardless of the outcome.

References:
2. NICE recommendations obtained from the NICE website: http://www.nice.org.uk/guidance/published?type=ta

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