HTA46: Key Issues in Health Economic Analysis in NICE Highly Specialised Technology Appraisals

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Background & objective

BACKGROUND

The NICE HST process continues to evolve. For those preparing submissions, it may be incredibly useful, even in the context of rare diseases or unique technology, to identify commonality amongst issues identified by Evidence Review Groups (ERGs) or NICE committees.

OBJECTIVE

To determine key themes and issues identified by Evidence Review Groups (ERG) and the NICE committee during the NICE Highly Specialised Technology (HST) Appraisal process. Also, to explore the relationship between different issues that limit the ability of the NICE committee to approve a product, as well as looking at ways that companies mitigate uncertainty in their appraisals.

Methods

All products that followed the NICE HST process (to December 2021) were identified and analysed.

In addition, an analysis of committee papers and subsequent publications was carried out, along with a targeted literature review of associated publications.

Figure 1 shows this research output.

Results

Of the 16 products that have followed the NICE HST process, the most common major criticism (87.5% of products) from the Evidence Review Group was that resource utilisation estimates were inaccurate, or that the methodology used was not sufficiently robust. Other common criticisms related to the utility modelling not being robust enough (68.75%), utility estimation by clinicians (56.25%), clinician estimates of efficacy (43.75%), model approaches not being sufficient for decision making (31.25%), and trial endpoint robustness (25%). This led to 93.75% of cases that resulted in a positive recommendation having managed access agreements and confidential discounts applied to them. Major criticisms of submissions tend to centre around the lack of a robust methodology for derivation of estimates (resource utilisation and utility values) from clinicians.

Conclusion

Products that qualify for a NICE HST process tend to be in a rare disease area, meaning there is typically a paucity of data. This usually leads to manufacturers turning to clinicians to seek estimates – it is crucial here to have a recognised, robust methodological process to elicit and validate estimates. Further review of NICE publications suggests that Modified Delphi and Vignette studies may be most appropriate if carried out in a robust and meaningful way; validation across multiple stakeholders can also add extra validity.

Discussion

The driver to resolving issues identified in the NICE HST process is almost always an improved commercial agreement (i.e., a price reduction). Therefore, this research offers an important insight into methods of bolstering a data package ahead of an HST submission.

Manufacturers entering into the NICE HST process should consider these findings and look at strategies to mitigate issues that may arise. NICE themselves have also highlighted mitigation strategies that could be considered acceptable in solving data gaps – this advice should not be ignored given that resolutions almost always come in the form of further price erosion.

Figure 1: Issues raised during the NICE HST Appraisal process (est = estimation)

HST code	Product	Issue 1	Issue 2	Issue 3	Issue 4	Resolution
1	Eculizumab	Model approach	Utility estimates	Clinician estimates		Unkown
2	Elosulfase alfa	Resource utilisation est	Utility estimates	Clinician estimates	Utility modelling	Managed access scheme
3	Ataluren	Resource utilisation est	Model approach	Utility estimates	Utility modelling	Managed access scheme
4	Migalustat	Resource utilisation est	Utility estimates	Clinician estimates		Patient access scheme
5	Eliglustat	Resource utilisation est	Utility estimates	Clinician estimates	Utility modelling	Patient access scheme
6	Asfotase alfa	Resource utilisation est	Utility estimates	Clinician estimates	Utility modelling	Managed access scheme
7	Strimvelis	Resource utilisation est	Utility modelling	Utility estimate		Commercial agreement
8	Burosumab	Resource utilisation est	Utility modellig	Clinician estimates		Commercial agreement
9	Inotersen	Resource utilisation est	Clinician estimates	Utility modelling		Commercial agreement
10	Patisiran	Resource utilisation est	Clinician estimates	Utility modelling		Commercial agreement
11	Voretigene neparvovec	Resource utilisation est	Model approach	Utility estimates	Utility modelling	Commercial agreement
12	Cerliponase alfa	Resource utilisation est	Model approach	Clinician estimates		Commercial agreement
13	Volanesorsen	Resource utilisation est	Model approach	End points		Commercial agreement
14	Metreleptin	Model approach	Utility estimates	Resource utilisation		Commercial agreement
15	Zolgensma	Utility modelling	Utility estimates	Clinical endpoint		Commercial agreement
16	Givosiran	Resuoruce utilisation est	Clinician estimates	Utility modelling		Commercial agreement

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