NICE HIGHLY SPECIALISED TECHNOLOGY ASSESSMENT: HOW ARE SELECTION CRITERIA APPLIED IN PRACTICE?

Abbey Child[1]

[1]Cogentia, Cambridge, UK
Contact the author via cogentia.co.uk/contact

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BACKGROUND

- NICE introduced the highly specialised technology (HST) programme in 2015 to consider drugs for very rare conditions. Willingness-to-pay thresholds in HST are higher and evidence requirements generally lower than in standard technology appraisals (TAs).
- Topics are selected based on seven criteria, all of which must apply for the drug to be assessed via the HST programme.
- The seven HST criteria are listed to the right.

OBJECTIVE

- This study compared the number of criteria met by orphan designation drugs assessed through the HST programme versus those assessed by the standard TA process.

METHODS

- Drugs receiving orphan designation from the European Medicines Agency were filtered on the EMA website (https://www.ema.europa.eu/en/medicines) using the following criteria:

Categories: Human + Medicine: EPAR + Medicine type: Orphan Medicine + Authorisation status: Authorised + MA date: 31/12/2013 – 24/05/2019

- EMA and NICE records for orphan drugs were extracted. Data points extracted were:

- Trade name and compound
- Date of EMA authorization
- Approved EMA indication
- NICE appraisal pathway

- Drugs were grouped according to their actual NICE appraisal pathway (HST or TA) and assigned identification numbers. Five of each appraisal type were randomly selected based on those identification numbers and were compared against HST criteria to assess how many of the seven were met.
- Where possible, assessments were made using evidence presented to or published by NICE, or based on other publicly available documentation in their absence:

Preferred sources
NICE final evaluation determinations or final appraisal document (FAD)
Published HST or TA guidance
Draft or Final Scope

Other sources
EMA documents
Prices published by other agencies (e.g. FDA, CADTH)
Peer reviewed literature

RESULTS

- Seventy-four orphan designated drugs were extracted from the EMA website. Of those, 10 were randomly selected for comparison against HST criteria: 5 assessed by HST and five assessed by standard TA.
- Drugs assessed through HST processes met 5-7 of the 7 criteria, whilst drugs assessed through TA processes met 2-5 criteria.
- Two of 5 HST drugs, and three of 5 TA drugs were judged to meet 5/7 HST criteria.
- Of the 2 HST drugs meeting only 5 criteria, neither met the life-long use criterion, one was not expected to be used in the context of a highly specialised service, and one did not target a clinically distinct patient group.
- Both HST drugs meeting fewer than 7 criteria were the most recently published / scoped, perhaps indicating that for recently scoped drugs, criteria for routing via HST were more relaxed.
- Of TA-assessed drugs, use in a highly specialised service was the criterion most commonly not met for routing via HST (4 of 5 drugs).
- All TA and HST drugs were judged to meet the criterion relating to need for national commissioning, likely due to their unmet need as orphan diseases.
- The table below presents the criteria met by each drug selected for analysis. Green denotes criteria that were deemed as being met whilst red denotes criteria that were deemed as not being met.

Figure 1 Cascade of selected orphan products undergoing technology appraisals by NICE

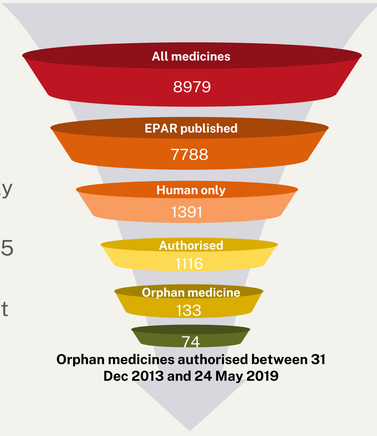

Table 1 Applicability of HST criteria to completed HST and TA appraisals

	HST					TA				
Drug	Velmanase alfa[1-4]	Voretigene neparvovec[1,3-5]	Strimvelis[1,3,4]	Elosulfase alfa[1,3,4]	Sebelipase alfa[1,3,4]	Tezacaftor and ivacaftor[1,3,4,6,7]	Daunorubicin hydrochloride/ cytarabine[1,3,4]	Mogamulizumab[1,3,4,8,11]	Holoclar[1,3,4,9]	Mexiletine hcl[1,3,4,10,11]
Indication assessed	Alpha-Mannosidosis	Retinal dystrophies caused by RPE65 gene	Severe combined immunodeficiency	Mucopolysaccharidosis IV	Lysosomal acid lipase deficiency	Cystic Fibrosis	Acute myeloid leukaemia	Sezary syndrome, mycosis fungoides	Corneal diseases	Myotonic disorders
Status	In progress	In progress	Complete	Complete	In progress	Suspended (2018)	Complete	In progress	Complete	Proposed
Year of publication	(FAD 2018)	(Expected 2020)	2018	2015	(FAD 2017)		2018	(Expected 2020)	2017	(TBC)
Number of criteria met	7	5	5	7	7	5	2	4	5	5
1. Small indication, few treatment centres										
2. Distinct patient group										
3. Chronic and severely disabling condition										
4. Highly specialised service										
5. Very high acquisition cost										
6. Potential life-long use										
7. Significant need for national commissioning										

Abbreviation, FAD, Final Appraisal Document; HST, Highly Specialised Technology; TA, technology appraisal; TBC, to be confirmed
Red = Not met, Green = met

- Main limitation: All judgements were based on an objective assessment of publicly available evidence and materials. It is possible that a different reviewer may have reached a diff erent conclusion. Some criteria, such as exclusive use in the context of a highly specialised service, are less subjective due to their quantification in the Manual for Prescribed Specialised Services[10].

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CONCLUSIONS

- In general, drugs assessed through HST met most, but not all criteria. Orphan drugs in the TA process on average met less criteria, but many met the same number as those assessed via HST. Consequently, some orphan drugs are being assessed via more challenging routes for reasons that are unpredictable and unclear.