

# The HST test: good, better, best?

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## Background

- Despite the significant unmet needs in ultra-orphan indications (prevalence: <1:50,000), therapies for such indications can have difficulties meeting Health Technology Assessment (HTA) clinical- and cost-effectiveness criteria due to low patient numbers limiting the supporting clinical evidence generated; additionally, small patient populations result in high per-patient prices
- Since 2013, NICE appraise Highly Specialised Technologies (HST) (“for use in the provision of services for rare and very rare conditions”) using a distinct appraisal framework

- This research compares NICE HST appraisal outcomes with corresponding guidance by other European HTA bodies, stratified by payer archetype: cost-effectiveness versus clinical-effectiveness

## Methods

- All NICE HST technology guidance was screened (January 1, 2013 to June 3, 2019) alongside corresponding guidance by clinical effectiveness HTAs (HAS, G-BA) and cost-effectiveness HTAs (NCPE, SMC, TLV, and ZIN)

## Results

- As of June 3, 2019, NICE have published nine HST guidance, all with positive recommendations, a median of 20 months (range 7–38) after European MA
  - An additional 11 HST guidance are in development which have had European market authorisation for a median of 14 months (range: 0–53)
    - Of these, six have had draft guidance issued, all being “not recommended” (Table 1)

Table 1: HST guidance issued / in development and the corresponding guidance from G-BA, HAS, NCPE, SMC, TLV & ZIN

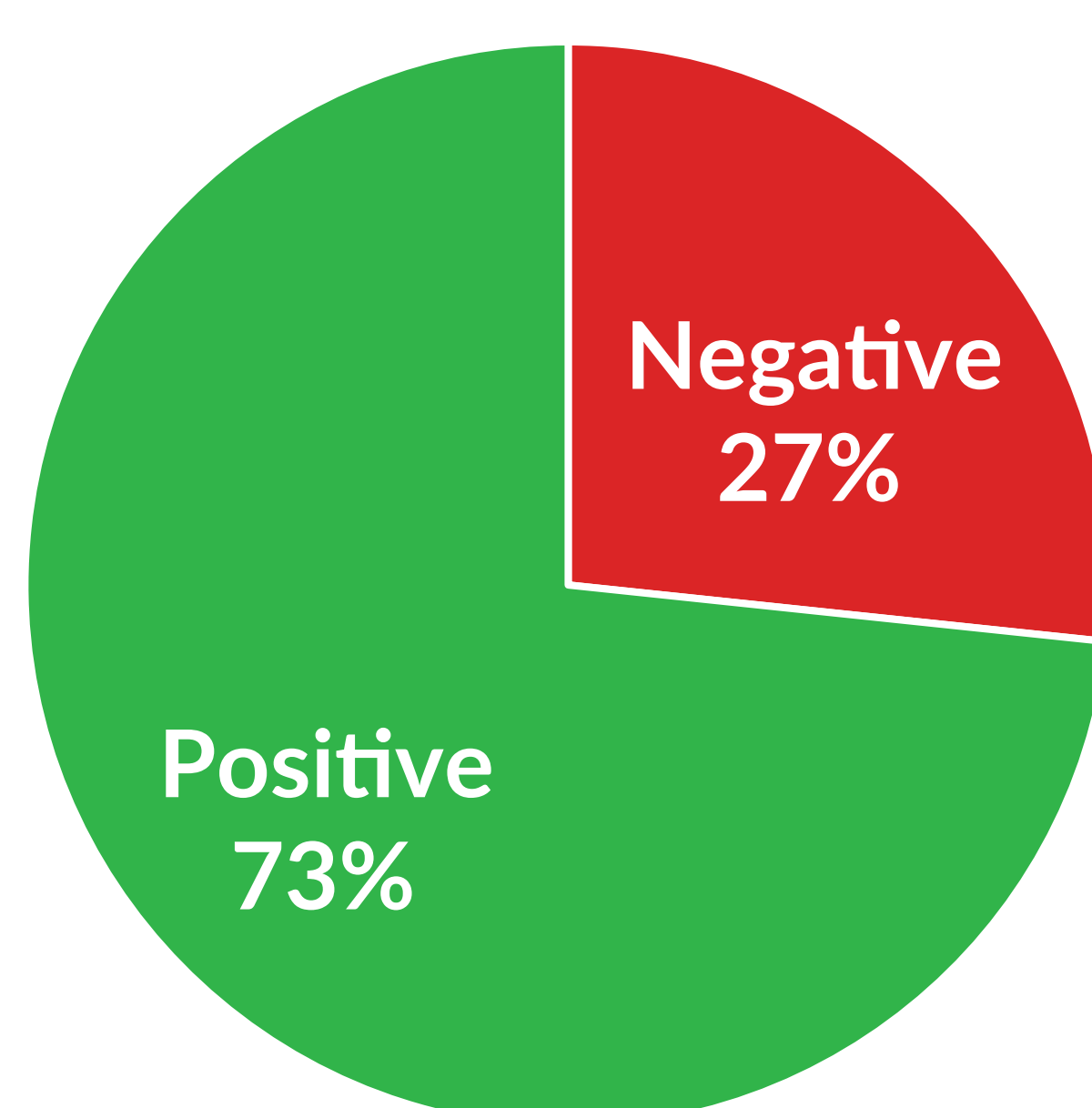
Drug	NICE HST	G-BA	HAS	NCPE	SMC	TLV	ZIN
Eculizumab	Recommended		ASMR II	Not recommended	Not recommended		Recommended
Elosulfase alfa	Recommended	Minor benefit	ASMR III	Recommended	Not recommended		Not recommended
Ataluren	Recommended	Minor benefit	ASMR V	Not recommended	Not recommended	Recommended	Not recommended
Eliglustat	Recommended	Unquantifiable benefit	ASMR V	Not recommended	Recommended	Not recommended	Not recommended
Asfotase alfa (Strimvelis)	Recommended						Recommended
Migalastat	Recommended	Unquantifiable benefit	ASMR IV	Recommended	Recommended		Not recommended
Burosumab	Recommended	Unquantifiable benefit	ASMR III	In development			
Afamelontide	(ACD) Not recommended	Unquantifiable benefit			In development		
Sebelipase alfa	(ACD) Not recommended	Unquantifiable benefit	ASMR III	Not recommended	Not recommended		
Human alpha1-proteinase inhibitor	(ACD) Not recommended	Unquantifiable benefit	ASMR V	Not recommended			
Cerliponase alfa	(ACD) Not recommended	Unquantifiable benefit	ASMR III	Not recommended			
Velmanase alfa	(ACD) Not recommended	Unquantifiable benefit	ASMR IV				
Inotersen	Recommended	Unquantifiable benefit	ASMR IV		In development		
Metreleptin	In development	Unquantifiable benefit	ASMR IV				
Patisiran	(ACD) Not recommended	Considerable benefit	ASMR III		In development		
Eteplirsen	In development						
Volanesorsen	In development						
Voretigene neparvovec	In development	In development	ASMR III				

KEY: ■ = negative outcome ■ = restricted/moderate ■ = positive outcome ■ = in development ■ = not appraised

- Of the 20 HSTs with NICE guidance published/in-development, 16, 14, 8, 6, 2, and 6 were assessed by HAS, G-BA, NCPE, SMC, TLV, and ZIN, respectively
  - Of these, 22/30 (73%) and 7/22 (32%) of assessments made by clinical-effectiveness and cost-effectiveness HTA bodies received positive outcomes, respectively, with median delays between European MA and positive appraisal outcomes of 7 and 39 months, respectively (Figure 1)

Figure 1: Corresponding HST guidance issued by: clinical-effectiveness HTAs (G-BA & HAS) vs. cost-effectiveness HTAs (NCPE, SMC, TLV & ZIN)

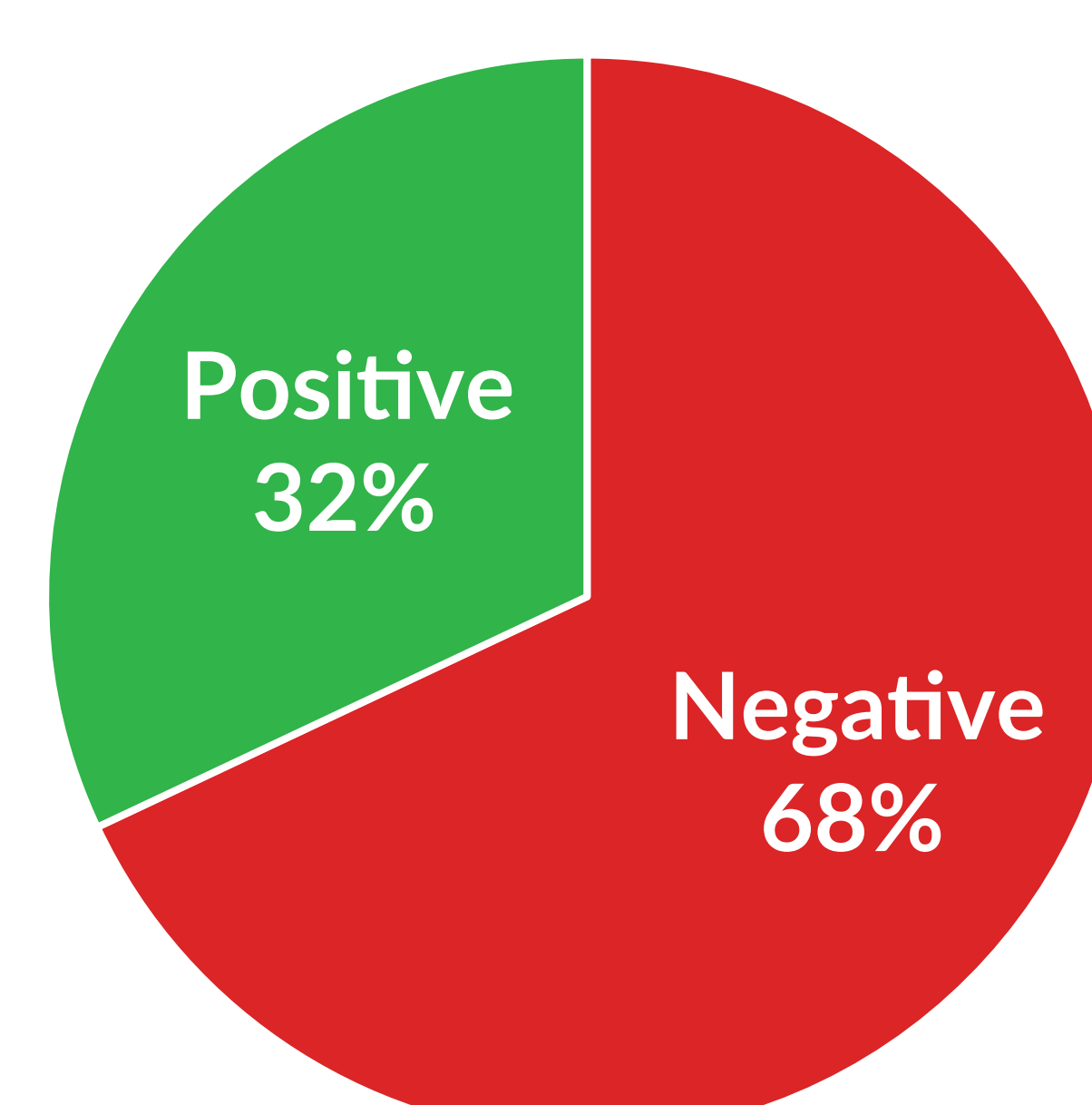
(A) Clinical-effectiveness



Median delay EC-approval to positive HTA

7 months

(B) Cost-effectiveness



Median delay EC-approval to positive HTA

39 months

Note, market access is immediate in Germany despite the delay from regulatory approval to G-BA decision

## Conclusions

- NICE HST appraisals have resulted in more positive recommendations and have faster time to recommendations following European MA than corresponding assessments by other cost-effectiveness HTA bodies
- However, compared with clinical-effectiveness HTA bodies, the recommendation rates are lower and time to positive recommendation is substantially delayed
- In 2018, a new SMC appraisal framework was introduced, whereby ultra-orphan therapies would be made available for ≥3-years while additional evidence is collected pending a final SMC appraisal. This could potentially prove a more suitable best-practice model for ultra-orphan HTA

ACD: appraisal consultation document; ASMR: Amélioration du service médical rendu; EC: European Commission; G-BA: Gemeinsamer Bundesausschuss; HAS: Haute Autorité de Santé; HST: Highly Specialised Technology; HTA: health technology assessment; MA: marketing authorisation; NCPE: National Centre for Pharmacoeconomics; NICE: National Institute of Health and Care Excellence; SMC: Scottish Medicines Consortium; SMR: Service médical rendu; TLV: Tandvårds- och läkemedelsförmånsverket; ZIN: Zorginstituut Nederland.

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