

A Systematic Review of the New NICE Highly Specialised Technologies Criteria: How do NICE Judge Criteria 3 and 4 to be Met?



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We investigated how NICE judge Criteria 3 and 4 to be met.

Objectives

Since February 2022, eligibility for the National Institute for Health and Care Excellence (NICE) Highly Specialised Technologies (HST) programme has been based on four revised criteria¹:



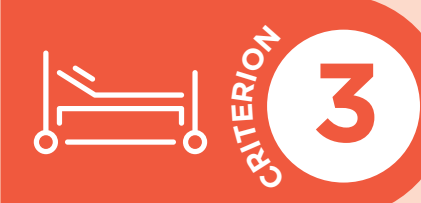
The disease is very rare

Defined as
1:50,000
in England



**No more than 300 people
in England are eligible in its
licensed indication**

And no more than 500
people across all indications



**The disease significantly
shortens life**

Or severely impairs quality
of life (QoL)



**There are no other
satisfactory treatment
options**

Or the technology is likely to
offer significant additional
benefit over existing options

Method

Targeted literature review

A targeted literature review was conducted to assess previous research into this topic, finding no published research that analysed the outcome of applications using the new HST criteria.

Systematic review

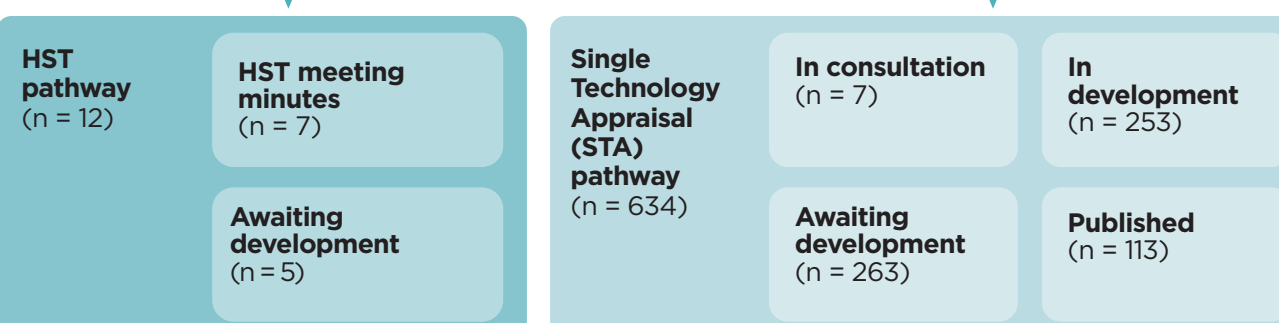
NICE 'guidance in development' and HST committee meeting minutes published from 01/02/2022 (launch of new HST criteria) to 03/03/2023 (date of search) were systematically reviewed to identify all published HST checklists; these summarise whether a technology meets each criterion and why.

The decision on whether the four criteria were met/not met and the description of how the technology met/did not meet the criteria were extracted from each checklist.

Systematic search flow

Search conducted for checklists published from the launch of the new HST criteria (01/02/2022) to the date of search (03/03/2023)

Health technology evaluations identified through the NICE website and HST committee meeting minutes (N = 646)



HST checklists identified (n = 10)

Achieved HST routing* (n = 1)

Did not achieve HST routing and proceeded to the STA pathway (n = 9)

*This product was originally proposed to be appraised via STA but was deemed to meet the HST criteria at a later date following a request from the company, and is currently going through the HST process (GID-TA10854).

Data analysis

1 The ten available checklists were ranked^a in order of the impact of the condition on life expectancy and QoL (Criterion 3) and on the severity of the unmet need (Criterion 4)

2 Descriptive statistics were generated to characterise the data included and outcomes of each application, with a focus on Criteria 3 and 4

3 The ranked tables were used to generate estimates of what was considered a 'significant' reduction in life expectancy/a 'severe' QoL impairment (Criterion 3) and a 'satisfactory' existing treatment (Criterion 4)

4 Due to differences in the level and type of data contained within the checklists included in this review, additional insights were synthesised qualitatively

^aBased on the judgement of the data extractor.

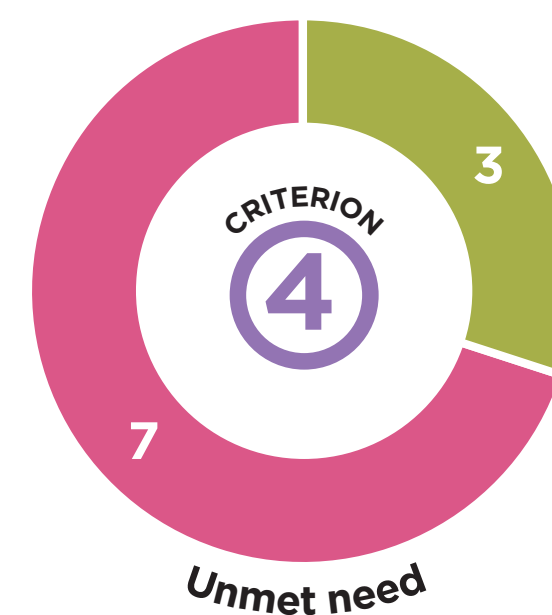
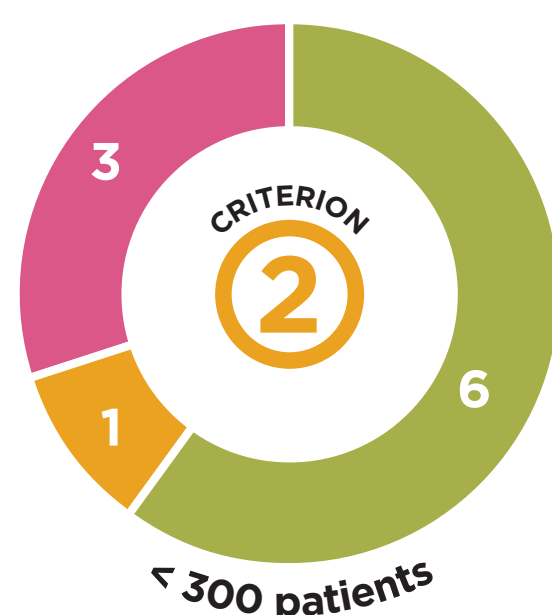
Results

Ten HST checklists were identified; of these, 6 checklists met Criterion 3 and 3 checklists met Criterion 4

From available checklists, Criterion 4 was the least likely to be met

Decision for each criteria from available checklists (N = 10):

Not met Unclear Met



Although the likelihood of meeting Criteria 1-3 ranged from 50-60%; 70% of therapies considered for HST failed to meet Criterion 4



What is considered a 'significant' reduction in life expectancy?

For therapies that met Criterion 3 and included data on life expectancy (n = 5), only one HST checklist presented median life expectancy for patients with the condition (66.5 years).

The remaining four met Criteria 3 based on survival rates (n = 3) or a qualitative appraisal of the impact of clinical features on mortality (n = 1). A 20-year survival rate of 75%, a 5-year survival rate of 67% and a 1-year survival rate of 30% for men and 25% for women were all sufficient to meet Criterion 3.



What is considered a 'severe' impairment of QoL?

Three therapies met Criterion 3 and included data on QoL. All conditions were associated with a multi-faceted and unambiguous QoL detriment:

- 1 Pruritus (itching) associated with cholestatic disease in Alagille syndrome severely impacts all aspects of QoL.
- 2 Primary clinical features of Bardet-Biedl syndrome are rod-cone dystrophy, polydactyly, obesity, learning disabilities, renal abnormalities and hyperphasia. Preoccupation with food dominates life and leads to obesity.
- 3 CDKL5 deficiency disorder (CDD) severely impacts QoL (frequent and severe seizures). Approximately 84% of patients with CDD will ultimately experience treatment-resistant, refractory seizures.



Criterion 4 was only met based on lack of treatment options (n = 3), rather than a significant benefit over existing therapies.

If alternative treatments exist, evidence of a statistically significant benefit over standard of care seems to be required (improved convenience/safety appears insufficient).

"Cipaglucosidase alfa with miglustat did not show a significant benefit over alglucosidase alfa in primary endpoint"

Excerpt from HST checklist: Cipaglucosidase alfa for Pompe disease

"Although the therapy is expected to offer some benefits over existing therapies, these are not expected to be significant. Expected benefits: Less frequent dosing schedule, reduced immunogenicity and fewer infusion-related reactions"

Excerpt from HST checklist: Pegunigalsidase alfa for Fabry disease

Lack of adequate treatments must also apply to a significant proportion of the patient population.

"The clinical benefit from mFOLFOLFOX (standard of care) may be limited but this is uncertain... as there are treatment options for the substantial proportion of people for this indication, this criterion is not considered as clearly met"

Excerpt from HST checklist: Ivosidenib for cholangiocarcinoma

Conclusion



Criterion 3 has been met with a median life expectancy of

66.5 years

or a 20-year survival rate of **75%**



Criterion 4 is the most difficult to meet, with high thresholds for evidence given the rarity of the conditions involved



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List of abbreviations and references

CDD, CDKL5 deficiency disorder; HST, highly specialised technology; ISPOR, The Professional Society for Health Economics and Outcomes Research; NICE, National Institute for Health and Care Excellence; QoL, quality of life; STA, Single Technology Appraisal; TLR, targeted literature review; mFOLFOLFOX, modified FOLFOLFOX regimens (folinic acid, fluorouracil and oxaliplatin).

1. National Institute for Health and Care Excellence. NICE health technology evaluation topic selection: the manual. Available from: <https://www.nice.org.uk/process/pmg37>. Accessed October 2023.

Conflict of interest declaration: AB and SCH are both employees of Takeda and own shares in Takeda.

