Is Ultra-Orphan Disease Treatment Access Equitable in the UK? Comparison Between SMC and NICE HST Guidance

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Introduction

Highly specialised technology (HST) evaluations in England are recommendations by the National Institute for Health and Care Excellence (NICE) on the use of new and existing highly specialised medicines for very rare conditions within the National Health Service (NHS).

In Scotland, treatments considered eligible within the ultra-orphan pathway after initial assessment are available on the NHS; and the Scottish Medicines Consortium (SMC) will reassess the medicine and decide on routine availability after 3 years following full assessment.

We aimed to compare the SMC decisions for the treatments identified for NICE HST evaluation.

Methods

Database	NICE National Institute for Health and Care Excellence https://www.nice.org.uk/guidance/published Scottish Medicines Consortium https://www.scottishmedicines.org.uk/medicines-advice/
Date	Database inception to June 30th 2021
Scope	All published NICE Highly Specialised Technologies Exclude: proposed, in consultation, in development appraisals
Extracted data	Corresponding SMC decisions compared with NICE, time to published decisions, commercial agreements, rationale for non acceptance

Results

As of 30th June 2021, NICE has published 14 HST guidance reports with approved use subject to discount or managed access agreements. Corresponding searches for Scotland of these treatments found that: 3 had not been reviewed, 4 were ultra-orphan initial assessments (considered eligible and therefore available), and 7 were full assessments (Figure 1).

Across the 14 published NICE reports, 4 reports by the SMC were published before NICE (mean 93 days earlier), 7 reports were reviewed by NICE before the SMC (mean 224 days earlier), and 3 reports had not been reviewed by the SMC.

Of the 7 full assessments by the SMC, 3 (43%) were approved, 1 (14%) was approved with restrictions, and 3 (43%) were not recommended (Table 1). The approved and approved with restricted use treatments all included a patient access scheme. The main reason for non-recommendation by the SMC was a lack of justification of the treatment cost in relation to the health benefits. The economic analyses were also generally considered not sufficiently robust to gain acceptance.

Figure 1: SMC submission and approval status of treatments approved by NICE HST guidance

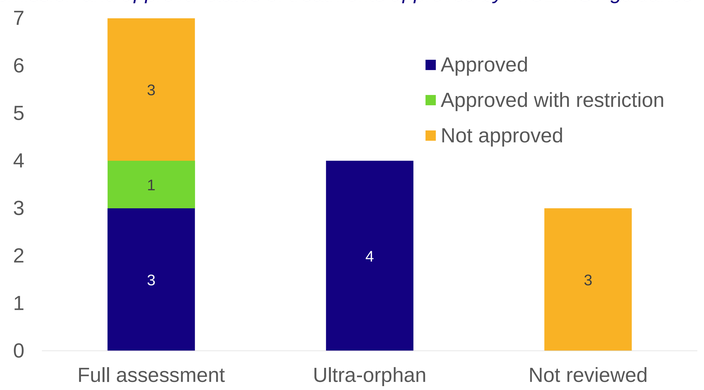

Table 1: Comparison of full published SMC decisions with approved NICE HST guidance

Therapy and indication	NICE ref number	NICE published	SMC published	Days difference SMC to NICE	SMC decision
Patisiran for hereditary transthyretin amyloidosis	HST10	14-Aug-19	10-Jun-19	-65	Accepted for use
Inotersen for hereditary transthyretin amyloidosis	HST9	22-May-19	12-Aug-19	82	Accepted for use
Eliglustat for type 1 Gaucher disease	HST5	28-Jun-17	11-Dec-17	166	Accepted for use
Migalastat for Fabry disease	HST4	22-Feb-17	7-Nov-16	-107	Accepted for restricted use
Ataluren for Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene in ambulatory patients aged ≥5 years	HST3	20-Jul-16	11-Apr-16	-100	Not recommended
Elosulfase alfa for mucopolysaccharidosis type IVa	HST2	16-Dec-15	7-Sep-15	-100	Not recommended
Eculizumab for atypical haemolytic uraemic syndrome	HST1	28-Jan-15	7-Feb-16	375	Not recommended

Conclusions

Despite the rare nature of the conditions, there were differences in speed and decisions between England and Scotland, primarily driven by the justification of treatment cost in relation to health benefits. Patient and clinician engagement (PACE) in Scotland has raised a potential equity issue with HSTs approved in other parts of the UK that are not accepted for use in NHS Scotland. It remains to be seen if the ultra-orphan initial assessments will follow HST approvals in the future with the required updated submissions from the manufacturer for reassessment to allow decision for routine use in NHS Scotland.