Exploring Potential Inequality in Access to Medicines for Ultra-Rare Disease in England and Scotland: SMC Ultra-Orphan Pathway Versus NICE HST

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BACKGROUND

- In 2018 the Scottish Medicines Consortium (SMC) introduced a new approach to the assessment of ultra-orphan (UO) medicines to enable faster access to new treatments for technologies meeting the following UO definition:
  - Condition prevalence of ≤1/50,000
  - Great Britain orphan marketing authorisation from the Medicines and Healthcare products Regulatory Agency
  - Chronic and severely disabling disease
  - Highly specialised management is required
- The National Institute for Health and Care Excellence (NICE) highly specialised technology (HST) evaluation process for very rare conditions has been in place since 2013. Technologies are selected for this appraisal route if they fulfil the following criteria[2]:
  - Very rare disease defined as: prevalence in England <1 in 50,000
  - Maximum of 300 eligible people for a licensed indication and 500 across all indications per technology
  - Rare disease significantly shortens life or severely impairs quality of life
  - No other satisfactory treatment exists or the technology under evaluation offers significant additional benefit over current options
- Our objective was to compare the outcomes and timing of decisions made by the two Health Technology Assessment (HTA) agencies.

METHODS

- All SMC recommendations that were issued via the UO pathway between 1 October 2018 to 22 September 2023 were identified from the SMC website.
- For each SMC ultra-orphan technology assessment, the corresponding NICE appraisals (if available) were then identified from the NICE website.
- For the appraisals identified, the following parameters were extracted from the NICE and SMC websites:
  - Date of marketing authorisation
  - Publication date of SMC and NICE decision
  - NICE routing: HST or single technology appraisal (STA)
  - HTA outcomes including whether a recommendation was optimised (restricted) or subject to a period of managed access.
  - Number of NICE committee meetings
- The average time from marketing authorisation to publication of HTA decision was calculated.

RESULTS

- A total of 21 SMC decisions were published that utilised the SMC UO pathway within the review period. All technologies were granted interim access in Scotland. Of which 2 have undergone reappraisal and routinely approved.
- The distribution of therapeutic areas covered by these 21 recommendations is shown in Figure 1. The highest proportion were conducted in oncology (24%).
- Of the 21 UO recommendations in Scotland, 19 also had published NICE guidance. The remaining 2 had NICE guidance in development.
- 52% of SMC UO products were routed through NICE via HST with the remainder (48%) following the STA route (Figure 2).
- The SMC UO pathway applies to a broader group of technologies than NICE HST. This was further confirmed by an analysis of the HST decisions in the same period, where 87% had been routed via the SMC UO pathway.

Figure 1. Distribution of therapeutic areas across health technology appraisals

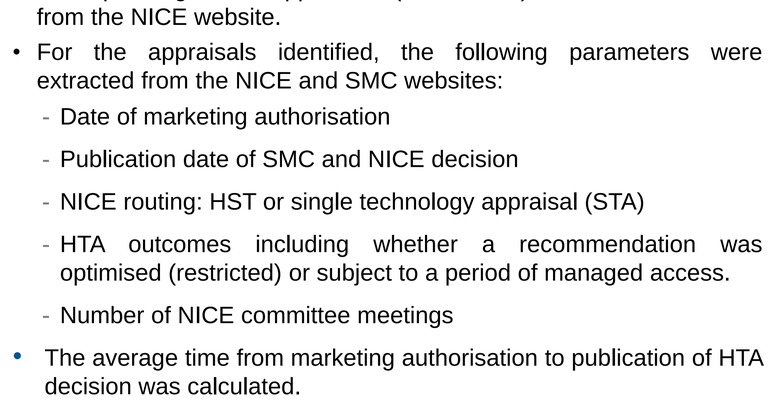
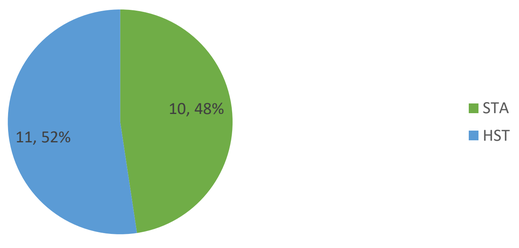


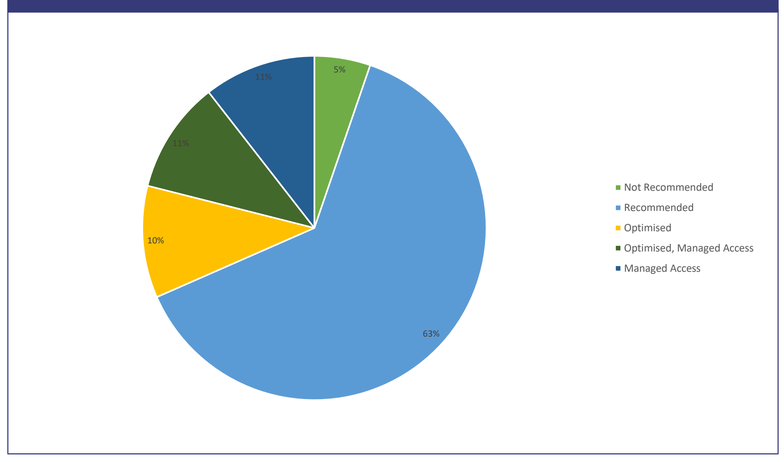
Figure 2. NICE appraisal routing for SMC UO products



NICE technology appraisal decisions for technologies granted access in Scotland via the SMC UO process

- Across the 21 UO products with access in Scotland, a total of 18 were recommended by NICE, 2 were still in-development and 1 was not recommended by NICE.
- Of the 18 that were recommended, 5 were subject to a managed access agreement, of which 4 were routed via the cancer drugs fund (CDF).
- Of those that were recommended by NICE, 22% received an 'optimised' recommendation, indicating access to a restricted patient population only.

Figure 3. NICE appraisal outcomes (n=19)



Timing between marketing authorisation and SMC UO and NICE decisions

- For the technologies identified, the overall average time between marketing authorisation and publication of HTA guidance was 26 months for SMC compared to 21 months for NICE.
- The time between marketing authorisation and publication of NICE guidance was 30 months for those technologies routed via HST versus 19 months for those routed by STA.

Figure 4. Timing between marketing authorisation and publication of HTA guidance

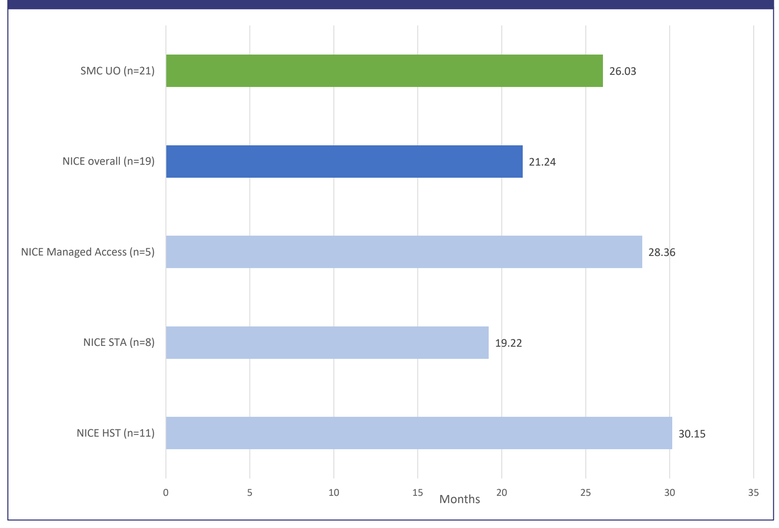
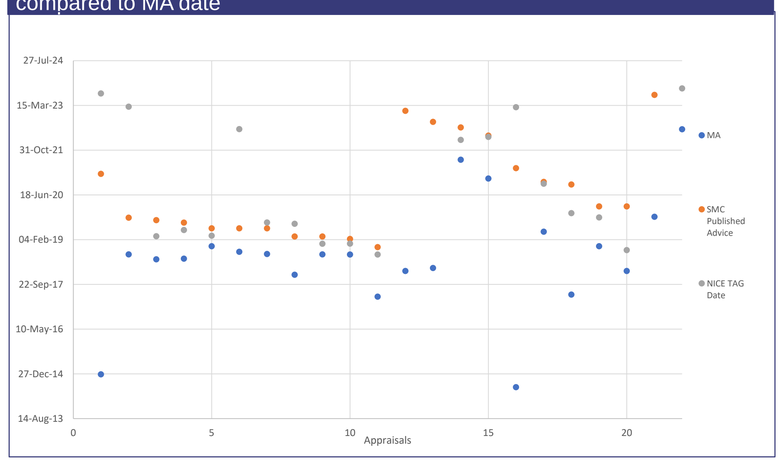


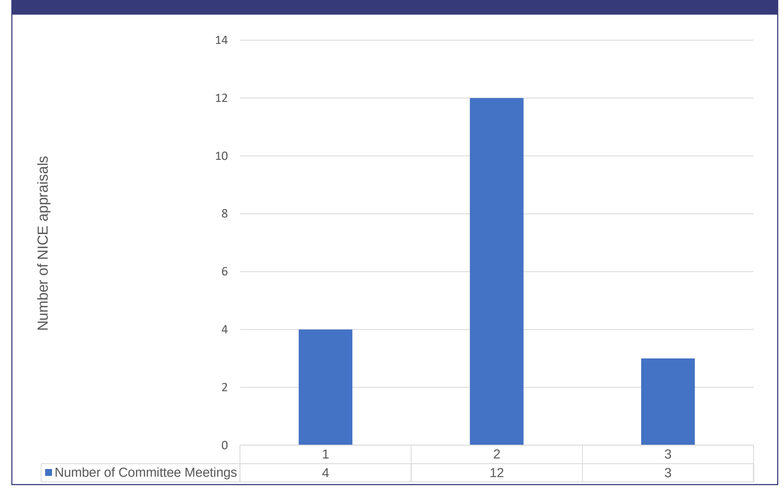
Figure 5. SMC UO appraisals published advice date & NICE published TAG compared to MA date



Number of NICE appraisal committee meetings for technologies granted access via the SMC UO process

- Of the 19 NICE appraisals only 4 (21%) received guidance on the basis of 1 committee meeting.
- 79% required at least 2 committee meetings.
- 16% of NICE appraisals required 3 committee meetings, of which 2 out of 3 were HST appraisals.

Figure 6: Rounds of NICE committee meetings across appraisals



LIMITATIONS

- Given the SMC UO pathway has only been in place since 2018 there was only a relatively small number of decisions on which to base this analysis.
- Timing from marketing authorisation to publication of HTA guidance varied widely and is likely to be influenced by multiple factors including the manufacturer's launch strategy. As such these figures can't be assumed to represent speed of HTA body decision making.
- Company submission dates were not available on the SMC website, so a more accurate comparison of decision speed was not possible.
- Most SMC UO decisions and the NICE managed access decisions constituted interim guidance and cannot be considered final guidance for the technologies.

CONCLUSIONS

- **The assessment of ultra-rare diseases by NICE and SMC are divergent.**
- **The SMC UO pathway applies to a broader group of technologies than NICE HST.**
- **NICE guidance for those products granted access in Scotland via the UO pathway was more restrictive with a higher proportion of technologies being issued a negative or restricted (optimised) recommendation.**
- **Whilst further data collection and reassessment is key to the Scottish UO pathway, only a small proportion of the same medicines were associated with a managed access agreement in England. Where there was a managed access agreement in place this did not result in faster reimbursement.**
- **There was a significant lag between marketing authorisation and HTA guidance publication for both NICE and SMC. For NICE this appears to be even greater for those products that were routed to HST.**
- **Whilst we don't have data comparing decision speed between the two agencies, the fact that most NICE decisions required more than 1 committee meeting suggests that decisions were likely made faster by SMC and that the similar timing presented here may reflect the relative timing of manufacturer NICE and SMC submissions.**
- **Timely access to medicines for rare disease could be improved by more timely submissions to the SMC (i.e., submitting at the same time or before submitting to NICE), a closer alignment of approaches by SMC and NICE to rare and very rare technologies and greater utilisation of managed access, including the innovative medicines fund, in England.**

REFERENCES

1. Ultra-orphan medicines for extremely rare conditions: SMC. Available at: Ultra-orphan medicines for extremely rare conditions (scottishmedicines.org.uk)
2. Heath technology evaluation topic selection: the manual: NICE (2022). Available at: 7 Highly specialised technologies | NICE health technology evaluation topic selection: the manual | Guidance | NICE

DISCLOSURES

NB, KM, OD and RA: Employees of Sanofi

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