Delays in NICE Highly Special Technology (HST) appraisal for medicines that treat ultra-orphan conditions

HTA318

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SUMMARY

OBJECTIVES

- To investigate the time it takes the National Institute for Health and Care Excellence (NICE) to conduct a Highly Special Technology (HST) appraisal following a positive opinion from the European Medicines Agency's (EMA) Committee for Medicinal Product for Human Use (CHMP). (1, 2)

METHODS

- All products (N=26) with publicly available NICE HST appraisal timelines were reviewed alongside the corresponding EMA marketing authorisation (MA) dates.
- In the base case scenario (n=19), the appraisal timelines of 6 HSTs were excluded.

FINDINGS

- This analysis reveals that the mean delay from CHMP opinion to the first CM is 8.3 months and 17.2 months until FED publication. Overall, HST appraisals average 16.1 months, significantly longer than NICE's target of 5.6 months.
- Delays stem from factors inherent to ultra-orphan disease treatments. By increasing transparency and taking an evidence-based and pragmatic approach to the process, industry and NICE could do more to tackle the issues rare disease treatments face.

BACKGROUND & AIMS

- Designed to accommodate the challenges of evaluating rare disease treatments, in England, treatments for ultra-rare diseases can undergo the Highly Special Technology (HST) appraisal if they meet specific criteria. NICE aims for assessments to be completed within about 5.6 months, but delays in this process are common, often due to issues associated with rare diseases such as a lack of evidence.
- The aim of this study is to investigate the time it takes the National Institute for Health and Care Excellence (NICE) to conduct a HST appraisal following a positive opinion from the European Medicines Agency's (EMA) Committee for Medicinal Product for Human Use (CHMP) (1, 2).

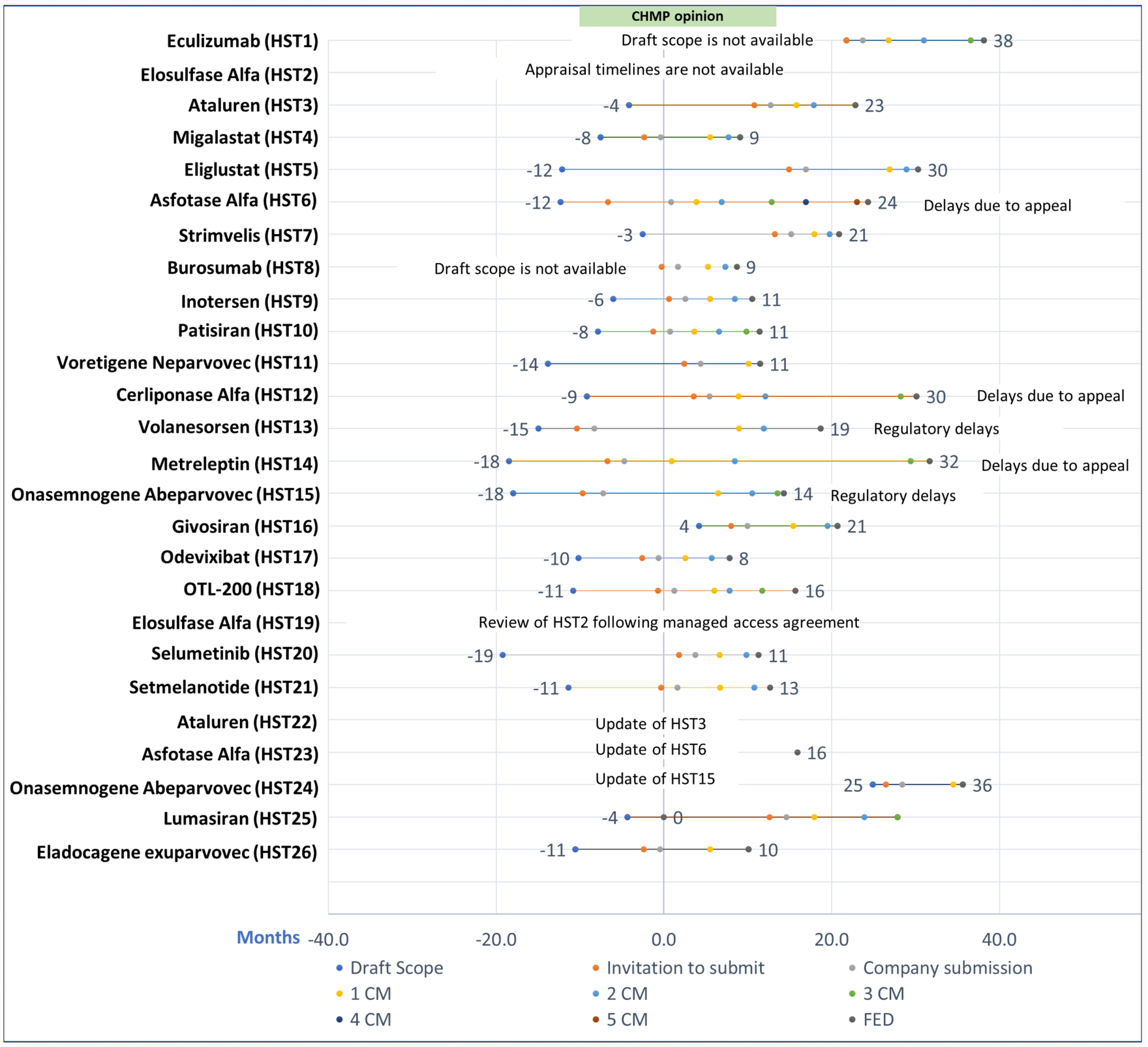
METHODS

- All products (N=26) with publicly available NICE HST appraisals timelines were reviewed, alongside the corresponding EMA marketing authorisation dates.
- In the base case scenario (n=19), the appraisal timelines for Soliris (Eculizumab) (HST1), Givlaari (Givosiran) (HST16) and Vimizim (Elosulfase Alfa) (HST2/19) were omitted, because the draft scope of Soliris and Givlaari were only available after the CHMP opinion was given, and the original appraisal timelines for Vimizim (HST2) are no longer publicly available.
- Ataluren (Translarna) (HST22), Asfotase alfa (Strensiq) (HST23) and Onasemnogene abeparvovec (Zolgensma) (HST24) were either partial or full updates and replacements of HST3, HST6 and HST15 and were also excluded from the base case.

RESULTS

- From the beginning of the NICE HST programme in 2013 to June 2023, 26 products (HST1-26) have been appraised (Figure 1).
- The mean duration from NICE's invitation to submit to Final Evaluation Determination (FED) publication is 16.1 months (median: 12.7; range: 7.7 – 38.4).
- In the base case scenario, the mean duration from CHPM opinion to the first Committee meeting is 8.3 months (6.4; 0.9 – 26.9); the mean duration from CHPM opinion to publication of the FED is 17.2 months (14.3; 7.9 – 31.7).
- All appraisals apart from Luxterna (Voretigene Neparvovec), Onasemnogene abeparvovec (Zolgensma) and Eladocagene exuparvovec (Upstaza) (n=22, 88%) required 2 or more committee meetings, while 8 (32%) required 3 or more committee meetings.

Figure 1. HST timelines.



CONCLUSIONS

- NICE schedules appraisals so that the first Committee meeting is as soon as possible after positive CHMP opinion. However, our analysis has shown that the mean delay from CHMP opinion to the first Committee meeting is 8.3 months and subsequent FED publication is 17.2 months. These delays are greater than anticipated, and in stark contrast to oncology products, which have a reported mean delay of 11 months from CHMP opinion to availability to patients. In addition, the mean HST appraisal length is 16.1 months, which is significantly longer than the NICE target of 5.6 months (168 days).
- Several factors contribute to these delays, many of which are inherent to medicines that treat ultra-orphan diseases, such as sparsity of data, development of de novo health economic models, expected high costs of medicines, marketing authorisation delays, and appraisal scheduling delays to name a few. However, industry and NICE could do more to tackle these issues through open communication and taking an evidence-based and pragmatic approach to the process.

References

1. *NICE website*
2. *EMA website*

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