HAS THE NICE METHODS AND PROCESSES UPDATE ACCELERATED ACCESS TO RARE DISEASE TECHNOLOGIES ROUTED VIA THE SINGLE TECHNOLOGY APPRAISAL (STA) PATHWAY?

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BACKGROUND

- NICE uses two appraisal pathways to evaluate medicines, namely the STA and Highly Specialised Technologies (HST) pathways.
- Due to the highly restrictive routing criteria, most rare disease technologies are not appraised via the HST route, and are instead appraised via the STA route, which has previously been deemed inadequate by manufacturers to account for the complexities typically associated with rare diseases[1]
- On 1st February 2022, NICE implemented its new combined methods and processes manual, with the aim of ensuring earlier access to innovative new treatments to patients by allowing greater flexibility over decisions about value for money and a broader evidence base[2]
- A previous analysis by Hale *et al*. (2023) conducted prior to the implementation of the new combined methods and processes manual reported on protracted access timelines for rare disease technologies appraised via the NICE STA pathway[1]

METHODS

- Non-oncology STAs published from January 1st 2023 to 11th November 2024 were identified and extracted from the NICE website.
- STAs were divided according to rarity (prevalence <1/2,000 people), appraisal committee (A,B,C or D), and year of final appraisal committee meeting (ACM)
- A set of 12 appraisals (six rare disease and six non-rare disease) for comparison and analysis were randomly selected, and each randomly selected rare disease was matched with a non-rare disease according to Appraisal Committee and Year of Final ACM (Table 1)
- For each appraisal, time to access (the number of days from MHRA marketing authorisation to the publication of final NICE guidance), time in NICE (the number of days from the date of dossier submission to the publication of final NICE guidance), and number of ACMs were analysed (Table 1)
- Results were compared to a similar analysis of six rare and six non-rare appraisals conducted prior to the update of the methods and processes manual[1]

OBJECTIVE

- This study explored the impact of the new combined methods and processes manual on access timelines for rare disease technologies routed via the NICE STA pathway

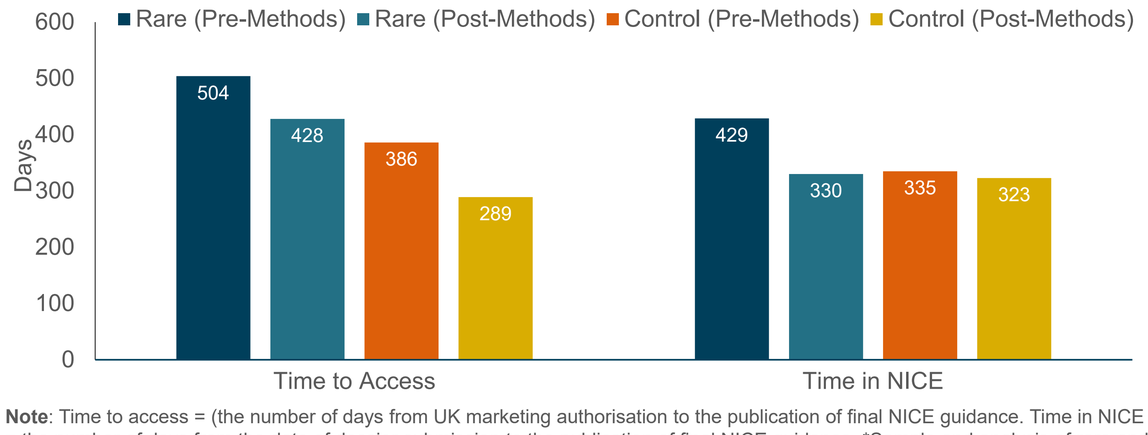
Table 1 Summary of appraisals included in the analysis and key milestones

Appraisal Overview							Timelines			
Generic name	Brand name	Manufacturer	Category (rare/control)	Indication under review	Appraisal year	NICE committee	Date of MHRA Marketing Auth.	Date of NICE Submission	Date of Final Guidance	No. of ACMs
Cipaglucosidase alfa/miglustat	Pombiliti/Opfolda	Amicus Therapeutics	Rare	Late-onset Pompe disease	2023	A	30/06/2023	02/12/2022	15/08/2023	1
Tirzepatide	Mounjaro	Eli Lilly	Control	Type 2 diabetes	2023	A	01/09/2023	17/03/2023	25/10/2023	2
Bulevirtide	Hepcludex	Gilead	Rare	Chronic hepatitis delta	2023	B	16/11/2021	25/04/2022	07/06/2023	2
Deucravacitinib	Sotyktu	BMS	Control	Moderate to severe plaque psoriasis	2023	B	10/05/2023	28/04/2022	28/06/2023	1
Pegunigalsidase alfa	Elfabrio	Chiesi	Rare	Fabry disease	2023	B	07/08/2023	25/01/2023	04/10/2023	1
Secukinumab	Cosentyx	Novartis	Control	Moderate to severe hidradenitis suppurativa	2023	B	01/06/2023	13/12/2022	06/12/2023	2
Evinacumab	Evkeeza	Ultragenyx	Rare	Homozygous familial hypercholesterolaemia	2024	C	26/08/2022	26/05/2023	11/09/2024	2
Linzagolix	Yselty	Theramex	Control	Moderate to severe symptoms of uterine fibroids	2024	C	14/06/2022	26/09/2023	14/08/2024	2
Cannabidiol	Epidyolex	GW Pharma	Rare	Seizures caused by tuberous sclerosis complex	2023	D	10/08/2021	07/06/2022	01/03/2023	2
Rimegepant	Vydura	Pfizer	Control	Preventing migraine	2023	D	10/06/2022	22/06/2022	05/07/2023	2
Belumosudil	Rezurock	Sanofi	Rare	Chronic graft-versus-host disease	2024	D	07/07/2022	20/03/2023	07/02/2024	2
Atogepant	Aquipta	AbbVie	Control	Preventing migraine	2034	D	30/08/2023	29/09/2023	15/05/5024	1

RESULTS

- Time to access for rare and control technologies has reduced since the implementation of the new combined methods and processes manual (Fig. 1)
- Despite this, rare disease technologies appraised via STA are, on average, are still subjected to a greater mean time to access from MHRA authorisation (Fig. 1)
- When compared against the pre-methods update appraisals analysed by Hale *et al* (2023), rare disease technologies spent substantially less time in NICE process (178 days fewer post-methods update), with appraisal timelines comparable to non-rare disease technologies (Fig. 1)
- The average number of ACMs remained similar pre- and post-update (~2 ACMs) (Table 1)

Figure 1 Time to access and time in NICE for rare and non-rare appraisals before and after the implementation of the methods and processes update*



Note: Time to access = (the number of days from UK marketing authorisation to the publication of final NICE guidance. Time in NICE = the number of days from the date of dossier submission to the publication of final NICE guidance. *Sample and analysis of rare and non-rare appraisals pre-methods and processes update taken from Hale *et al*. (2023) (n=12)[1]

DISCUSSION

- Improvements in appraisal timelines for rare-disease technologies infers that the updated NICE methods and processes manual has improved the flexibility and pragmatism provided by NICE towards uncertainties in rare disease technologies
- Notably, the ABPI's CONNIE report recently found that only 2 of 16 sampled rare or ultra-rare disease technologies reported committees accepting a higher degree of uncertainty[3]. Ultimately, there are multiple categories of evidence that must be considered in decision-making.
- Despite the policy changes and subsequent decrease in NICE appraisal timelines, the time to access for rare disease technologies still lags behind that of non-rare disease technologies

CONCLUSIONS

- Despite updates to the NICE methods and processes manual, rare disease technologies are still subjected to similar delays in access timelines
- However, according to our sample, rare disease technologies are now observed to spend less time in NICE appraisal process, with appraisal timelines comparable to those of non-rare disease technologies
- The implementation of the updated NICE methods and processes manual has contributed to the improved applicability and usability of the STA pathway to address uncertainties in rare disease technologies; however, this conclusion is based on a limited number of sampled appraisals and the transparency on the flexibility applied in decision-making remains limited.
- Continued monitoring of rare disease technologies appraised via the STA pathway is required to gather further evidence on whether STA routing is fit-for-purpose for rare disease technologies

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