THE INNOVATIVE MEDICINES FUND OR THE IN NEED OF MEDICINES FUND: ANALYSIS OF NICE TECHNOLOGY APPRAISALS TO EXPLORE BARRIERS TO MANAGED ACCESS AGREEMENTS

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INTRODUCTION

- The Innovative Medicines Fund launched to much fanfare in June 2022, promising to provide a managed access alternative to the highly successful Cancer Drugs Fund for non-oncology drugs[1]
- Like the CDF, the IMF has a ringfenced budget of £340m to deploy on fast-tracking highly promising drugs with significant data uncertainties
- However, as of October 2024, over two years since the IMF's introduction, only two technologies have been recommended for it, both single-dose gene therapies

Table 1 Founding principles of the Innovative Medicines Fund[1]

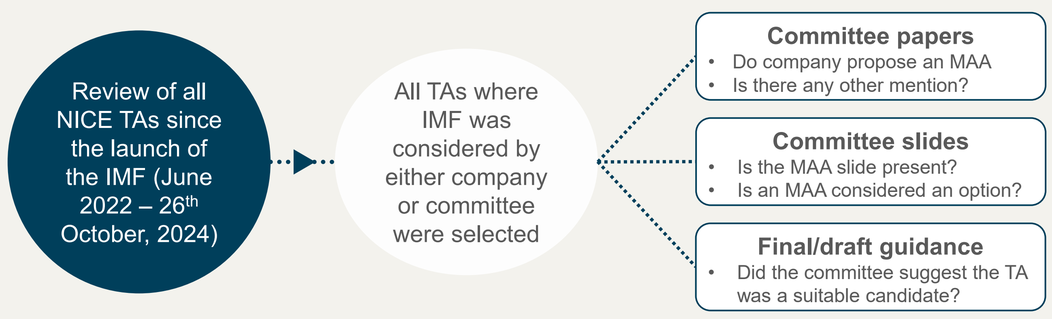
Principle 1: IMF should support equality of opportunity for non-oncology & oncology indications	**Principle 2**: IMF should prioritise the most promising medicines, with significant remaining uncertainty
Principle 3: IMF is reserved for medicines that are a) plausibly cost-effective b) priced responsibly during MAA	**Principle 4:** Managed access should be for the shortest time necessary to collect required data (< 5 years)
Principle 5: the entire eligible population, determined by NICE, should have the opportunity to access treatment	**Principle 6:** all medicines that enter the IMF will be re-evaluated by NICE for a routine decision
Principle 7: any patient treated in the IMF should have the option of continuing in the event of a NICE rejection	**Principle 8:** the IMF should never close to new entrants.

OBJECTIVE

- The objective of this research was to review NICE technology appraisals (TAs) where access through the IMF was considered, to identify key themes emerging and explore barriers to managed access via the IMF

METHODS

- All published non-oncology TAs starting from June 2022 were analysed up to 26th October 2024
- All TAs where the IMF (or managed access more generally) is discussed in the published documentation were included in this analysis
- In addition, all TAs in consultation or development that had a publication date of June 2022 onwards were included. Those without a publication date were excluded on the basis that these TAs are not sufficiently advanced for IMF to have been deliberated, or documentation made public
- For all included TAs, the current recommendation, entry via IMF (yes, no), and rationale where IMF was not utilised were tabulated
- The reasons for not entering the IMF were then assigned to broad categories, to support the development of potential themes & recommendations



RESULTS

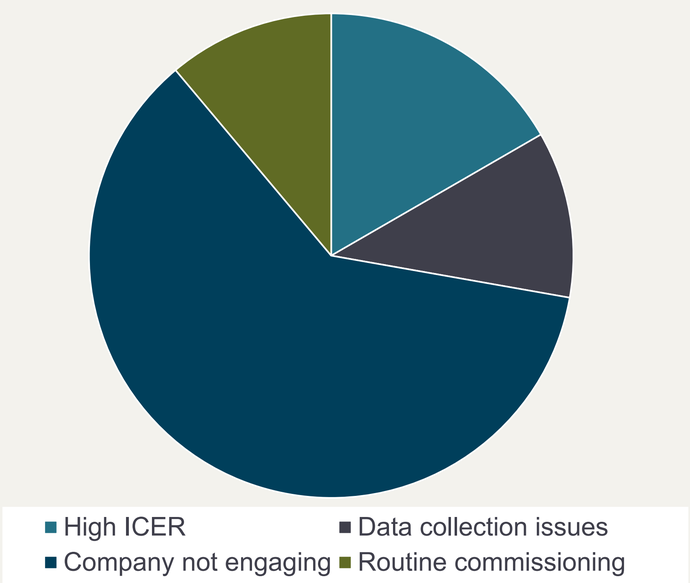
- In total we identified 20 NICE TAs where managed access via the IMF had been explicitly considered an option, either by the manufacturer or NICE (Table 2)
- Of these, 11 TAs have been recommended for routine commissioning, 2 recommended via the IMF, and 7 technologies not recommended
- As of this analysis, 2 technologies were recommended via the IMF, both of which are ATMPs (Exagamglogene autotemcel; etranacogene dezaparvovec)
- Of the 18 technologies not routed to the IMF, the reasons included the company not engaging (11 of 18), a high ICER (3 of 18), data collection issues (2 of 18), and recommendation via routine commissioning (2 of 18)' (Figure 1)

Table 2 Analysis of NICE Technology Appraisals where Managed Access (via IMF) was considered

Technology	Disease Area	Company	TA	Reimbursed?	IMF?	Rationale If No
Voclosporin	Lupus Nephritis	Otsuka	892	Yes	No	Data collection issues
Bulevirtide	Hepatitis Delta	Gilead	896	Yes	No	Company not engaging
Baracitinib	Alopecia	Lilly	926	No (FAD)	No	Prohibitively high ICER
Cipaglucosidase alfa	Pompe Disease	Amicus	912	Yes	No	Not required (routine ACM1)
Mavacamten	oHCM	BMS	913	Yes	No	Company not engaging
Voxelotor	Sickle Cell Disease	GBT/Pfizer	981	Yes	No	Company not engaging
Eladocagene exuparvovec	AADC Deficiency	PTC	HST26	Yes	No	Company not engaging
Efgartigimod	Myasthenia Gravis	Argenx	GID	No (ACD)	No	Company not engaging
Afamelanotide	EPP	Clinuvel	HST27	No (FAD)	No	Prohibitively high ICER
Ganaxolone	CDKL5 Disorder	Orion	GID	No (appeal)	No	Company not engaging
Etranacogene dezaparvovec	Haemophilia B	CSL Behring	989	Yes	Yes	N/A
Setmelanotide	Bardet Biedl	Rhythm	HST31	Yes	No	Company not engaging
Sebelipase alfa	Wolman Disease	Alexion	HST30	Yes	No	Company not engaging
Birch bark extract	Dystrophic EB	Chiesi	HST28	Yes	No	Routine commissioning
Belumosudil	Chronic GvHD	Sanofi	949	Yes	No	Company not engaging
Velmanase alfa	Alpha mannosidosis	Chiesi	HST29	Yes	No	Data collection issues
Olipudase alfa	Niemann-Pick	Sanofi	GID	No (appeal)	No	Prohibitively high ICER
Exagamglogene autotemcel	TDT	Vertex	1003	Yes	Yes	N/A
Vamorolone	Duchenne	Santhera	GID	No (ACD)	No	Company not engaging
Idebenone	LHON	Chiesi	GID	No (ACD)	No	Company not engaging

Key: AADC, Aromatic L-amino acid decarboxylase deficiency; ACD, Appraisal Consultation Document; BBS, bardet biedl syndomre; FAD, Final Appraisal Document; GID, Guidance In Development; GvHD, graft-versus-host disease; LHON, leber's hereditary optic neuropathy; oHCM, obstructive hypertrophic cardiomyopathy; TDT, transfusion-dependent thalassemia.

Figure 1 Reasons for the Innovative Medicines Fund not being utilised



The two IMF entrants: ATMPs

- So far, the only two IMF entrants are ATMPs, i.e. one-and-done treatment options
- Firstly, etranacogene dezaparvovec was recommended via the IMF in July 2024 to address uncertainties including durability of effect & subsequent treatments
- A month later, in August 2024, exagamglogene autotemcel became the second entrant, with the IMF addressing issues including the non-reference discount rate
- Re-submissions post data collection will take place between 2027 & 2028

DISCUSSION

- Our analysis builds on work published in 2023, with very similar findings.
- The overwhelming majority of technologies considered for the IMF do not end up being routed to the IMF, typically due to company decision
- Of 18 instances where a technology considered for an MAA did not enter the IMF, 11 of 18 were due to the company refusing to submit a managed access proposal
- Notably, despite the relatively low number of ATMPs going through NICE relative to non-ATMPs, the only two uses of the IMF thus far are for ATMPs

CONCLUSIONS

- Based on our analysis, the IMF continues to face resistance from manufacturers, with a majority of companies with a potentially eligible technology refusing to engage
- As previously described, reasons for this likely include the need to price responsibly during the MAA (which may mean at the lower end of the WTP threshold), and to provide free drug should NICE not recommend the technology following managed access
- It is important to remember that per principle 7 of the IMF 'any patient prescribed a medicine when it was in the IMF will continue to receive it at the companies cost if NICE does not recommend routine commissioning'
- On this basis, perhaps it is unsurprising to see the only IMF entrants to-date are one-time therapies, whereby the cost is incurred upfront, mitigating the risk of long-term provision of free drug
- For manufacturers of chronically dosed technologies, they may still feel that principle 7 stacks the chips against them when it comes to negotiations with NHSEngland upon conclusion of IMF data collection, explaining their reticence to engage

REFERENCES

1. https://www.england.nhs.uk/wp-content/uploads/2022/06/B1686-the-innovate-medicines-fund-principles-june-2022.pdf