THE IN NEED OF MEDICINES FUND: ANALYSIS OF NICE TECHNOLOGY APPRAISALS TO EXPLORE BARRIERS TO ACCESS VIA THE IMF

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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 2023, some 16 months after launch, we are still awaiting the first technology to be recommended via the IMF

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

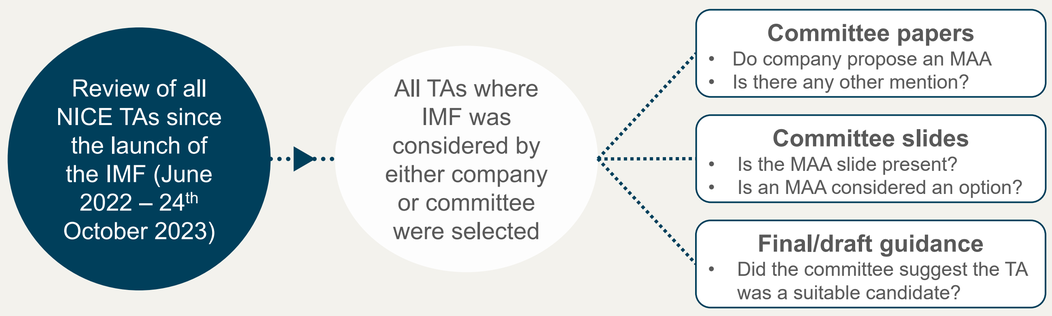
Principle 1: IMF should support equality of opportunity for non-oncology & oncology indications	**Principle 2**: IMF should prioritise the most promising medicines, with significant remaining uncertainty
Principle 3: IMF is reserved for medicines that are a) plausibly cost-effective b) priced responsibly 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 24th October 2023
- 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

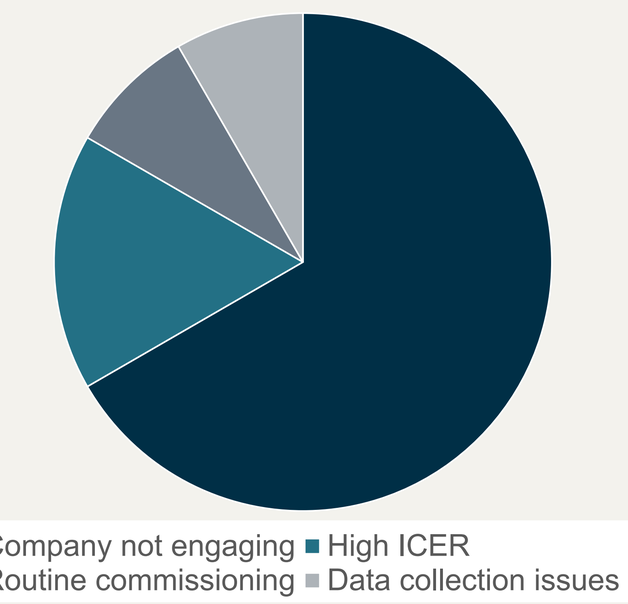
- In total we identified 13 NICE TAs where managed access via the IMF had been explicitly considered an option, either by the manufacturer or NICE (Table 2)
- Of these, 5 TAs have received a positive outcome, 5 received an initial negative recommendation, and 3 have not been recommended by NICE in final guidance
- As expected, none of the 13 TAs have been recommended via IMF
- In 9 of 13 TAs, the reason for not entering the IMF was that the company chose not to submit a proposal for an MAA, with the typical reason being 'the Company is committed to securing a positive routine commissioning decision' (Figure 1)
- Indeed, in multiple cases where a positive recommendation was achieved, the Company elected to submit an increased discount rather than engage in IMF discussion
- Other reasons for the IMF not being used include a prohibitively high ICER meaning the technology could not be considered plausibly cost-effective (2 of 13), data collection issues (1 of 13), and IMF not required due to a routine commissioning recommendation after one committee (1 of 13)

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	GID	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	GID	No (FAD)	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 (ACD)	No	Company not engaging
Entranacogene dezaparvovec	Haemophilia B	CSL Behring	GID	No (ACD)	No	Company not engaging
Setmelanotide	Bardet Biedl	Rhythm	GID	No (ACD)	No	Company not engaging
Sebelipase alfa	Wolman Disease	Alexion	GID	No (ACD)	No	Company not engaging

Key: AADC, Aromatic L-amino acid decarboxylase deficiency; ACD, Appraisal Consultation Document; FAD, Final Appraisal Document; GID, Guidance In Development; oHCM, obstructive hypertrophic cardiomyopathy.

Figure 1 Reasons for the Innovative Medicines Fund not being utilised



Voxelotor case study: avoiding IMF at all costs

- The company stance at submission was that they were committed to securing a positive routine commissioning decision, and as such were not pursuing the IMF
- Notably, the manufacturer claimed that the additional data required to resolve remaining uncertainties could not be generated through an MAA, despite the committee believing it could be (e.g. rate of transfusion)
- The manufacturer maintains this position **despite negative final draft guidance**

DISCUSSION

- Our analysis provides insights that help explore & better characterise the reason the IMF remains unused 16 months into launch
- Companies appear keen to avoid the IMF, despite committees expressing the view that at least some of the TAs analysed would be suitable candidates
- Reasons for not engaging vary slightly by company, but almost always relate to a 'commitment to achieving routine commissioning'
- All of the above suggests manufacturers are unhappy with IMF in current form

CONCLUSIONS

- Based on our analysis, whilst some reasons for not entering the IMF are driven by eligibility criteria (high ICER, data collection issues), the vast majority are driven by the manufacturer actively avoiding the IMF
- In the NICE process, there are two ways to manage uncertainty. One is via managed access, the other is by increasing the confidential discount to achieve routine commissioning. Invariably, companies to date are electing for the latter
- We propose two reasons for this:

1. **Principle 7** stipulates that 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. This long-term commitment likely concerns manufacturers
2. **Principle 3** requires that any drug in the IMF should be priced responsibly. The implication here may be that the achievable price in IMF is lower than via routine commissioning, and manufacturers may not believe the price will go up post-MAA

- In conclusion, whilst managed access is the ultimate buzz phrase in market access, the IMF in its current state demonstrates that it is not a silver bullet, and may not even be an attractive option for manufacturers

REFERENCES

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