NICE National Institute for Health and Care Excellence

NHS

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Response to engagement on proposals for the Innovative Medicines Fund

6 June 2022

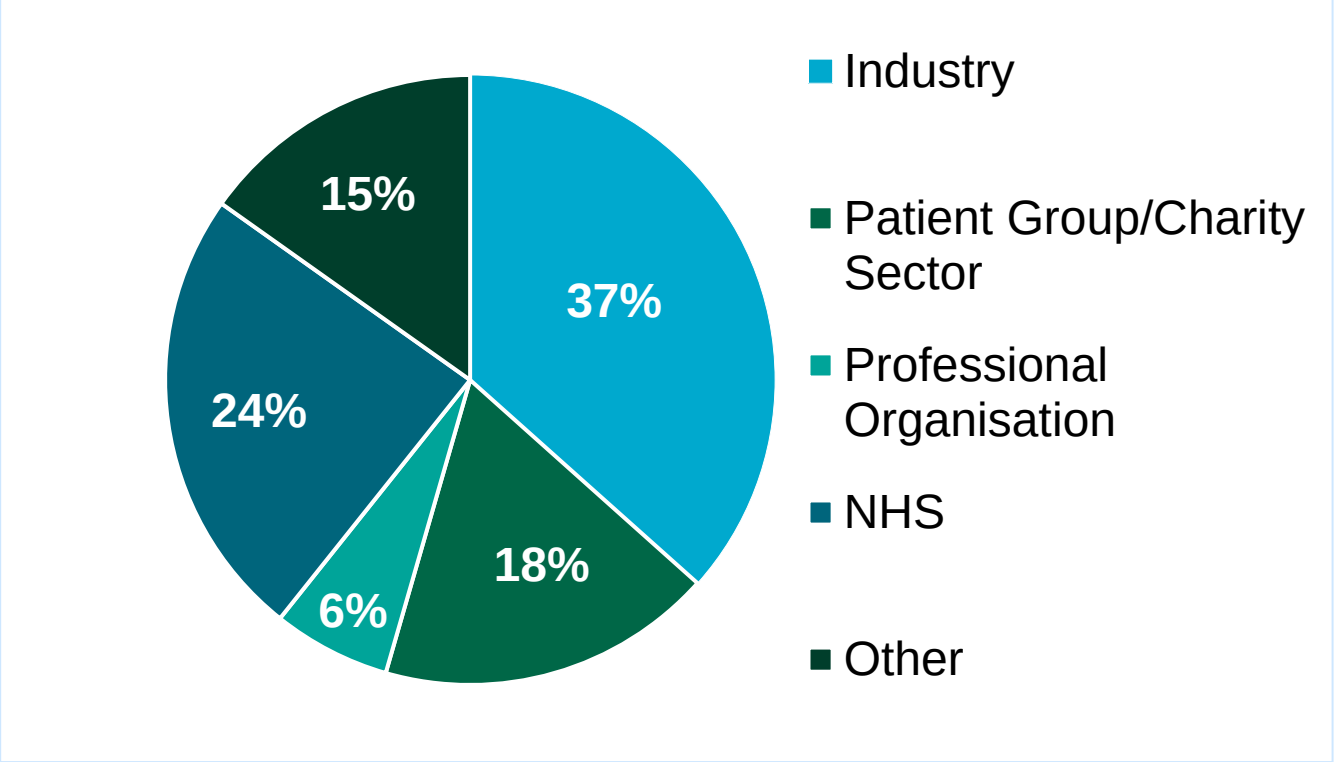
The Innovative Medicines Fund (IMF)

Response to engagement

NHS England and the National Institute for Health and Care Excellence (NICE) asked for comments on our proposals for the IMF. This engagement exercise began on 19 November 2021 and closed on 11 February 2022. As part of the engagement we held two virtual events, via Microsoft Teams.

A total of 112 responses were received with the breakdown provided in **Figure 1.** This document summarises the feedback received from the engagement exercise and the NHS England and NICE response to this feedback.

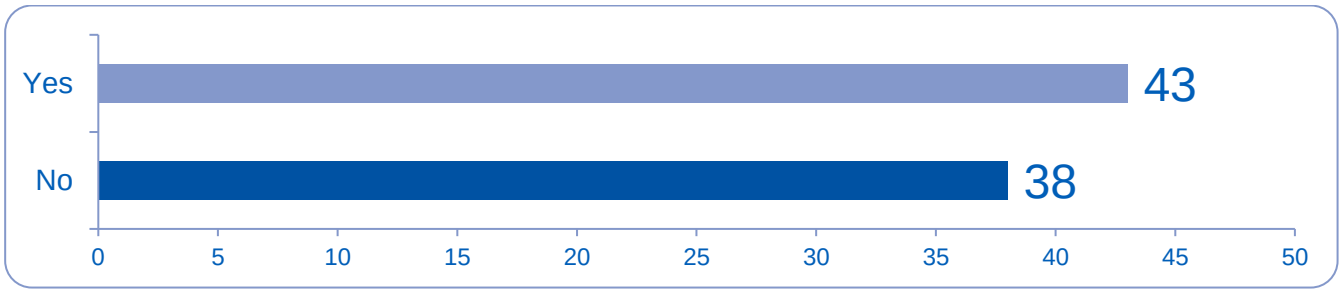
Figure 1: Breakdown of Responses to Engagement Exercise by Sector



Conflict of interest disclosures

Respondents were asked whether they had received any payments, grants or other funding from the pharmaceutical industry* in the last three years**.

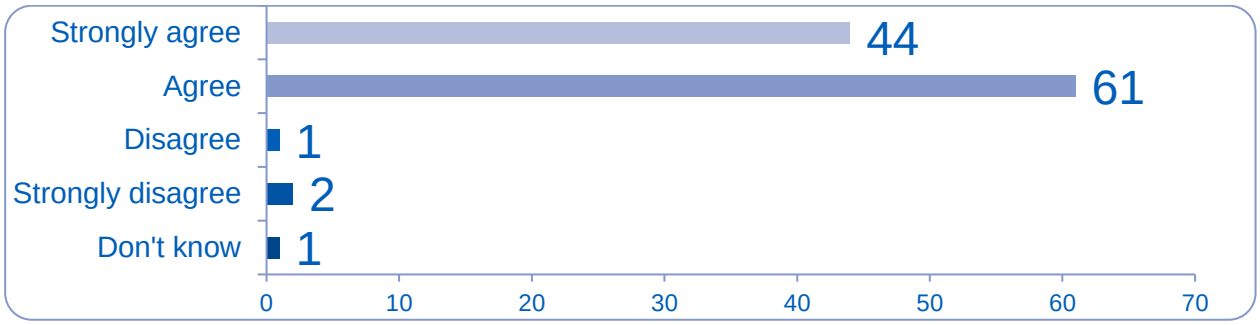
n=81



*Pharmaceutical companies have been excluded from this statistic.

**It should be noted that the responses received via email/written correspondence are not included in the headline statistics.

Question 1: Do you agree with the purpose of the IMF? n=109



Analysis of responses

- There was widespread support for the purpose of the IMF with 96% of respondents either strongly agreeing, or agreeing, with the purpose of the IMF.

  "[We] welcome the creation of the IMF in England, as originally outlined in the government's 2019 general election manifesto. The allocation of £340m per annum of additional funding is good news for patients, the NHS, and the life sciences sector." *Industry Organisation*

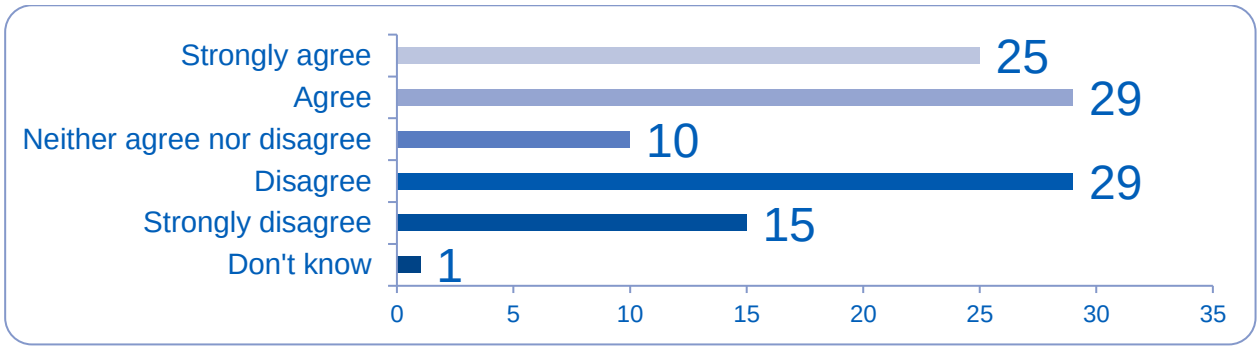
- Many respondents noted their support for extending the opportunities afforded to cancer drugs via the successful operation of the Cancer Drugs Fund (CDF) to non-cancer medicines, such that a wider group of patients could potentially benefit from earlier access to advanced, life-saving treatments.

  "[We] support both the purpose of the IMF, and its establishment alongside but distinct from the CDF. We applaud the decision to ringfence £340m for the IMF, as we welcome any additional funding which aid patients in accessing innovative therapies." *Patient Organisation*

NHS England and NICE Response

- We were encouraged to see such widespread support for the purpose of the IMF across all stakeholder groups. As such, no fundamental changes are proposed to the purpose of the IMF.

Question 2: Do you agree that the IMF should operate alongside, and on similar terms to the Cancer Drugs Fund? n=109



Analysis of responses

- Stakeholders were generally supportive of the IMF operating on the same principles as the CDF, with approximately 50% either strongly agreeing or agreeing. There was a clear acknowledgement that data issues and data collection requirements were likely to be different for non-oncology products and that rare diseases may face a number of nuanced challenges.

  "While the IMF is modelled on the CDF it should not necessarily replicate all aspects of it. It is important that lessons are learned from the experience of the CDF (and other MAAs) and applied beneficially to the IMF." *Pharmaceutical Company*

- Whilst it was welcomed that the IMF would build on the CDF operational model, in general stakeholders stressed the need for pragmatism and a requirement to learn and build from this experience. Some industry respondents were keen for the IMF to show further ambition, suggesting that purely matching the CDF would be a missed opportunity from the perspective of global companies.

  "We would go further here in stressing that the proposals as featured, do not seem to be aligned with the ambitions of the Life Sciences vision in making the UK one of the best places to launch innovative treatments, or harness the power of advances in treatment such as cell and gene therapies." *Pharmaceutical Company*

- A number of patient groups said that they would like further clarity and details on the future direction of the CDF and IMF, including whether the allocated funding would be enough to cover the scope of the IMF. A view also expressed was that because more non-cancer drugs are developed each year, the proportionate benefits for non-cancer drugs will remain less than for cancer drugs through the two managed access funds.

  "Furthermore, Government, NHS England, and NICE should at the earliest opportunity clarify the longer-term policy direction for how the CDF will interlink with the IMF and whether there will be plans to merge the funds." *Patient Organisation*

NHS England and NICE Response

- The IMF builds on the successful operational model for the CDF, which has delivered cancer treatments to over 80,000 patients. The CDF continues to evolve, based on operational experience and this expertise will be applied to ensure the forthcoming IMF supports patient access to promising but uncertain treatments.

- The IMF brings equity by providing up to £340 million per annum for non-cancer medicines creating a total of up to £680 million of ringfenced NHS England funding for early access to potentially life-saving new medicines. This funding means any patient, regardless of their condition, will have equal potential opportunity to benefit from promising but uncertain medicines and reflects the balanced pipeline of new medicines development of cancer versus non-cancer treatments.

- The design of the IMF has been informed by NHS England and NICE's experience from the CDF, other commercial and data collection arrangements and engagement with industry, patient organisations, clinicians, academics and data custodians. Extending the principles and operational expertise of the CDF will therefore build on this experience and ensure consistency of approach.

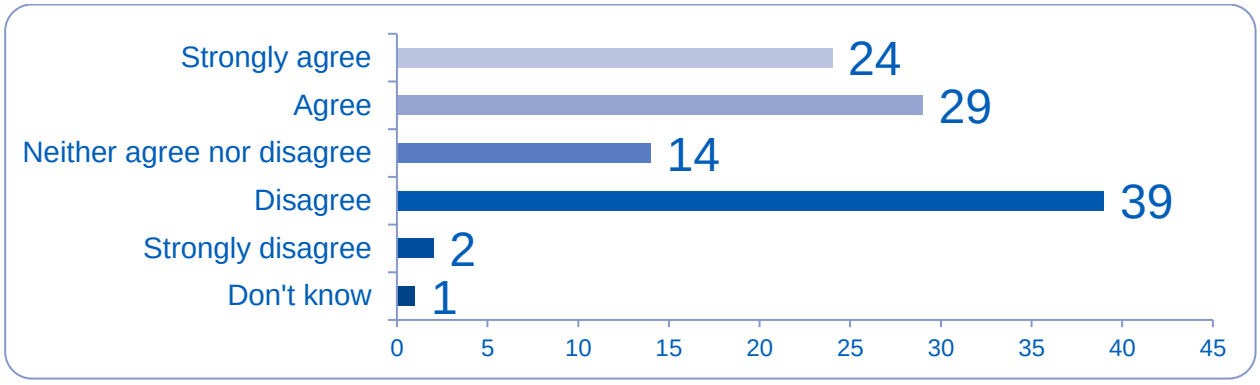
- The operation of both the IMF and the CDF will be kept under continuous review and utilise horizon scanning information to understand the pipeline mix between oncology and non-oncology products. It is important to recognise that the number of products observed in an area, does not directly translate to spend (e.g. spend on one product/indication, might outweigh the spend on multiple products/indications).

- The expenditure control mechanism (ECM), whereby any spend above the fixed £340m budget is paid back on a proportional basis by all companies benefitting from funding from the IMF, will mean that the IMF will never have to close to potential new entrants.

- There are currently no plans to merge the IMF and CDF.

- Please see our response to Question 3 for points related to rare diseases and Question 6 for data issues.

Question 3: Do you agree with the objectives and guiding principles underpinning the IMF? n=109



Analysis of responses

- Overall, there was broad support for the objectives and principles underpinning the IMF with 49% of respondents either strongly agreeing or agreeing with the proposed objectives and principles underpinning the IMF. 38% disagreed or strongly disagreed but where this was the case, concerns tended to focus on a specific principle rather than all of them.

  "[We] strongly agree with the principle that the IMF should offer everyone, regardless of their condition, equal potential opportunity to benefit from promising but uncertain medicines and feel this should be at the core of its aims." *Patient Organisation*

  "The guiding principles are balanced, with an appropriate focus on the clinical aspects e.g., the only reason that the medicine cannot be recommended for use is significant clinical uncertainty. The principles will ensure that there is greater equity between the access for treatment for people with rare diseases compared to cancer." *NHS Organisation*

- Some stakeholders felt that as well as supporting promising but uncertain medicines, the IMF had the potential to support faster access to medicines in general.

  "The Innovative Medicines Fund could provide a potential source of funding to support earlier access to certain medicines that NICE is able to recommend for routine use in the NHS." *Industry Organisation*

- Some industry responses suggested that the concept of eligibility criteria was contradictory to principle 1.

  "[We] would agree with the ABPI that the detail of Principle 2 appears to directly contradict Principle 1 by introducing a restriction in the form of further criteria to be applied beyond just evidential uncertainties to determine which medicines should benefit from the fund." *Pharmaceutical Company*

- A number of responses from patient groups raised the issue of whether rare diseases might be disadvantaged due to the nature of such conditions and through the implications for data collection or commercial agreements.

  "Our disappointment is with the proposals' failure to incentivise new technologies and the lack of focus on rare diseases." *Patient Organisation*

- Respondents also indicated that the proposals could go further in supporting faster patient access to Advanced Therapy Medicinal Products (ATMPs).

  "Currently we are concerned that the scope of IMF and ambition may prevent ATMP companies from being able to benefit from the IMF as was originally intended." *Pharmaceutical Company*

NHS England and NICE Response

- Managed access has already enabled patient access to many promising treatments for rare diseases. Our flexible and inclusive approach, working with companies, patient organisations and clinicians to build the specific requirements of each data collection for a medicine going into managed access ensures that the best data and analyses can be available to NICE when these medicines are evaluated following their period of managed access data collection.

- NICE found, as part of their methods and process review, that there was no evidence that society values health benefits in rare diseases more highly. However, NICE's methods and process review included detailed consideration of rare diseases and made a number of important improvements relevant to rare diseases. These include the introduction of a severity modifier that rare diseases will benefit from, the flexibility to accept greater uncertainty when evidence generation is difficult, as well as an emphasis on a comprehensive evidence base including real world evidence, qualitative evidence, surrogate outcomes and expert elicitation.

- As a result of NHS England's commercial flexibilities, NHS patients are already benefiting from the most promising innovations via investment in cutting-edge and clinically and cost-effective technologies and the IMF will further support this trend,. A recent example of patient impact is autologous CD34+ cells encoding ARSA gene (Libmeldy™), a life-saving gene therapy for metachromatic leukodystrophy (MLD), a rare disease affecting babies.

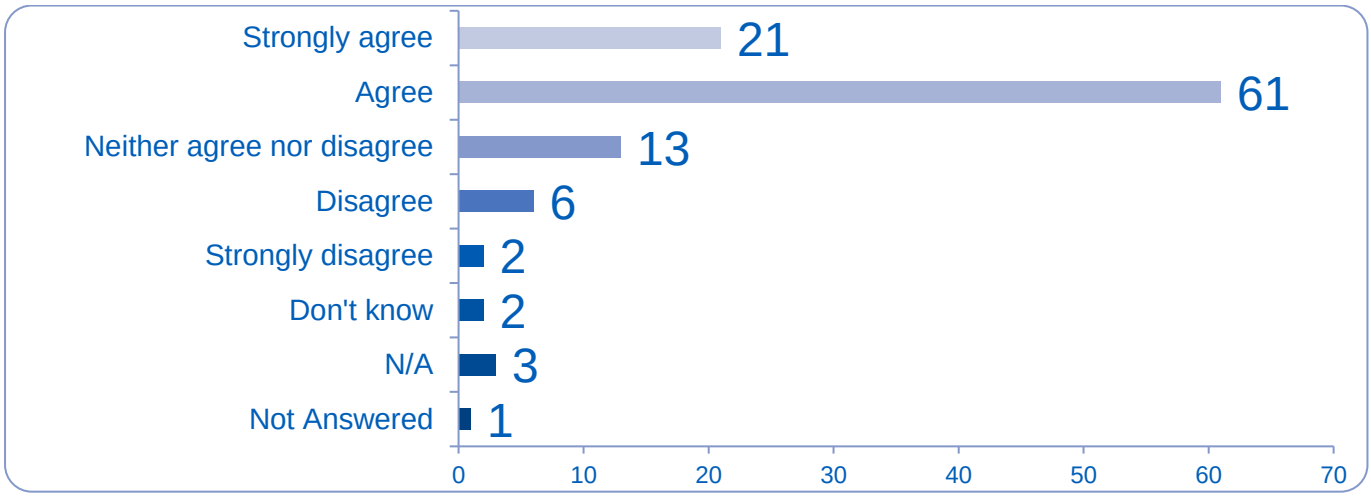
- We have also secured access to a first gene-therapy for spinal muscular atrophy (SMA), onasemnogene abeparvovec. This landmark commercial deal, secured a one-time gene therapy for infants with type 1 SMA and delivers a second SMA treatment for NHS patients within two years; meeting an unmet treatment need where prior to 2019 there were no licensed SMA treatments.

- Furthermore, it is important to note that, to date, NICE has approved the overwhelming majority of ATMPs that it has appraised. With the additional opportunity the IMF offers for resolving evidential uncertainty, the IMF will further support patient access to the most promising and innovative new medicines whilst further data is collected.

- In addition to supporting promising but uncertain medicines, the IMF (like the CDF) also presents an opportunity, through interim funding, to accelerate the introduction of proven medicines where NICE recommends a medicine for routine use in the NHS. The potential for NHS England to agree interim funding arrangements will be limited to medicines that are used within the context of a prescribed specialised service, reflecting NHS England's role as the accountable commissioner for these services.

- Early access (Question 9) and eligibility criteria (Question 5) are responded to under their respective sections below.

Q4. To what extent do you agree with the following key features of the IMF? NICE recommending a medicine in the IMF. n=109



Analysis of responses

- There was widespread support for NICE playing a pivotal role in the IMF with 75% of respondents either agreeing or strongly agreeing with this proposal.
  "NICE has a strong, international reputation for its approach to appraising new medicines. NICE's involvement in the recommendation process for the IMF is not only required but will enhance the decision-making process." *Patient Organisation*

- Some industry respondents suggested a 'light touch' mechanism should be introduced, where a full NICE evaluation is not conducted for a medicine to enter the IMF, in order to speed up access.

  "We believe a new lighter touch NICE process is required for entry into the IMF (when this is the access route that the company has signalled it wishes to utilise)." *Industry Organisation*

- A number of respondents highlighted the importance of the IMF being aligned with accelerated regulatory processes such as the Innovative Licensing and Access Pathway (ILAP), to ensure a cohesive approach for the UK market. Others flagged that they did not want medicines to be restricted based on regulatory criteria (e.g. only ILAP medicines can qualify).

  "A more joined-up methodology is needed to optimise these initiatives and enable faster patient access to the latest innovations." *Pharmaceutical Company*

- Across all stakeholder groups, the importance of full alignment with the NICE health technology evaluation manual (2022) was emphasised – the focus here was ensuring that key recent developments relating to modifiers on severity and uncertainty were implemented for the IMF.

  "The IMF must also be placed in the broader context of the changes being made by NICE to its processes and methods for technology appraisal and highly specialised technology evaluations, published on 31st January 2022." *Industry Organisation*

NHS England and NICE Response

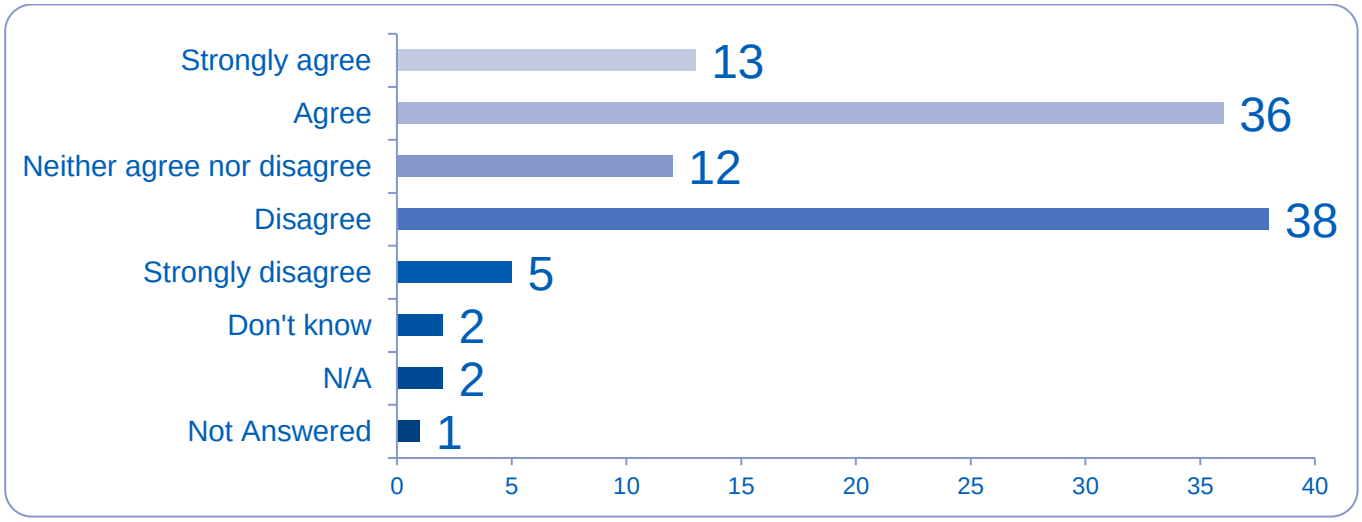
- We are encouraged to see widespread support for NICE playing a central role in the IMF. NICE has an essential role through producing evidence-based guidance, which identifies clinically effective and cost-effective new treatments.

- NICE will consult on any updates to the NICE health technology evaluation manual (2022) concerning early conditional recommendations for entry into managed access.

- We are working with the Medicines and Healthcare products Regulatory Agency (MHRA) and other partners to ensure alignment between the operation of the IMF and regulatory and access initiatives and processes, including ILAP, Project Orbis, the Early Access to Medicines Scheme (EAMS) and any subsequent adaptions to the relevant processes for England. However, eligibility for the IMF will not be defined by regulatory criteria or processes. Please see Question 5 below for further information on eligibility criteria.

- The NICE health technology evaluation manual (2022) and the IMF are fully aligned and should be read alongside the NHS England Commercial Framework for New Medicines to understand the commercial options available to companies bringing new medicines to patients in the NHS.

Q5. To what extent do you agree with the following key features of the IMF? *Criteria for entry into the IMF.* n=108



Analysis of Responses

- There was both support and challenge from respondents to the proposed eligibility criteria (45% strongly agree/agree versus 40% strongly disagree/disagree).

- Stakeholders who disagreed felt that eligibility criteria directly contradicted Principle 1 and that it was unclear how a company would demonstrate their product met the eligibility criteria.

- Stakeholders who agreed with the eligibility criteria felt that they were necessary in order to ensure that IMF resources were targeted appropriately to the most important and impactful treatments for patients.

  "The CDF has worked well without the need for such additional criteria, and we would therefore suggest removing this principle." *Pharmaceutical Company*

  "[We] are in agreement that any medicine recommended for use through the IMF should addresses [sic] a high unmet need; provide significant clinical benefits and represents a step-change in treatment for patients and clinicians with evidential uncertainties can be resolved in a reasonable time." *Pharmaceutical Company*

- Some respondents wanted further clarity on what a prescribed specialised service was and were concerned that the application of this criteria was restrictive.

  "We would like clarity on the stated expectation that interim funding will only be offered to medicines recommended by NICE that are commissioned in the context of a prescribed specialised service." *Patient Organisation*

NHS England and NICE Response

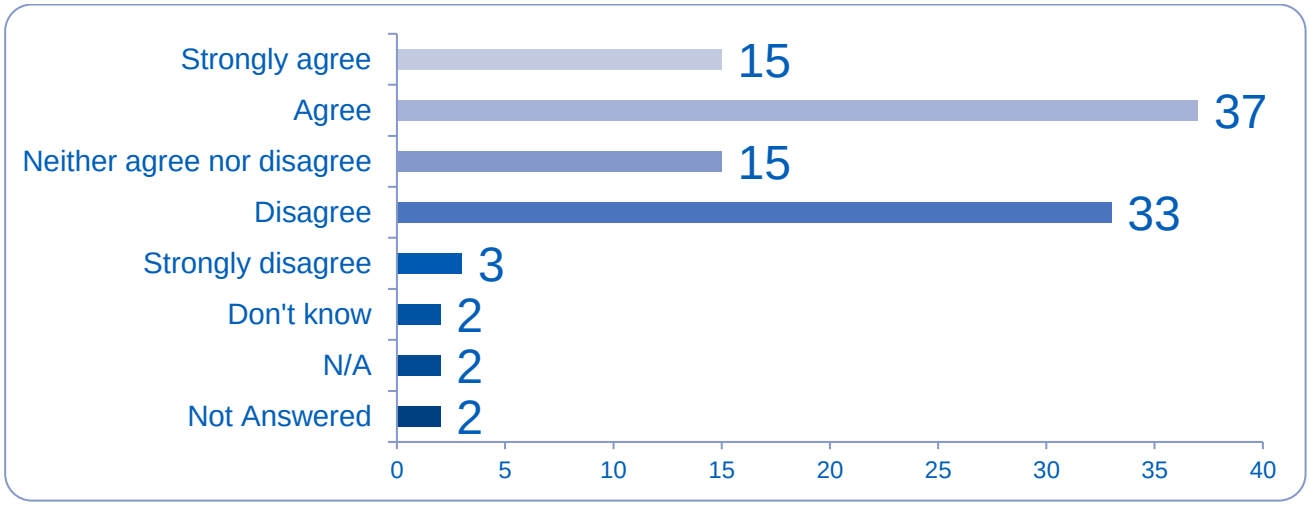
- We recognise the challenging balance between ensuring the IMF is targeted at innovative treatments potentially offering high health gain in areas of unmet need; providing financial sustainability; and not being overly restrictive to potential candidates.

- Principle 2 has been revised to make clear that a range of factors will be considered when assessing the suitability of a medicine for entry into the IMF. These will not be applied as a rigid set of criteria. The core principle remains unchanged - the IMF should target the most promising medicines for which there is significant remaining uncertainty around the level of clinical benefit and cost effectiveness.

- These considerations will help the identification of promising new treatments that might be recommended for managed access by NICE because of their evidential uncertainty. The considerations will help focus resources on the development of commercial and data collection arrangements that are feasible, manageable and deliverable to enable patient access as early as possible.

- The definition of a specialised service can be found by following this link and this has been included in the updated IMF principles document.

Q6. To what extent do you agree with the following key features of the IMF? *Resolving uncertainty through the IMF.* n=107



Analysis of Responses

- 49% of respondents strongly agreed or agreed with the proposals for resolving uncertainty through the IMF with 34% disagreeing or strongly disagreeing.

- Many respondents indicated that 5 years may not be long enough to resolve evidential uncertainty for particular types of medicines such as ATMPs, especially those treating rare diseases. Respondents suggested that:

  – Timeframes to demonstrate longer term cost-effectiveness for rare disease treatments versus cancer drugs is likely to vary considerably.

- The data collection timeframe should be considered on a case-by-case basis. It was stressed that 5-year agreements (or longer) would represent a long-term commitment and the burden on both patients and clinics would be significant.

  "I can imagine 5 years will be sufficient for many new cancer drugs to demonstrate longer term cost-effectiveness for NICE but rare disease therapies frequently are longer term treatments (often life-long), with the evidence for cost-effectiveness needing very young patients (often not included in initial clinical trials) to be treated for a longer period of time (maybe 10-15 years)." *NHS Clinician*

- The importance of the Systemic Anti-Cancer Therapy (SACT) data source for the CDF was highlighted and respondents thought that the IMF would require a similar comprehensive data structure to facilitate the managed access approach outside of oncology.

  "NICE's Appraisal Committees will need to utilise much more diverse non-UK real world evidence datasets than has been the case up to now, as cancer medicines have benefitted from the existence of the SACT database for England." *Pharmaceutical Company*

- Using Real-World Evidence (RWE) to address uncertainty in the IMF was broadly welcomed; respondents flagged that, due to the global nature of clinical development, the IMF may need to utilise international data sources.

  "It is important to use the learnings from the CDF to acknowledge the need to rely upon the maturation of global clinical evidence development programmes for resolving uncertainty." *Industry Group*

- Respondents also wanted more clarity on how costs relating to data collection and analysis would be shared between companies and the NHS – it was suggested that the scoping stage at the start of a NICE evaluation could be the appropriate time to have this discussion.

  "Further clarity is needed on how the costs of any necessary data collection will be fairly established and shared between companies and the NHS. Discussions on this need to happen early on with companies at the initial scoping stages." *Industry Organisation*

- It was widely acknowledged that due to the uncertainty involved with potential treatments made available through the IMF it would be important to have flexibility and apply a pragmatic approach to decision making.

- Industry stakeholders flagged that it may be commercially unviable to treat the entire patient population within the managed access period - in particular, for one-off single treatments such as cell and gene therapies. Companies would like greater flexibilities to allow a phased approach to be adopted, depending on the specific circumstances.

  "Flexibility may be needed to allow a phased approach depending on the individual circumstances of the medicine and the disease area, and an understanding of the commercial and service impact of treating the population." *Industry Organisation*

- Early and widespread stakeholder engagement was encouraged. It was highlighted that stakeholders such as patients, patient groups and clinicians could play a pivotal role in designing and collecting the data needed to resolve the evidential uncertainty.

  "We welcome the commitment to involve stakeholders in the process of developing the data collection agreement (DCA). We urge that there is clear guidance to stakeholders as to what the opportunity is to contribute, and how the process will work." *Patient Organisation*

NHS England and NICE Response

- The gold standard of evidence for a NICE evaluation is most likely to come from clinical trials that were established prior to a recommendation with managed access. Therefore, the additional 5-year data collection period should be sufficient to deliver more mature data for the evaluation and to resolve the evidential uncertainty.

- The 5-year timeline in the IMF is the maximum duration of the data collection period for managed access. It has been established as a proportionate period to allow evidence from ongoing clinical trials and real world data collection to mature whilst the NHS pays for an uncertain treatment.

- Patients, their families and NHS clinicians involved in ongoing data collections stress the additional impact of the clinical monitoring required during a period of managed access and their experience highlights why this process must be completed in the shortest time possible. To date the maximum of 5 years has enabled patients to access promising new treatments, including ATMPs. Further reasons to include a maximum time period are:

  – The reasonableness of collecting data and applying a managed access approach for a product if the uncertainty cannot be reduced within a 5 year period.

  – To account for changing treatment pathways; as new treatments become available and with the potential for one treatment to displace another within the data collection period.

  – Additional burden placed on patients, their families and clinicians who are required to attend clinic more frequently, for a wider range of clinical assessments for example, throughout the data collection period – we want to minimise this burden for patients, their carers and the NHS.

  – Financial risk to the NHS of funding treatments that have not yet proven to be cost-effective. It should be noted that placing a product in managed access for most of its branded patent life, post-launch, is unlikely to be desirable for the NHS, taxpayers or the company.

- We, via the Accelerated Access Collaborative's data infrastructure workstream, have outlined a framework for data collection and analysis, building on existing NHS non-cancer data infrastructure and emphasising the importance of early engagement with companies about potential data collection needs for managed access.

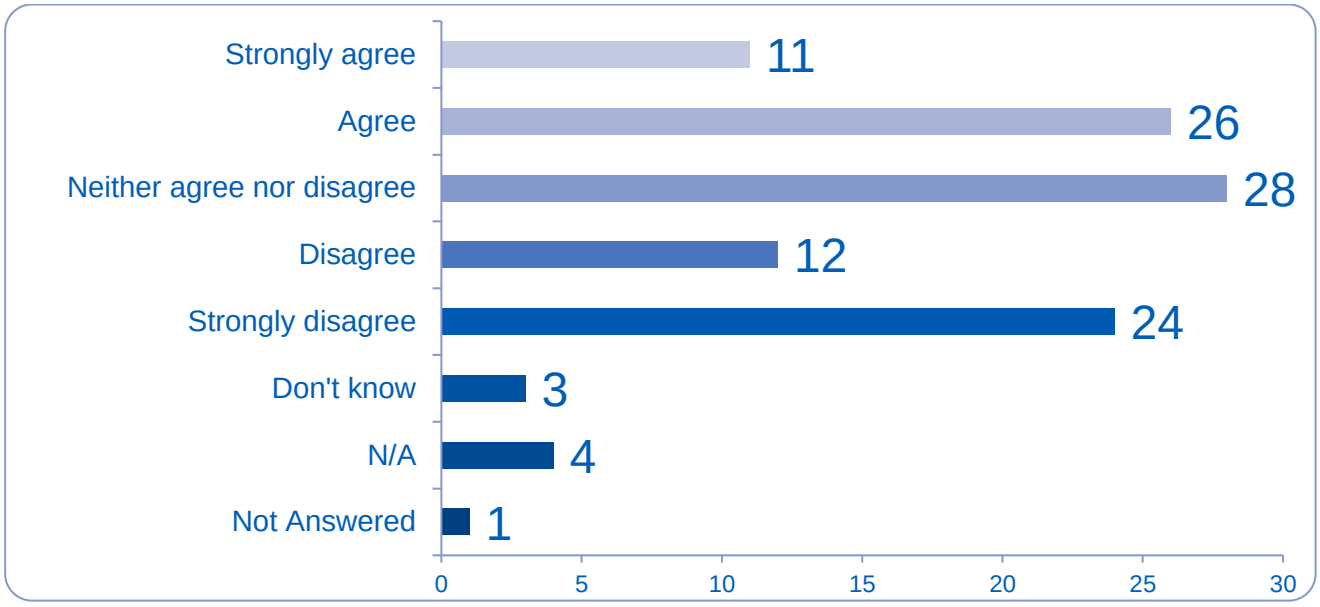
- NICE's health technology evaluation manual (2022) also outlines opportunities for companies to engage with NICE and NHS England about their managed access proposals. Additionally the NICE real world evidence framework outlines how NICE will approach the use of real world data to inform health technology assessment.

- While NICE has a general preference for data relating directly to the UK population that reflects current care in the NHS, the potential value of international data is recognised where limited information is available. Both NHS England and NICE anticipate that international data sources could be utilised in data collection agreements in the future, where they would sufficiently resolve the evidential uncertainties. A number of existing managed access agreements already reference international data collections that will be used in their NICE guidance update.

- NICE is developing further information for companies and other stakeholders on data collection plans, including greater detail about the costs that companies may need to cover to facilitate real-world data collection. This information will be available on the NICE website in the summer of 2022.

- Further detail has been provided in the IMF principles document on the approach to data collection and analysis to resolve evidential uncertainty and the importance of early engagement with NICE so we can broker relationship with NHS data custodians, clinicians and patient organisations who will likely be involved in the data collection.

Q7. To what extent do you agree with the following key features of the IMF? *Commercial Access Agreements (CAA).* n=107



Analysis of Responses

- There was a broad range of opinions received on the proposals for CAAs; approximately a quarter of responses (26%) neither agreed nor disagreed with the proposals, highlighting that this feature was of greatest interest to industry respondents.

- Generally, industry respondents felt that the offer for managed access should be based at the mid-point of the plausible ICER range. This would share the risk equally between companies and the NHS. Clarity was also sought around how 'plausibly' cost-effective would be defined.

  "[We] strongly believe that commercial access agreements should be put in place which as a starting point generally reflect the mid-point of the plausible ICER range, as determined by NICE." *Industry Organisation*

- Clarification was also requested on how the highly specialised technology (HST) thresholds (and QALY weightings) would apply given the recent NICE Methods Review.

  "The proposals also need to clarify that the HST thresholds, taking into account any QALY weightings applicable at the time, apply for medicines being evaluated through the HST Programme and coming into the IMF." *Pharmaceutical Company*

- It was suggested that the wording of principle 3 should be altered to remove reference to 'responsibly priced'.

  "We disagree with part (b) of the principle which should be removed. As stated earlier, NICE appraisal processes generate quantified outcomes by applying formal published methodologies to identify plausible cost-effective prices, taking into account levels of uncertainty. This is an objective approach and therefore we find the use of the subjective

term "responsible pricing" in the IMF proposals to be incompatible with this and unnecessarily emotive." *Pharmaceutical Company*

- One pharmaceutical company suggested that principle 3 would present a barrier to entry into the IMF for medicines treating rare diseases, such as ATMPs.

  "[We have] concerns that meeting existing cost-effective thresholds will be very challenging for rare and ultra-rare conditions going into the IMF." *Pharmaceutical Company*

- There was also a request to use the IMF as a platform for more innovative reimbursement approaches and payment mechanisms (e.g. outcomes-based payments and multi-year staged payments).

  "Outcomes-based payment models with multi-year staged payment approaches (already being deployed in some European countries) provide new ways to address affordability and uncertainty challenges." *Industry Organisation*

NHS England and NICE Response

- NHS England's Commercial Framework for New Medicines states that enhanced commercial arrangements would normally be reserved for medicines expected to have value propositions at or below the lower end of the standard NICE cost-effectiveness threshold range, with greater flexibilities made available for value propositions at even greater levels of cost-effectiveness, taking into account any applicable QALY weightings.

- It is therefore entirely appropriate that, for the IMF, the level of reimbursement is reflective of the decision uncertainty; to be consistent with the Commercial Framework, companies will need to present an offer that brings the range of potentially plausible cost-effectiveness estimates, as determined by NICE, to below the relevant cost effectiveness threshold, taking account of any applicable QALY weightings.

- Medicines that are evaluated through the HST route at NICE will be eligible for the IMF via the relevant thresholds and this has been clarified in the IMF principles document.

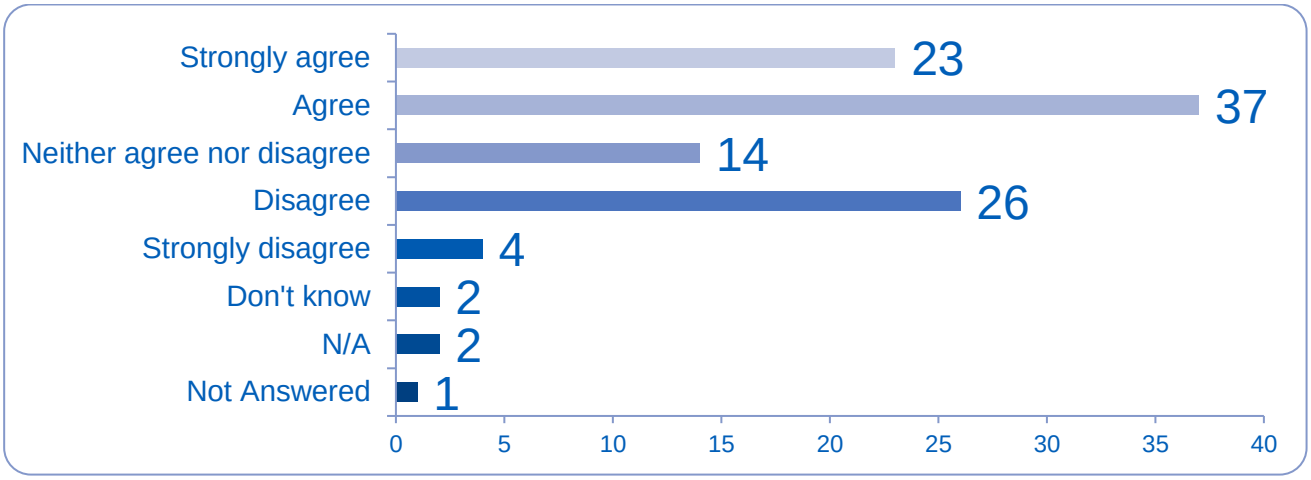
- NICE plays a critical role in ensuring equitable patient access to new medicines and treatments by evaluating the evidence concerning clinical- and cost-effectiveness. Medicines recommended with managed access in the IMF will be evaluated following the NICE health technology evaluation manual (2022), following the processes and methods set out for either a technology appraisal or a HST evaluation.

- The IMF principles document has been updated to provide further detail on the roles and interplay of the Fund with the NICE health technology evaluation manual (2022) and specifically how the QALY weightings, via severity modifiers, will apply.

- The wording in principle 3 around responsible pricing will be retained and is entirely consistent with the Commercial Framework for New Medicines. It is important to signal to all stakeholders that value is being delivered on medicines where there is uncertain benefit.

- The Commercial Framework already allows companies to propose complex commercial arrangements, such as outcomes-based arrangements and we are always able to consider these. Due to the administrative burden of transacting and monitoring these types of arrangements, the NHS has a strong preference for simple patient access schemes. The onus is on companies to demonstrate the necessity for these schemes and justify the additional value that it will provide to the NHS compared to the burden they place on the NHS and the resultant uncertainty for patients.

Q8. To what extent do you agree with the following key features of the IMF? *Updating NICE guidance following a period of managed access and exiting the IMF.* n=108



Key Themes

- 56% of stakeholders agreed or strongly agreed with the approach proposed for updating NICE guidance following a period of managed access and exiting the IMF.

  "We support the proposal that all treatments funded through the IMF should have a NICE appraisal following their period of managed access. This should provide an exit route from the IMF for all those treatments. We welcome the commitment that patients who commenced treatment during the period of managed access will continue to be able to receive medicines until no longer clinically indicated." *Patient Organisation*

- Some industry stakeholders felt that the budget impact test (BIT) should not be applied on exit from the Fund because the BIT is designed to cover the first three years after a product launches.

  "We do not agree that medicines should be subject to a budget impact test after re-evaluation given how long the medicine may have already been on the market" *Pharmaceutical Company*

- Industry respondents disagreed with the proposal that companies should fund ongoing treatment after exit from the IMF if the medicine was not recommended by NICE. Respondents felt that this should be a cost shared between the NHS and the company.

  "[We are] concerned about the implications of treatments leaving the IMF that are not approved for routine commissioning and the financial burden that may be placed on small companies with a limited income stream, particularly in the case of chronic diseases." *International Collaborative*

- Respondents supported NICE guidance updates following a period of managed access, as it offered an opportunity for the price the NHS pays for the drug to increase or decrease.

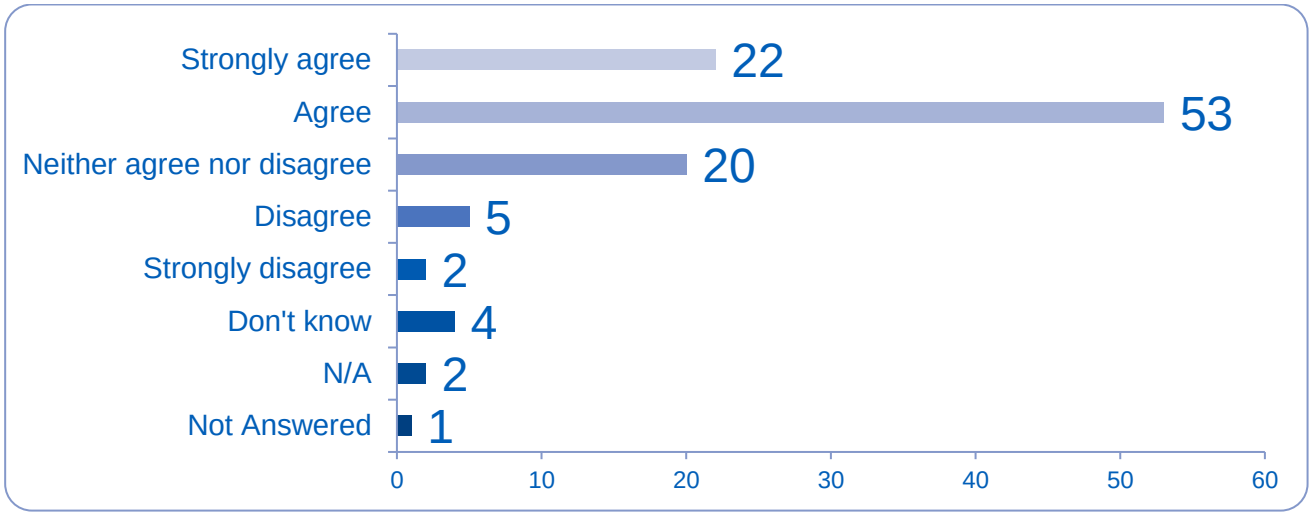
"We welcome acknowledgement that the appraisal by NICE could lead to either a price increase or decrease on exit of the IMF to reflect the value of the medicine once uncertainty has been resolved." *Pharmaceutical Company*

NHS England and NICE Response

- We have a responsibility to ensure that any routinely commissioned drugs do not present an affordability challenge for the wider NHS. We will therefore continue to apply the budget impact test to products that are entering routine commissioning – which includes those exiting the IMF. This is consistent with the policy applied in the CDF.

- Any patient who has been prescribed the medicine during the managed access period, has a right to continue to receive treatment until they and their treating clinician deems it appropriate to discontinue treatment and/or they meet a treatment stopping criteria (in line with NHS treatment continuation policies or company-sponsored free of charge schemes). The NHS does not fund treatments that are not recommended by NICE. Companies will have the opportunity to re-negotiate their commercial arrangement with NHS England at the end of the manged access period to ensure it is both clinically and cost effective. Companies who are not able to offer a cost-effective price will be required to honour their commitment to patients who start treatment during the managed access period, and will have a choice of whether or not to accept the terms of entry into the IMF.

Q9. To what extent do you agree with the following key features of the IMF? *Interim Funding for NICE recommended medicines.* n=108



Key Themes

- The majority of responders (69%) supported the proposed interim funding approach and industry stakeholders specifically suggested that the interim funding period should be expanded to give funding from the point of marketing authorisation, regardless of whether NICE had made a recommendation at that point. It was suggested that:-
  - This would align with accelerated regulatory initiatives (e.g. ILAP and Project Orbis).
  - The French ATU process could be a potential model for the UK to consider.

  "There is an opportunity for the IMF to test more innovative approaches to funding so that patients could benefit even earlier. For example, interim funding could be provided from the point of marketing authorisation up until the end of the 90-day guidance implementation period (30 days for EAMS medicines) for certain categories of medicines such as those with an EAMS or ILAP designation." *Industry Group*

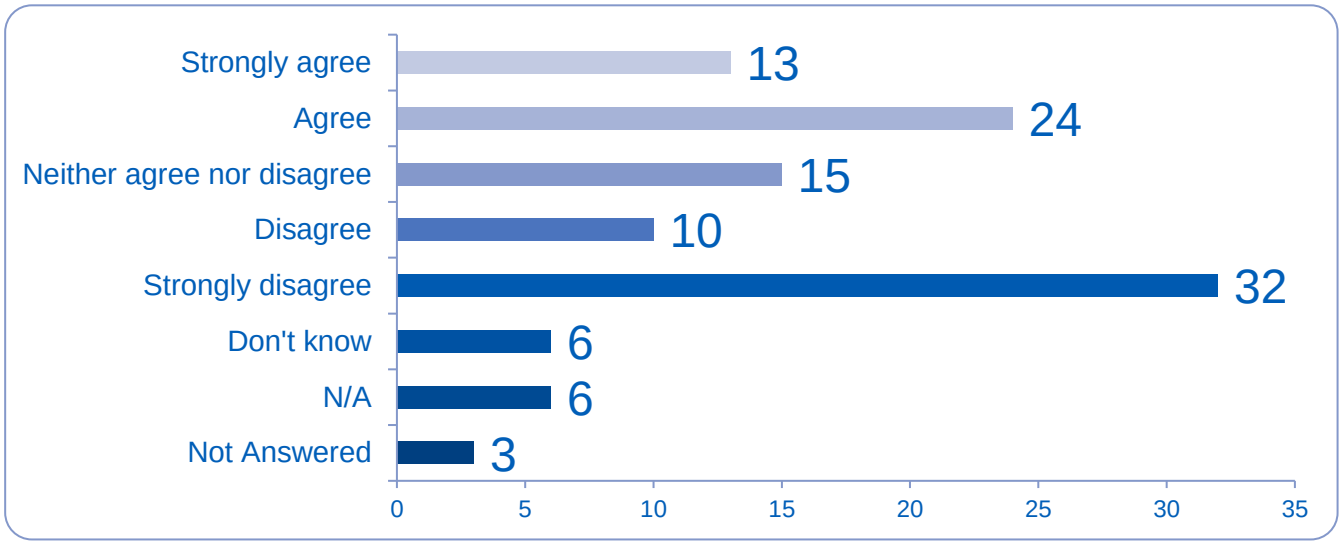
- Paradoxically, many stakeholders were also concerned that the IMF budget could be exhausted rapidly and that expanding interim funding would exacerbate this.

NHS England and NICE Response

- The existing EAMS aims to give patients, with life threatening or seriously debilitating conditions, access to medicines that do not yet have a marketing authorisation when there is a clear unmet medical need. There is, therefore, an option for early access and it is not within the scope of the IMF to bring forward funding pre-marketing authorisation and before NICE have recommended a medicine for managed access.

- See response to question 3 for further detail on interim funding.

Q10. To what extent do you agree with the following key features of the IMF? *Financial control.* n=106



Key Themes

- The financial control mechanism divided respondents with 35% agreeing or strongly agreeing with the proposed approach and 40% either disagreeing or strongly disagreeing.

- It was argued that the expenditure control mechanism (ECM) was not necessary and the 2019 Voluntary Scheme for Branded Medicines Pricing and Access was already in place to cap overspend.
  - It was stressed that the application of the ECM would limit companies' willingness to consider managed access in the IMF.
  - It was argued that the ECM might disadvantage smaller companies and that companies had no control over IMF expenditure in aggregate.
  - It was highlighted that the CDF has never overspent, and the SMC ultra-orphan pathway does not have a spending cap.

  "The commercial risk imposed by the ECM is likely to be too great for many companies to bear – particularly smaller and medium sized companies, many of which are leading the way in development of rare disease medicines and ATMPs." *Pharmaceutical Company*

  "I liked the feature about industry carrying some of the risk." *NHS Clinician*

- It was proposed that the IMF (and CDF) budget could be set on a 2-year basis, utilising enhanced horizon scanning information and measured against KPIs to track the functionality of the fund size, budget and impact.

"Rolling reviews of the funds size, budget and impact would enable the process to adapt to meet the needs of patients and the healthcare system." *Patient Organisation Coalition*

NHS England and NICE Response

- The ECM is a fundamental component of the IMF, which will ensure that the fund remains open to new entrants and guarantees that the fund remains financially sustainable.

- The NHS utilises horizon scanning to ensure that the NHS is aware of forthcoming technologies and to ensure any necessary preparatory work is completed in advance of NICE evaluation. Due to the dynamic nature of pharmaceutical development and uncertainty on key variables such as price, uptake and the eligible population, we do not consider it viable to set either the IMF, or CDF, budget based on these information streams.

Additional Revisions

We are grateful for the requests received, as part of the consultation, to include a number of clarifications, corrections and revisions. These have been incorporated into the IMF principles document.

Appendix 1: Glossary

BIT: Budget Impact Test	**HTA:** Health Technology Assessment
CAA: Commercial Access Agreement	**Interim funding:** IMF funding for a NICE recommended medicine can start at the point NICE issues a draft positive final guidance.
CCG: Clinical Commissioning Group	**MAA:** Managed Access Agreement
CDF: Cancer Drugs Fund	**Managed access period:** The duration of a managed access agreement, including both the data collection period and the time required for the NICE guidance update.
DCA: Data Collection Agreement	**MTA:** Multiple Technology Appraisal
Data collection period: The time specified in the data collection agreement that covers the period of data collection and time for analytical outputs to be developed.	**NICE:** National Institute for Health and Care Excellence
EAMS: Early Access to Medicines Scheme	**PAS:** Patient Access Scheme
ECM: Expenditure Control Mechanism	**QALY:** Quality-Adjusted Life Year
HST: Highly Specialised Technologies	**STA:** Single Technology Appraisal

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