Background and purpose and introduction	
Do you agree with purpose of the IMF?	**Strongly Agree** The BIA strongly supports the purpose of the Innovative Medicines Fund (IMF) to improve patient access to promising new innovative medicines while additional data is collected to resolve clinical uncertainty. We believe that the IMF could be an important vehicle for enabling greater access to the growing pipeline of innovative medicines for rare and ultra-rare diseases and want to ensure that the fund is accessible to these medicines. It is encouraging that the consultation engagement document (paragraph 5) recognises that the IMF could be particularly beneficial for innovative medicines for rare conditions which would otherwise receive negative NICE guidance due to limited data and significant clinical uncertainty. However, as they stand, the proposals fail to resolve the challenges faced by rare disease medicines associated with resolving uncertainty and demonstrating plausible cost-effectiveness. As such, the IMF is unlikely to significantly improve patient access to innovative medicines for rare diseases. An NHSE announcement on the IMF in July 2021 made specific mention of "early access to potentially life-saving new medicines, including cutting-edge gene therapies". However, the proposals do not reference gene and cell therapies or advanced therapy medicinal products (ATMPs) which are key, promising treatments that can improve outcomes and transform the lives of patients and families in the rare disease community. Over the last decade, the UK has played a major role in the research, development, and adoption of ATMPs. Through the Cell and Gene Therapy Catapult's Advanced Therapy Treatment Centre (ATTC) network, the NHS is a central partner in accelerating the adoption of ATMPs. It is vital that the IMF operates as a seamless part of the jigsaw in this area by building on the capability and infrastructure already in in place. There is a need for greater clarity on how the IMF will align with initiatives to support treatments for rare diseases, such as the ambition set out in priority four of the UK Rare Diseases Framework (RDF), which concerns "improving access to specialist care, treatments and drugs". With the conclusion of the NICE Methods and Process Review, greater clarity is also required on how the IMF will align with the changes to NICE's manual to support medicines for rare diseases in Health Technology Assessments (HTAs). The BIA would ultimately like NICE to be able to routinely recommend medicines for rare conditions without needing to use Managed Access Agreements (MAAs) and the associated requirement to reappraise therapies. Nevertheless, we recognise the valuable role that MAAs can play, and have already played, in providing accelerated patient access

	to rare disease medicines, in the absence of suitable provisions within NICE's HTA methods and processes for rare disease medicines. NHSE has many years of experience in providing MAAs for non-oncology medicines and we disagree that the IMF is required to fund this well-established approach. The BIA believes that limiting the IMF to funding a well-established NHSE approach will severely limit the potential of the IMF. We recommend that rather than just facilitating MAAs, the IMF also be used to test and pilot innovative mechanisms for reimbursement (such as pay-by-performance) for treatments where significant uncertainty exists. The BIA recently published a report which explores the merits of introducing an innovative payment model that balances risk and benefits between the NHS and industry. With a growing pipeline of ATMPs and other innovative medicines for rare diseases, it is crucial that the UK develops a more sustainable long-term approach to funding these treatments in a way that minimises the financial risk to healthcare providers. The IMF presents the perfect opportunity to test these alternative approaches. Over the past two years, the BIA has engaged with NICE throughout the Methods and Process Review to support NICE in addressing the specific challenges faced by rare disease medicines in the HTA process. Developing an evaluation system that routinely works for rare and ultra-rare disease medicines – without the need for MAAs – is crucial if the UK is to meet the aspiration set out in the Life Sciences Vision for the UK to be the world leader for development, testing, access, and uptake of new innovative treatments and technologies. The BIA welcomes changes to enable Committees to accept a higher degree of uncertainty where evidence is difficult to obtain, as well as those to allow greater consideration of real-world evidence. However, the BIA believes that, when taken together, the changes will have minimal impact on the prospects of rare diseases that are routed to the Single Technology Appraisal (STA) programme – a programme that is entirely unsuitable for evaluating rare disease medicines. As a pathway that was created for medicines for more common diseases, it is extremely challenging for rare disease medicines that are routed to this programme to meet the required cost-effectiveness threshold of £30k/QALY. Contrary to NICE's assertion that steps (including the introduction of a severity modifier) have been taken to address the needs of rare disease technologies in the STA programme, the proposed changes will have minimal effect on the prospects of orphan and ultra-orphan medicines securing positive reimbursement decisions via the STA programme. These challenges are likely to be exacerbated by NICE's changes to the entry criteria for its Highly Specialised Technology (HST) programme, which is designed to evaluate treatments for very rare diseases. The refined criteria will reduce the number of medicines eligible for HST by up to a half, thereby increasing the number of orphan and ultra-orphan

	medicines that will be routed to the (STA) programme. With these changes, the IMF will be vital for enabling rare and ultra-rare disease medicines to resolve the clinical uncertainty that would otherwise result in negative recommendation in the STA programme. In light of these changes to NICE's programme manual, the IMF could provide an important vehicle for bridging the gap between marketing authorisation and positive reimbursement for rare disease medicines that are routed to STA. The BIA is keen to work with NHSE&I and NICE to ensure that the IMF is accessible and suitable for these medicines. The Life Sciences Vision recognises that as the UK seeks to ensure it has the industrial capacity needed to respond to future pandemics and disease, it is critical that this is met with a focus on improving the speed and scale with which new medicines and technologies are utilised in the UK over the coming years. By developing a system through which the NHS can seamlessly pay for and adopt the innovative products being created by the life sciences industry, this ambition can be realised. As a key 'innovation partner' in the delivery of the Life Sciences Vision, the NHS has the opportunity, through the deployment of the IMF, to implement the change required to bolster the UK's medicinal capacity. At the same time, by positioning the UK as an attractive destination for innovative companies, the IMF can play a pivotal role in elevating the UK's status as a life science superpower. *Other comments* No governance details are provided in the proposals. A vision for anticipated governance of IMF needs to be defined and communicated to all stakeholders. All stakeholder groups (including industry, patient and clinician representatives) should be represented in governance structure/processes, not just NHSE&I and NICE. The BIA requests that the IMF be reviewed after a recommended time of one year. This would allow all stakeholders to provide feedback on the process and for changes to be made that would better align the process with the demands placed on it. The BIA also requests that NHSE&I and NICE collect and publish appropriate metrics on the impact of the IMF in improving access to innovative medicines. An NHSE announcement on the IMF in July 2021 noted that the IMF will "significantly reduce the time it takes for most promising medicines to reach patients". As such, it is important that metrics are gathered to demonstrate whether the IMF reduces the time taken for patients to access treatments, as well as metrics on IMF eligibility and outcome. These metrics must specifically identify the impact of the IMF on the treatment areas that NHSE has explicitly stated are intended to benefit from the IMF, including medicines for rare diseases and gene therapies.

Do you agree that the Innovative Medicines Fund should operate alongside, and on similar terms to the Cancer Drugs Fund?	**Strongly Disagree** The BIA recognises that the Cancer Drugs Fund (CDF) has helped to bring innovative but unproven medicines to thousands of cancer patients. We hope that the IMF will similarly help to improve patient access to innovative non-cancer medicines. While certain aspects of the CDF are applicable to the IMF, it is necessary to consider the terms of that scheme as they pertain to the IMF. It is unclear how the proposed funding envelope of £340m per annum has been determined, especially given the high volume of potential non-oncology innovative medicines that are likely to become available in coming months and years. Principle 1 states that the IMF is designed to provide equal potential opportunity for non-oncology patients to benefit from promising medicines. It is difficult to see how this will be achieved by a fund of equal size to the CDF, given the broad range of disease areas for which innovative medicines are being developed. It is likely that with the number of ATMPs and other non-oncology treatments which may require managed access agreements, the £340m will not be sufficient. For example, a very small number of products which include very large indications (like innovative medicines to treat Asthma, Lupus or CVD) could use up the fund very quickly. Following the changes to NICE's entry criteria for the HST programme which are likely to result in an increase in treatments for rare and ultra-rare diseases that will be routed to the STA programme, additional pressures will be placed on the IMF which will be an important vehicle for addressing the challenges these treatments will face. The BIA is disappointed by NICE's recommendation to introduce an expenditure control mechanism (ECM). This would result in a double rebate being required for some companies if this was required in addition to VPAS rebates. Given that the £340m is likely to be insufficient, and that the ECM would require companies in the scheme to repay any spend above this, the IMF will present significant risk to many smaller companies that develop innovative medicines. We are concerned that the uncertainty regarding future costs could act as a disincentive to these companies to develop innovative treatments and that this could inadvertently disadvantage certain disease areas, including rare diseases. The BIA requests that NHSE&I reconsider the proposed sum of £340m per annum and introduces a regular review of the IMF budget informed by horizon scanning activities. The BIA is also very concerned by the suggestion made during a NHSE&I/NICE webinar on the IMF on 13 January that following the IMF's launch, the only way for non-oncology medicines to access an MAA will be via the IMF. Given the concerns we convey in this consultation response that the IMF will not work for many ATMPs and rare disease treatments as was intended, this decision will not have the effect of

	providing greater equity of access between oncology and non-oncology. Significant emphasis has been placed by system partners on the opportunity that the IMF presents for innovative medicines for rare diseases – where there is often significant clinical uncertainty. However, we believe that the financial risk that a widely accessible fund of this size poses to (particularly smaller) companies, coupled with the proposed entry criteria, is in fact likely to restrict patient access to innovative medicines for rare diseases. This is especially the case if MAAs for non-oncology medicines are made inaccessible outside the IMF. Not only would this significantly impact patients in the short term by delaying access to the treatments that they need, but it could also risk companies choosing to launch these technologies in the UK later in their lifecycle, or not at all. The BIA requests further clarification on whether MAAs will be available outside the IMF and CDF.

Guiding principles for the Innovative Medicines Fund	
Do you agree with the objectives and guiding principles underpinning the IMF?	**Disagree** Principle 1 – equity of access for patients regardless of their condition is a sound principle but the current proposals will not lead to equity for patients with rare diseases. Principle 2 – we agree with the IMF intention to target the most promising medicines for which there is significant remaining uncertainty around the level of clinical benefit, however the current proposals would not allow for all types of uncertainty to be resolved. We also question how NHSE&I will establish whether a medicine is considered 'promising' given that measuring potential benefit is difficult where there is uncertainty. Principle 3 – we would like clarity on the basis on which 'plausible potential to be cost-effective' will be determined. This principle does not align with Principle 2, since it is challenging to show cost-effectiveness when there is significant uncertainty about the level of clinical benefit. The BIA recommends that, given likely uncertainty in assumptions, in order to be defined as plausibly cost-effective, only clinical plausible scenarios should be considered, and it should not be the case that all incremental cost-effectiveness ratios (ICERs) from all scenarios be required to sit below the relevant threshold. This would more fairly recognise the potential value of the new medicines for both patients and the NHS. Since 'responsible pricing' is understood as plausible cost-effectiveness, we also suggest removing reference of this

	from the proposals. We believe that cost-effectiveness should be sufficient to establish the price. Principle 4 – the five-year limit associated with resolving uncertainty is too restrictive and will disadvantage certain medicines and diseases. It also contradicts Principle 1 since restricting entry to medicines where uncertainty can be resolved in five years will not lead to equity of access. While five years will be long enough for some medicines to revolve uncertainty, for other medicines, like ATMPs, five years is unlikely to be long enough. Ultimately, people suffering from these diseases will be disadvantaged. Principle 5 – we support the principle that the entire eligible patient population should have the opportunity to access medicines recommended for the IMF in the managed access period. We believe that the IMF should support access to all eligible patients as determined by the licensed indications for a therapy and not limited by NICE to subgroups. The decision about the eligible population should be made jointly with the submitting company. Principle 6 – we are pleased to see recognition of the potential for price adjustments upwards, based on value and benefit to patients. We also request that NICE offers all companies the opportunity of a 'light touch' appraisal on entry to the IMF. The requirement for a two full HTA appraisals on entry and exit is not commensurate with the CDF and so is not in keeping with the principle to provide equity of access for patients regardless of their condition. Principle 7 – we strongly disagree with the requirement for industry to shoulder all the financial risk of a potential negative recommendation and having to fund lifelong treatments for all patients in perpetuity, which could be up to 70 years. Not only does this place too much risk on commercial organisations but it is a logistically unviable requirement. This proposal would deter developers of lifelong treatments from considering or accepting a recommendation through the IMF. Principle 8 – we are extremely disappointed by the current proposals for an ECM as they place an undue amount of financial risk on companies. The ECM could result in companies having to make repayments for both the VPAS rebate and the IMF ECM.

To what extent do you agree with the following key features of the Innovative Medicines Fund	
NICE recommending a medicine in the IMF?	**Disagree** The BIA agrees that it should be the role of NICE, rather than NHSE, to recommend a medicine in the IMF.

	While the exit process is reasonably clear – medicines must go through an HTA with NICE at the end of an MAA to receive a recommendation on routine commissioning – the entry process is less clear. It is implied but not specified that there will be a requirement for a full HTA before entry into the IMF, and we request clarity on whether this is the case. If a full HTA is required before entry and exit to the IMF this will be costly for smaller companies, particularly given the increased cost for technology appraisals, and is likely to act as another disincentive for developers of innovative medicines. In addition, we are concerned that the requirement for two full HTAs will add more time to the overall process thereby delaying patient access to safe treatments that are, in some cases, urgently needed. The BIA recommends that all candidates may (at the request of the company) be given the option of a 'light touch' appraisal (focused on clinical effectiveness) and direct entry into the IMF, rather than via a full HTA process. It is encouraging to see the emphasis throughout the proposals on the importance of early engagement with NICE and NHSE&I and that the Innovative Licensing and Access Pathway (ILAP) and the Early Access to Medicines Scheme (EAMS) are referenced as important in the identification of treatments which are potential candidates for the IMF. The existence of these accelerated pathways for regulatory approval provides the opportunity for the IMF to streamline the process from marketing authorisation and the HTA. It is important that opportunities are taken to create a joined-up approach for the whole access pathway to support early patient access and position the UK as a first launch country for innovative and transformative treatments. It is unfortunate that the proposals do not provide further clarification on how IMF entry links with ILAP. For example, the proposals fail to set out how medicines with an Innovation Passport could be assessed for the IMF. The BIA proposes that products with an Innovation Passport be given accelerated entry to the IMF. Having received an Innovation Passport, these products will already have already met the criteria for innovation required by the MHRA and NICE and should be able to access the IMF without delay at the point of marketing authorisation. This will reduce the time patients have to wait to access medicines once they receive marketing authorisation and will have the additional benefit of incentivising companies to engage with ILAP. We are also aware that NICE has several other process changes and developments, which have been promised as part of the Methods and Processes Review, but which have not been progressed. We request further detail on the 'straight-to-managed-access' route, as well as the expedited route as a matter of urgency. It is important that we understand how the IMF will relate to these changes in order to feed into the consultation.

Criteria for entry into the IMF?	**Disagree** The BIA supports the principle that clear and robust criteria are needed to ensure that the IMF targets the most promising medicines for which there is significant remaining uncertainty around the level of clinical benefit and that any medicine may be recommended for the fund if it meets the entry requirements. IMF qualification should be based on criteria as originally envisaged for the IMF, not to 'level the playing field' but rather to enable more rapid patient access to innovative medicines in specific arenas e.g., autoimmune, and rare diseases. The BIA is concerned that the proposed entry criteria for the IMF could prevent innovative medicines for rare and ultra-rare diseases, including ATMPs, from accessing the fund. The criterion that the technology must demonstrate the potential to be plausibly cost-effective at the price being offered will create a significant barrier for rare disease medicines, particularly for ATMPs such as cell and gene therapies. The criterion for the new evidence to be generated and 'considered meaningful', whilst being able to 'sufficiently reduce uncertainty' will also create a barrier for rare disease medicines. Generating robust data is particularly difficult due to small patient populations and causes challenges in conducting randomised controlled trials with appropriate comparators. Without wider change in NICE assessment around the handling of uncertainty and data for small patient populations, the current IMF entry criteria will continue to represent a significant challenge for rare and ultra-rare medicines. This criterion must also be seen in the context of the proposed maximum five-year period of data collection which may not allow for enough robust data to be collected to address uncertainties, especially for many ATMPs. Whilst it might be viable for some ATMPs to resolve uncertainty within this time frame, for those that have long-term benefit realisation – notably one-time gene therapy treatments – this will not be possible. If the IMF is to be accessible to all innovative medicines outside oncology, including cell and gene therapies, NHSE&I and NICE must work with industry and avoid implementing a 'one size fits all' approach. The BIA is extremely concerned by comments made during the NHSE&I/NICE webinar on the IMF on 13 January that the fund may not be suitable for any medicines that would require longer than five years to resolve clinical uncertainty. This would severely restrict many ATMPs and rare disease medicines from accessing the fund, and risk undermining Principle 1 of the IMF by providing inequitable access to treatments for patients with certain conditions, including many rare and life-limiting diseases.

	If MAAs are deemed to be unsuitable for many ATMPs then the IMF should be able to provide access to these innovative treatments through other mechanisms, such as alternative reimbursement mechanisms like pay-by-performance.
Resolving uncertainty through the IMF?	**Disagree** It is disappointing that the opportunity has not been taken for the IMF to explore mechanisms other than MAAs through which to resolve clinical uncertainty for innovative medicines. The BIA understands that from conception the IMF has been modelled on the CDF and has therefore retained the approach of using MAAs. It is important that there is recognition that MAAs in the IMF will not be able to fully resolve uncertainties that arise from structural issues such as small patient populations and the need for lifelong treatment. In those instances, NICE Committees need to deal with this type of uncertainty appropriately by adopting a pragmatic/less risk averse approach to managing inherent uncertainty. MAAs with a maximum data collection period of five years will not be long enough for robust data to be collected to address uncertainties for many innovative treatments where there are significant challenges in terms of long-term data. The BIA proposes that in addition to operating MAAs, the IMF also be used to pilot and trial other innovative reimbursement mechanisms, including those that would play out beyond five years. This would be particularly beneficial for cell and gene therapies, for which long-term clinical uncertainty will make the currently proposed IMF inaccessible. There are a series of flexible commercial options that can be utilised in a Commercial Access Agreement (CAA) as needed and as appropriate for the medicine in question. Such mechanisms could have the benefit of enabling patient access to innovative medicines whilst balancing the risk between the NHS and industry. For example, a pay-by-performance model ensures that the NHS only pays for the outcomes a medicine provides to a patient. The BIA has recently published a report which explores the potential use of innovative payment mechanisms as a way of balancing risk between industry and the NHS. The IMF would provide the perfect opportunity to pilot these alternative payment models. The BIA also believes that the IMF presents an opportunity to bridge the gap between marketing authorisation and HTA decision by providing interim funding for those products that obtain an earlier license via ILAP. By providing interim funding from the point of marketing authorisation, the IMF could ensure minimal delay in patient access upon marketing authorisation. Doing so would not only act as an incentive for companies to engage early through ILAP but would also allow additional time for NHSE to discuss with the company which commercial options (including managed access or

	alternative reimbursement mechanisms) might be best suited for the product in question. Interim funding could be provided based on the company Patient Access Scheme (PAS) price and a rebate based on the eventual cost-effectiveness price. For treatments where an MAA would be appropriate, it remains important that the duration of a Data Collection Agreement (DCA) is considered on a case-by-case basis and that it will include review points to keep track of progress. It is positive that NICE will seek advice on data collection from a diverse group of stakeholders including patient groups, clinicians, academics and data custodians to ensure that each DCA takes account of the complexities relating to a treatment and will work with the company to facilitate the development of an appropriate framework for data collection. In designing DCAs, it is important that NICE aims to collect evidence supporting a broad concept of value that extends beyond cost-effectiveness including real world data. Medicines for rare diseases have the potential to significantly improve the quality of life for both patients and their families and carers and these considerations should inform any understanding of the 'value' a medicine has. It is unclear how these proposals will address the additional challenges in data collection for rare and ultra-rare conditions and in particular for ATMPs. While the CDF collects data via the Systematic Anti-Cancer Therapy (SACT) dataset for cancer medicines, there is no equivalent for rare diseases and for conditions with very small patient numbers there is often no established disease registry. The BIA would like to see further detail on how challenges associated with rare disease medicines will be addressed. The proposals state that companies will be responsible for paying a proportionate share of the costs of data collection, validation and analysis. We request clarity on what this will mean for industry as well further detail on the responsibilities of NHSE&I in DCAs. The BIA would also welcome further information on contingency planning for the IMF and CDF should a period of data collection be disrupted, or the required data is unable to be collected (e.g., in a pandemic situation).
Commercial Access Agreements (CAA)	**Disagree** The BIA is concerned about the criterion that companies must fund the cost of continued treatment beyond the period of managed access if a negative NICE recommendation is made. While this criterion was suitable for the CDF, where treatment was likely discontinued after a short period of time (usually less than five years),

	the same does not apply in the non-oncology space, where patients may receive treatment over the course of a lifetime. Rare diseases can often require lifelong treatment. In such cases, it is unviable for a company to fund the remaining treatment in perpetuity if a NICE recommendation following managed access is not made. It is unreasonable for NICE and NHSE&I to expect commercial organisations to be prepared to provide therapies free of charge that are lifelong treatments initiated in infancy. This could mean funding treatment for up to 70 years. This proposal would essentially make the IMF non-viable for these types of lifelong medicines. The BIA proposes that an exemption from this requirement apply to medicines that require lifelong treatment or those without a defined treatment duration. The BIA is also concerned that NHSE&I and NICE have not considered the implications this proposal will have for one-off treatments, such as gene therapies. Gene therapies are life changing and potentially curative treatments that are at the forefront of life sciences innovation. In July 2021, an NHSE announcement on the IMF stated the intention for the IMF to provide access to cutting-edge gene therapies. Unfortunately, we are unable to see how the IMF will work for any of the medicines that NHSE previously claimed will benefit from the IMF, including medicines for rare diseases and cutting-edge gene therapies. For one-off gene therapy treatments, the majority of the patient population may be treated within the first five years of license. Under the current proposals however, there is no mechanism for companies to recoup the financial outlay if the treatment is not recommended for use at the point of IMF exit. We believe that this strongly supports the rationale for the IMF to be used to pilot more innovative risk sharing schemes, such as pay-by-performance, as well as operating MAAs. The proposals also set out that the level of reimbursement should reflect the decision uncertainty and that a company will need to present an offer that brings the range of potentially plausible cost effectiveness estimates to below the relevant cost effectiveness threshold (i.e., £20k-£30k per QALY, taking account of any applicable QALY weightings). They also set out that greater flexibilities will be reserved for products that offer greater value and potential health gain to the NHS. This will represent a significant barrier for treatments for rare and ultra-rare conditions in the absence of appropriately weighted modifiers and particularly for ATMPs. Many orphan and ultra-orphan medicines that are routed to NICE's STA are unable to meet the cost effectiveness threshold of £30k. Small patient populations and lack of current treatment alternatives make it particularly challenging for these treatments to demonstrate their clinical effectiveness. Coupled with high

	development costs, orphan medicines are not able to meet the threshold that was designed for treatments for more common diseases. Though NICE has proposed to replace the existing end-of-life modifier with a severity modifier, the proposed severity modifier does not specifically target orphan medicines, nor does it address the unique challenges associated with rarity. Though some rare disease medicines may receive a severity modifier where the disease is considered severe enough, NICE's own research shows that a host of orphan medicines previously appraised in STA would not qualify for a severity modifier. Even where they do, a QALY weighting of 1.7 (equivalent to a threshold of £50k) is unlikely to make the difference. In our response to NICE's consultation on proposals for change, the BIA indicated that in order to address the challenge faced by orphan and ultra-orphan treatments in the STA programme, a modifier is needed that bridges the gap between the upper STA threshold (£30k) and the baseline HST threshold (£100k) for rare disease treatments. Without this change, developers of innovative medicines for rare and ultra-rare diseases will be unable to present an offer that brings the range of potentially plausible cost-effectiveness estimates below the £30k threshold. This will severely limit the opportunity that the IMF could provide for rare and ultra-rare diseases and will enable earlier access for patients with a high degree of unmet need. The BIA requests that NHSE&I and NICE use the midpoint rather than the entire plausible range of cost effectiveness when reviewing offers made by companies. We suggest that the proposals be changed to "the company will need to present an offer that brings the mid-point of the range of potentials plausible cost-effectiveness estimates as determined by NICE to below the relevant cost-effectiveness threshold". The proposals state that a Budget Impact Test (BIT) assessment will not apply to medicines recommended for managed access use in the IMF but will be applied at the point of NICE re-evaluation. The BIA disagrees with the proposal to apply a BIT at the point of re-evaluation. The BIT was introduced as a mechanism to signal the need for dialogue between companies and NHS England to agree special arrangements to better manage the introduction of new technologies and as a means of managing the introduction of medicines to the NHS. We consider it inappropriate to apply the BIT on exit of the IMF since medicines will already have been in the NHS, in some cases for five years.

Updating NICE guidance following a period of manages access and exiting the IMF?	**Neither agree nor disagree** In the case that a medicine receives an 'optimised' recommendation after access via the IMF, it is unclear whether the company will be required to continue providing treatment, without charge, to the sub-group excluded from routine funding.
Interim Funding for NICE recommended medicines?	**Agree** The BIA agrees in principle with the proposal for the IMF to provide interim funding. This proposal would benefit developers of innovative medicines where pathway change required would result in delayed implementation of the NICE recommendation. We request clarity on how a medicine would qualify for interim funding. For example, it is unclear whether a medicine is required to have been through a NICE HTA, or whether early funded access would be provided pending a full HTA. We also request greater clarity on the types of treatment that will be eligible for interim funding through the IMF. The proposals currently state that NHSE envisages that interim funding will only be made for medicines that have been commissioned in the context of a prescribed specialised service. The BIA is concerned about the prospect of interim funding being allocated too broadly and resulting in a significant amount of the £340m annual funding allocated to the IMF being used up, potentially triggering the ECM. The BIA emphasises that the IMF should be reserved for the most innovative medicines. If interim funding is available to any medicine that NICE can recommend for routine commissioning in the NHS, this would not be in keeping with the original vision of the IMF and would not increase equity of access to the most innovative medicines. We strongly recommend that interim funding be limited to the most innovative medicines including those that are part of ILAP or EAMS. These medicines should be able to access IMF interim funding via a 'light touch' appraisal to minimise delay.
Financial control?	**Disagree** The BIA is disappointed by NICE's recommendation to introduce an ECM. This would result in a double rebate being required for some companies if this was required in addition to VPAS rebates. The VPAS rebate acts as an expenditure control mechanism for the total branded medicines budget. Ultimately, if the NHS and patients are benefitting from a potentially transformative medicine at a cost-effective price then a rebate should not be required. The BIA recognises that the ECM has been proposed to ensure that the IMF will never have to close to potential new entrants. Despite this,

	the risk the ECM poses to small companies makes the IMF unviable for them. Given the proportion of innovative medicines that are developed by smaller companies, we are very concerned that the level of risk posed by the IMF will disincentivise these companies from choosing the UK as a first launch country for innovative technologies. In particular, the BIA is concerned about the impact the IMF's financial control proposals will have on patient access to medicines for rare diseases, including ATMPs. The BIA feels that rarity is a specific challenge that has not been adequately addressed through the NICE Methods Review. Unfortunately, this will have the effect of increasing the magnitude of the challenge that the IMF is designed to address. To ensure rare disease patients in the UK are able to access the innovative medicines they need, it is crucial that the IMF is accessible to and set up to address the specific challenges faced by rare disease medicines in demonstrating their clinical and cost-effectiveness. Under the current proposals, IMF poses too great a risk for developers of rare disease medicines, many of whom are small companies, due to the size of the fund and the uncertainty regarding future costs. If the IMF is unviable for these developers, this could have a detrimental impact on the prospects of rare disease medicines reaching patients and would not result in the improved access that the IMF aims to achieve. To avoid disincentivising companies from developing innovative treatments, and to ensure the UK is a viable place to bring rare disease medicines to market, the BIA recommends that if the ECM is to apply, medicines for rare diseases, including ATMPs, should be exempted from the rebate.