National Institute for Health and Care Excellence

The NICE methods of health technology evaluation: the case for change

Consultation: 6 November – 18 December 2020

Introduction

Thank you for participating the in the consultation on the NICE methods of health technology evaluation: the case for change.

We are interested in hearing your thoughts about:

- our proposals
- how we've taken the evidence and considerations into account
- any potential effects and implications for patients and their families, health technologies, the life sciences industry and the NHS.

The information collected will be used to inform the next steps for the development of the NICE methods for health technology evaluation. Comments will be published in full on the NICE website after the consultation closes (excluding responses from NICE staff and committees). **Please do not include any personal information in your response**. NICE will not respond to individual comments or suggestions.

Instructions

There are 5 sections of the potential areas for change:

- Valuing the benefits of health technologies
- Understanding and improving the evidence base
- Structured decision making
- Challenging technologies, conditions and evaluations
- Aligning methods across programmes

This form provides space to respond to the consultation questions for each area. There is space for additional comments. You do not have to provide comments for all sections.

When responding, please remember the objectives of the review and the boundaries of the current stage, as described in the consultation document. In particular, this consultation focuses specifically on the methods of health technology evaluation (and not its processes or other related developments, which are considered

separately), and presents the evidence and case for change only (a finalised methods framework will be developed in the next stage).

Please type your responses directly into the tables in this form. If you wish to refer to a particular section, paragraph or proposal, or any of the supporting documents, please indicate the relevant name, number or letter that you are referring to within your response. Please do not include any personal details in your comments.

Submitting your response

Return your completed response form via email to methodsandprocess@nice.org.uk by 11:59pm on 18 December 2020. Responses submitted in any other format will not be accepted

Privacy notice

For more information about how your data will be processed please see our Privacy Notice

About you

To help us understand and theme your comments during review, please indicate which category best describes who your response is from by adding the name of the organisation next to the relevant category

Alternatively, if you are responding as an individual, please add your job title next to the individual that best describes your role.

Organisations

Category	**Name of organisation**
example organisation type	*e.g. Write the name of organisation here*
Academic body	
Device industry	
Devolved nation	
Diagnostic industry	
Industry body	UK Bioindustry Association (BIA)
Life sciences consultancy	
NHS organisation	
Patient organisation	
Pharmaceutical industry	
Professional organisation	
Other type of organisation	

Individuals

Individual	**Job title**
Example individual	*e.g. Write job title here*
NICE committee member	
NICE staff	
Other individual response	

Consultation comments

Valuing the benefits of health technologies

Consultation questions - valuing the benefits of health technologies	Comments
Do the proposals and cases for change provide a suitable basis to inform the final methods? • Do you have any comments or feedback on the methodological evidence and considerations that have been taken into account, or how the evidence has been interpreted? • Do you have any comments or feedback on how well the proposals will achieve the aims of the review? What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS? • What are the potential benefits of the proposed cases for change? • Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them? Do you have any comments or feedback on how well the proposed methods will support innovation for patients, science, society and the life sciences industry?	**Discounting** The BIA agrees with NICE that there is a case to change the reference-case discounting rate. It is positive that NICE acknowledges this case for change. The BIA appreciate the significant amount of work that system partners, patient groups, academics and industry have put into carrying out the analysis and developing the Task and Finish Group report, and we are pleased to see that the case for change is reflected in this consultation. While we are pleased to see the recognition of the case for change, we firmly believe that this should remain within the scope of this review and should inform the final methods. The review aims to ensure that NICE's methods 'remain cutting edge and future proof.' Further review of the discount rate will contribute to achieving this objective. We therefore think the discounting rate must be within scope of this review. Advanced and personalised medicines, such as ATMPs and medicines which are able to treat rare genetic disorders early in the disease pathway, are increasingly emerging as treatments that address a high level of unmet need. These medicines tend to incur a high up-front cost while the benefits are seen over a long timeframe and may occur far in the future. Decreasing the discount rate to 1.5% will ensure that

Consultation questions - valuing the benefits of health technologies	Comments
	the benefits of these treatments are adequately evaluated based on the value they bring in the long term. This change would allow NICE to more fairly evaluate technologies where the benefits occur further into the future by ensuring these benefits are adequately valued in the ICER calculation. It would also bring NICE's methodology in line with that used by other Government departments, such as DEFRA, PHE and DHSC. The BIA believe that there is a case for differential discounting rates of 3.5% for costs and 1.5% for health effects. We acknowledge that the Discounting Task and Finish Group and the Working Group have reviewed a significant quantity of evidence and carried out extensive analysis before concluding that the best available evidence suggests there is a case for changing the reference-case discount rate from 3.5% to 1.5%, for both costs and health effects. However, differential discounting rates will align with the Treasury Green Book which was updated in 2018 and states: "The Social Time Preference Rate used in the Green Book is set at 3.5% in real terms, with exception for risk to life values which use a lower rate of 1.5% (…) because the 'wealth effect', or real per capita consumption growth element of the discount rate, is excluded."[1] The current review would be the appropriate forum to consult on the options for change to NICE's discounting rates.

[1] HM Treasury (2020), The Green Book: https://www.gov.uk/government/publications/the-green-book-appraisal-and-evaluation-in-central-governent

Consultation questions - valuing the benefits of health technologies	Comments
	Changing the discount rate to account for the long-term benefits of ATMPs and other medicines with long-term benefits would send a strong signal and 'support the attractiveness of the UK as a first-launch country for important and promising new health technologies', something NICE has stated as an objective of this review. Leaving it outside the scope of this review has the potential to negatively impact innovation, with companies choosing to launch these technologies in the UK later in their lifecycle, if at all. Consequently, patients may face delayed or limited access and recent efforts by the MHRA to position the UK as a first launch-country may be impeded. **Severity modifier** The proposal to replace the current modifier for life-extending treatments at the end of life with a severity modifier is welcome. The BIA strongly welcomes the progress on modifiers which has so far been achieved in this review. The severity modifier could lead to greater flexibility for a wider range of orphan and ultra-orphan medicines that have not traditionally qualified for the end-of-life criteria. As the end-of-life criteria tends to benefit cancer patients compared to other patients, this proposal will help to ensure greater equality between cancer therapies and therapies for other severe conditions. The BIA also welcomes and agrees with the proposal that the severity modifier be applied in all technology evaluations. If implemented appropriately, this proposal will

Consultation questions - valuing the benefits of health technologies	Comments
	increase equality in NICE's HTA methods and contribute to achieving NICE's stated aim of more flexible, agile, and robust methods. At the current stage, The BIA supports the proposal that severity will implicitly encompass concepts such as the burden of illness and the degree of unmet need in a condition. Rare and ultra-rare diseases place a significant burden on patients, families, carers and society. People living with rare and ultra-rare diseases face high degrees of unmet need, with the great majority of these conditions lacking licensed treatments options. It is therefore positive that NICE recognises these factors as integral to defining severity. This has the potential to facilitate access to treatments for the patients who face the toughest burdens of illness and unmet needs, such as rare disease patients. The BIA believes that this proposal has the potential to better recognise the value of innovation in severe conditions, which will benefit patients, the NHS, and the wider life sciences ecosystem. As NICE also acknowledges, further work on how to define, measure, and implement the severity modifier is needed. In addition to burden of illness and unmet need, this should consider both the impact on quality-of-life and length-of-life for a particular condition. We strongly recommend a broader consideration of how severity may manifest in both current health status and

Consultation questions - valuing the benefits of health technologies	Comments
	expected future health status in the absence of treatment, thereby placing greater value on avoided expected severe events/consequences. We believe that this modifier should therefore account for technologies that prevent imminent, although not current, severe health states. It is vital that industry and patient groups continue to be involved in this process as the methods review progresses into its next phases. Getting this right will be essential in order for the severity modifier to deliver in terms of the potential impact for appropriately valuing treatments for severe conditions. The BIA has been greatly encouraged by the level of cooperation between system partners, patient groups, and industry, and we look forward to seeing this continue in the next phase. The BIA is concerned, however, that the proposed severity modifier, while a very positive step forward, may result in some patients with rare diseases, in particular, falling through the gaps. We believe it will be important to highlight rarity and innovation as distinct topics and attributes in decision making that attract social value over and above characteristics of the evidence base. **Uncertainty** The BIA welcomes the case to retain uncertainty as a key factor that influences decision making and to introduce more flexibility in interpreting uncertainty. We support the proposal

Consultation questions - valuing the benefits of health technologies	Comments
	that committees should have the flexibility to accept greater uncertainty and risk. As NICE acknowledges, this will have positive implications for a broad range of treatments for rare and ultra-rare diseases where uncertainty and data limitations are inherent due to e.g. small patient populations and less well understood epidemiology. We are pleased to see that NICE recognises the complexities and difficulties involved in generating evidence in this area and that proposals are included to address this. If implemented appropriately, flexibility to accept greater uncertainty and risk can facilitate earlier and enhanced access to rare and ultra-rare disease treatments, which in turn can achieve NICE's stated aim to 'support the attractiveness of the UK as a first-launch country for important and promising new health technologies'. This will support innovation and benefit patients, the NHS and the life sciences industry. We furthermore welcome the proposal to accept greater degrees of uncertainty for innovative technologies and in cases where there is likely to be a high degree of benefit, and an acceptance of uncertainty which can be managed through a managed access arrangement. This is particularly important to build on the work of MHRA, which has recently specified how to integrate real world evidence as part of data-generation. The review should continue to build on this progress in order to achieve NICE's stated aim of aligning with the changing UK regulatory system.

Consultation questions - valuing the benefits of health technologies	Comments
	As stated by NICE, further work is needed to define more precisely the situations in which greater uncertainty can be accepted. The BIA agrees that a clear understanding and definition of innovation is needed, and we suggest that this definition encompasses medicines which address an unmet need. It is crucial that industry and patient groups continue to be involved in this process as the methods review progresses into its next phases. **Health inequalities** The BIA believes it will be important to appropriately define health inequalities if there are to be applied to a modifier. In particular, the we would be keen to understand how the broader socioeconomic elements of health inequalities – such as housing, deprivation and disabilities – will be incorporated into any changes to the decision-making process.
What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	The BIA is supportive of implementing similar approaches to valuing technologies across all of NICE's programmes.
Do the proposals create any equalities concerns, particularly for NICE's legal responsibilities and the important need to eliminate unlawful discrimination and promote equality?	We believe the cases for change and proposals discussed above will increase equality across all NICE's programmes.

Consultation questions - valuing the benefits of health technologies	Comments
General comments: If you have additional comments on this section please share them here:	

Understanding and improving the evidence base

	Consultation questions - understanding and improving the evidence base	Comments
1	Do the proposals and cases for change provide a suitable basis to inform the final methods? • Do you have any comments or feedback on the methodological evidence and considerations that have been taken into account, or how the evidence has been interpreted? • Do you have any comments or feedback on how well the proposals will achieve the aims of the review? What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS? • What are the potential benefits of the proposed cases for change? • Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them? Do you have any comments or feedback on how well the proposed methods will support innovation for patients, science, society and the life sciences industry?	**Real-world evidence** We welcome the recognition of the potential value of real-world evidence for HTA as this will support the ambition for patients to have access to the latest medicines as close to licencing as possible. In the context of small RCT populations, greater understanding of the value of real-world evidence is beneficial alongside guidance within the methods on collecting, analysing and reporting real world evidence. However, we suggest that in order to further recognise and mitigate the challenges faced by some therapies, such as those for the treatment of rare diseases, it is vital that NICE's proposals go further. We recognise there has been significant progress in tackling the impact of uncertainty in the context of appraisals. However, we would like to emphasise that greater acceptance of real-world evidence will provide much needed flexibility in cases where randomised control trial (RCT) data are difficult to obtain. This is particularly true in the case of rare diseases, where small patient populations and lack of a benchmark standard of care are significant challenges. Beyond that, even in therapy areas with established histories, diseases are being targeted with ever increasing accuracy, leading to similar challenges in areas, such as oncology. We would also be encouraged to see recognition of the potential of novel clinical trial designs to deliver effective and conclusive data.

	Consultation questions - understanding and improving the evidence base	Comments
		We call on NICE to give further consideration to the matter of the sources behind evidence and its transferability from other jurisdictions. **Costs used in HTA** In order for NICE to fairly assess therapies which have the potential to extend life of patients, direct healthcare costs incurred in periods of extended survival due to the novel intervention should be excluded. This is not currently the case, which leads to medicines which significantly extend life being disadvantaged compared to those that offer no life extension. We look forward to further details on how NICE intends to account for costs and benefits in periods of extended life due to health technology intervention. **Health-Related Quality of Life** We support NICE's proposal for the acceptance of utility measures other than EQ-5D but believe that it does not address the challenges with measuring health related quality of life in chronic genetic conditions. Therefore, we call on NICE to accept non-generic utility measures in circumstances where generic measures prove to be inadequate A proposal to outline a hierarchy of evidence around health-related quality-of-life when EQ-5D is not available or appropriate is also positive. While we recognise the need to be

	Consultation questions - understanding and improving the evidence base	Comments
		able to compare the impact of interventions across a range of conditions in a consistent way, it is also important that the quality-of-life impact is appropriately measured. In this context, there should be an explicit acceptance of additional disease-specific quality-of-life measures which may be more sensitive to highlighting the impact of interventions. We welcome the recognition that changes in the methods of measuring quality-of-life in children and young people are required and that research is underway to explore this complex topic. It is disappointing that there is no immediate recognition of a case to change the option to include carer health-related quality-of-life (although research will be undertaken). In both instances, research should be accelerated and interim measures put in place to ensure that treatments that have a significant impact on children and their families and carers are not disadvantaged as these research projects are progressed. **Benefits** There are very high levels of unmet need among people with rare diseases. As science begins to provide treatments for the first time, we believe the appraisal system will need to flex to accommodate the specific challenges around small patient populations. We feel there are clear benefits in the proposal for patients with rare diseases, in particular in these proposals around real-world evidence, which opens up a significant resource to support the appraisal of medicines in this area.

	Consultation questions - understanding and improving the evidence base	Comments
		A more flexible approach will support NICE in being able to make fast recommendations, close to medicines receiving their Marketing Authorisation. It will ensure NICE moves at the same pace as the regulators, including MHRA, in understanding novel trial designs and using the best available evidence to support decision-making. We support the proposals to clarify NICE's preferred approach for modelling sensitivity analyses and extrapolations of data. It is critical that Appraisal Committees are basing their decisions on plausible parameters and we consider there to be a significant opportunity to better support this through the update of the methods and NICE's review of appraisal processes. **Risks** Further information is needed on exactly how these proposals will be implemented. While ensuring that the changes do increase access to patient populations which experience high levels of unmet need – e.g. rare diseases – we must ensure that they do not result in lack of access in other areas, such as the care and treatment of older people.
3	What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	We understand that work is underway to investigate how to assess the impact on carer quality of life as part of the appraisal process. These data will be very important to understand the value of medicines, particular those which treat potentially

	Consultation questions - understanding and improving the evidence base	Comments
		debilitating diseases and disproportionately affect younger people.
4	Do the proposals create any equalities concerns, particularly for NICE's legal responsibilities and the important need to eliminate unlawful discrimination and promote equality?	Please note our comment above in the 'risk' section about avoiding impact on the care of older people.
5	General comments: If you have additional comments on this section please share them here:	

Structured decision making

	Consultation questions - structured decision making	Comments
1,2	Do the proposals and cases for change provide a suitable basis to inform the final methods? • Do you have any comments or feedback on the methodological evidence and considerations that have been taken into account, or how the evidence has been interpreted? • Do you have any comments or feedback on how well the proposals will achieve the aims of the review? What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS? • What are the potential benefits of the proposed cases for change? • Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them? Do you have any comments or feedback on how well the proposed methods will support innovation for patients, science, society and the life sciences industry?	**Subgroups** NICE states that committees may choose to exclude a subgroup from a recommendation if they consider it appropriate, even if the technology is clinically and cost-effective in the whole patient population. The BIA opposes this proposal since limiting access for subgroups where a technology has been deemed clinically and cost- effective increases inequality and reduces consistency in NICE's methods. We also suggest that this proposal goes against the spirit of NICE's ethical and legal duty to support fairness and equality. It also goes against NICE's stated aim of 'ensuring rapid access to clinically and cost-effective technologies.' We would also be keen to understand what plans are in place to review decisions should additional data become available.
3	What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	The proposal on excluding certain subgroups risks increasing inequality and reduce consistency in NICE's methods.

	Consultation questions - structured decision making	Comments
4	Do the proposals create any equalities concerns, particularly for NICE's legal responsibilities and the important need to eliminate unlawful discrimination and promote equality?	As stated above, we believe the proposal on excluding certain subgroups from recommendations creates equality concerns in NICE's programmes.
5	General comments: If you have additional comments on this section please share them here:	

Challenging technologies, conditions and evaluations

	Consultation questions - challenging technologies, conditions and evaluations	Comments
1	Do the proposals and cases for change provide a suitable basis to inform the final methods? • Do you have any comments or feedback on the methodological evidence and considerations that have been taken into account, or how the evidence has been interpreted? • Do you have any comments or feedback on how well the proposals will achieve the aims of the review?	**Advanced Therapeutic Medicinal Products** ATMPs face very specific challenges which include the uncertainty (particularly in cases where treatments are potentially curative), high upfront costs, the impact of discounting and variation in how delivery costs are considered in the appraisal process. These present real and pressing challenges in the context of the current medicines appraisal process. The proposal to change the discount rate to 1.5% is critical to ensure the longer-term benefits ATMPs offer are appropriately valued. Accepting more uncertainty in the evidence base will better reflect the level of certainty that is possible when considering the value of such innovative therapies when the scientific promise is there, but it cannot be fully evidenced for several years, in many cases even a lifetime given the time horizon of the model. The true potential of these technologies will only be evidenced and highly certain in decades' time and this must be acknowledged in the management of uncertainty. Uncertainty is often thought of as a bad thing, but it can also be viewed as very positive - it represents the fact the industry is innovating and making progress in new ways of understanding and treating diseases. The implementation of modifiers in the decision-making framework, particularly the severity modifier, will also be critical

	Consultation questions - challenging technologies, conditions and evaluations	Comments
		to having a decision-making framework that is fit for purpose for ATMPs. The longer-term impact on patients, their families and carers, the health service and society more broadly need to be sufficiently factored into the costs and benefits that are weighed up in the appraisal. ATMPs offer significant opportunity to save the healthcare system costs and resource that would otherwise be spent managing the symptoms of disease, not all of which have been considered relevant in appraisals. Similarly, the enormous impact that offering a potential cure can have, often in childhood diseases, on not only the patient but their family and carers is under-valued. This impact simply cannot be captured in the QALY and is not considered by Appraisal Committees outside of the HST evaluation programme. If these proposals in the consultation are not taken forwards and implemented in a sufficiently progressive way, there will be a need to revisit the Technology Appraisal methods from an ATMP perspective. **Rarity** The BIA believes that medicines for rare diseases in and of themselves present specific challenges that are not given sufficient regard in the current appraisal process. We recognise that the HST programme is intended to provide a route to positive appraisal, but there remains a significant gap between

	Consultation questions - challenging technologies, conditions and evaluations	Comments
		STA and HST. The BIA is supportive of a rarity modifier to provide an effective access route. The NHS is intended to be a universal health service and it would seem unequal to deny access to treatments for patients with rare diseases simply because they are rare. We understand that the modifiers Task and Finish Group specification sets out a proposed maximum QALY weighting of x1.7. This is insufficient to sufficiently bridge the gap between the STA and HST thresholds for rare disease medicines.
2	What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS? • What are the potential benefits of the proposed cases for change? • Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them? • Do you have any comments or feedback on how well the proposed methods will support innovation for patients, science, society and the life sciences industry?	**Benefits** Improving horizon scanning processes to identify new types of technology earlier and develop action orientated plans to support their journey through NICE, commissioning and implementation in the NHS, will benefit patients, their families, the life sciences industry and the NHS. It will help ensure we have an innovation friendly ecosystem that allows rapid introduction of the newest, most innovative treatments. Evolving NICE's methods to address the challenges faced by highly innovative treatments like ATMPs offers significant opportunity to benefit patients, their families, the NHS and society more broadly. The life sciences industry will be encouraged to continue investing in new ways of treating, even curing, disease which has huge implications for supporting a healthy, productive society and economy.

	Consultation questions - challenging technologies, conditions and evaluations	Comments
		Risks We believe the biggest risk would be to do nothing at all. We are heading into a world where ATMPs are becoming increasingly common, not just in oncology but across a wider range of therapy areas. Action is therefore needed now before many of these treatments come online to ensure that the HTA system is ready to assess these treatments and recognise the potentially transformative impact they will have on individual, the NHS and wider society.
3	What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	NICE's appraisal of ATMPs is the subject one of the Accelerated Access Collaborative's (AAC's) live workstreams on ATMPs. It is important that this work, and the work of the AAC in this context are sufficiently joined up, both to ensure that the HTA system is suitable for ATMPs and also to help support systemic changes being considered by the AAC to ensure access to ATMPs for patients.
4	Do the proposals create any equalities concerns, particularly for NICE's legal responsibilities and the important need to eliminate unlawful discrimination and promote equality?	

	Consultation questions - challenging technologies, conditions and evaluations	Comments
5	General comments: If you have additional comments on this section please share them here:	

Aligning methods across programmes

	Consultation questions - aligning methods across programmes	Comments
1	Do the proposals and cases for change provide a suitable basis to inform the final methods? • Do you have any comments or feedback on the methodological evidence and considerations that have been taken into account, or how the evidence has been interpreted? • Do you have any comments or feedback on how well the proposals will achieve the aims of the review?	We broadly welcome the proposal to align cost comparison methods across the evaluation programmes.
2	What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS? • What are the potential benefits of the proposed cases for change? • Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them? • Do you have any comments or feedback on how well the proposed methods will support innovation for patients, science, society and the life sciences industry?	

	Consultation questions - aligning methods across programmes	Comments
3	What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	
4	Do the proposals create any equalities concerns, particularly for NICE's legal responsibilities and the important need to eliminate unlawful discrimination and promote equality?	
5	General comments: If you have additional comments on this section please share them here:	

General comments

Please provide any other comments you may have here.

In this current review, the industry has been encouraged by the scope of engagement for participants, both as part of the Working Group and the specific Task and Finish Groups sitting under it. This has been made possible in spite of extremely challenging circumstances during the COVID-19 pandemic. Efforts were made early on to promote effective engagement and input from industry, patient groups and others and we hope that any future review will seek to replicate that. Recognising how much work has already been put into getting the case for change ready for consultation, we must be mindful that there remains a long road ahead before the proposals are ready to be put into effect. As set out above, while we welcome the general thrust of the planned changes, the detail on how they will operate in reality will be vitally important. We look forward to the technical consultation in the New Year to understand NICE's plans to implement these changes fully. One area of concern highlighted in the more detailed response above the need for greater focus and regard to issues surrounding rarity. While the severity modifier and the changes proposed to tackle uncertainty do go some way to ameliorating

some of the issues related to rarity in medicines appraisals, we are unconvinced that they go far enough in addressing the specific challenges faced by rare disease medicines in this process. Rare disease medicines which do not qualify for a severity modifier would therefore be assessed against a £30k threshold. This would significantly impact the ability to launch these medicines in the UK, resulting in limited access for UK patients. The BIA has recently worked with PwC to publish a report – *A rare chance for reform* – setting out the case for a rarity modifier and other changes to the medicines appraisal process that we believe would deliver fairness to the NHS and secure access to these medicines for patients. These recommendations include:

Accelerate access through a conditional access period:

Introduce a fast initial evaluation that grants conditional access through a Managed Access Agreement, at a price consistent with other fast-adopting countries. The proposed Innovative Medicines Fund (IMF) would be the ideal vehicle to fund medicines within the Managed Access Agreement. This initial access should be followed by a more in-depth reevaluation after a period agreed on a medicine by medicine basis, to improve the certainty and quality of data available for assessment. This process should be aligned with the existing accelerated regulatory processes by which drugs are often approved and be supported by adequate infrastructure to enable collection of real world evidence.

Address systemic issues to build a strong environment for access to orphan and ultra-orphan medicines:

Resolve systemic issues such as consistency in evaluations, balancing value for money and patient needs, and ensuring appropriate infrastructure is in place to maximise the value of these treatments. Increase sustainability of funding for rare diseases: Increase sustainability in funding arrangements for orphan and ultra-orphan medicines by reinvesting savings made from appropriate use of biosimilars and generics, and agreements such as VPAS, into the orphan medicine ecosystem.

Update the evaluation framework to better account for the unique challenges of rare and ultra-rare diseases:

Assessments should be adapted to determine the value of orphan and ultra-orphan medicines holistically, by capturing direct health benefits and indirect benefits. This can be achieved by amending the way that clinical and cost effectiveness are calculated and pragmatically used, and increasing the flexibility for incremental cost-effectiveness ratio (ICER) thresholds through modifiers, to ensure the process is fairer and more robust.

Evaluate orphan medicines and ultra-orphan medicines through a single rare disease process:

Adopt a single process to ensure that all orphan and ultra-orphan medicines are assessed by a process that accounts for their unique challenges.

Assess empirically based ICER thresholds on a sliding scale:

Create a sliding scale of thresholds for assessing orphan medicines supported by clear criteria on where an orphan medicine falls on the scale, to remove the need for arbitrary thresholds.

Continue to create a supportive atmosphere for patient groups:

Strengthen NICE's existing approach to empower patient groups by identifying and addressing the concerns of smaller patient organisations, improving communication with stakeholders during the evaluation process and providing clarity on how evidence presented by patient groups translates into decisions.

We look forward to having the opportunity going forward to discuss these proposed changes that could form the basis of a future review.

Thank you for completing the consultation

Your participation is appreciated. Your responses will be used to inform the next steps for the development of the NICE methods for health technology evaluation.

Submitting your response

Return your completed response form via email to methodsandprocess@nice.org.uk by 11:59pm on 18 December 2020.
Responses submitted in any other format will not be accepted