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	
Life sciences consultancy	
NHS organisation	
Patient organisation	Blood Cancer Alliance
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?	The future of blood cancer treatment is promising, with many new treatment options on the horizon. However, this means little if they cannot be accessed by the patients that need them. While we fully support NICE's efforts to improve its methods, it must represent meaningful change to address the challenges that currently exist in the appraisal process, particularly for treatments for rarer conditions, where patient cohorts are small and meeting traditional RCT evidence requirements is sometimes impossible. MODIFIERS In the Blood Cancer Alliance's new Access to Medicine report, published in October 2020, we identified the need for NICE to bring in a greater number of modifiers. We cautiously welcome the new severity modifier, as in principle, it may prove helpful in the appraisal of rare cancers, such as leukaemia and other blood cancers. Rare cancers often have fewer treatment options available than more common cancers, creating greater severity. It is also important that the new modifier should not disadvantage those indications that already

Consultation questions - valuing the benefits of health technologies	Comments
	meet end of life criteria; this is an inherently severe state of health. We do not believe the addition of the new severity modifier is sufficient, however. We understand that a rarity modifier was considered by NICE, but disregarded. We do not agree with this decision. There are specific challenges with rarer diseases which warrant the inclusion of the ability to make adjustments – and a modifier would be appropriate to achieve this. We understand that within the Highly-Specialised Technology (HST) process rarity is considered. However, HST has not considered blood cancer treatments in the past, and we understand that treatments for any cancer indications are extremely unlikely to fall within HST criteria in future. If the HST is not available to cancer treatments, a modifier is required to consider rarity, and its impact on evidence, within the Technology Appraisal (TA) process. If NICE is unwilling to re-evaluate its decision on a rarity modifier, then consideration must be given to other processes that can ensure equity of access for patients with rare diseases. While not strictly an issue of modifiers, we would also like to raise our concern that there is a lack of consistency as to what patient population is small enough for HST or large enough for TA. NICE

Consultation questions - valuing the benefits of health technologies	Comments
	should take the opportunity of the imminent HST review to better clarify the HST criteria. We also strongly urge NICE to include within this imminent review consideration of changes that need to be made to HST criteria so as not to immediately exclude cancer treatments from this process. By achieving this, in combination with a rarity modifier in the TA process, NICE can ensure treatments for rarer blood cancers are not disadvantaged by NICE processes, including the TA process, which takes a narrower perspective than the HST programme and does not allow for higher cost per QALYs. UNCERTAINITIES, REAL WORLD AND NON-RCT DATA We welcome proposals to clarify, in NICE's methods, its approach to real world data collection, and also for data collection from non-randomised controlled trial (non-RCT) sources, such as evidence drawn from managed access schemes such as the Cancer Drugs Fund (CDF). We would like to see stronger guidance as to how, under the CDF, NICE and NHSE should establish a link between the main clinical uncertainties identified at the time of the first appraisal by NICE and the clinical data that is generated during the time that a treatment is within the CDF. This will ensure that while available

Consultation questions - valuing the benefits of health technologies	Comments
	under CDF, the right data is being collected to ensure treatments can move through the STA process when appropriate. We welcome proposals for clearer guidance on the circumstances in which non-randomised controlled trial evidence is acceptable and would be useful. Research has suggested that in some cases, including in blood cancer, there is not always a match between the uncertainty identified by NICE and the data being collected. We fully support the proposal for NICE to issue detailed guidance to aid companies in considering what real-world evidence, including features of registries. We also urge NICE to include patient group surveys as sources of real-world evidence acceptable to support submissions. Guidance should go beyond the submission if appropriate, to help guide real-world evidence generation that can be conducted to address uncertainties at the time of appraisal. This will help send signals to all those involved in setting up and reforming existing real-world data sources as to NICE's requirements. The BCA would like to see greater transparency in the NICE decision-making process around factors other than cost-per-QALY that are taken into account. These factors should be made clear in the decision-making framework.

Consultation questions - valuing the benefits of health technologies	Comments
	Overall, we welcome the proposal to be more flexible about uncertainty, but we would welcome greater detail about how this will work in practice. INNOVATION We would like to see better mechanisms for patient engagement in NICE's innovation processes.
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?	We recognise that the proposals on severity modifier have the potential to benefit blood cancer patients but could also potentially disadvantage those who qualify for the existing end of life modifier. More information is required before a full assessment can be made. In general, we feel disappointed, however, by the lack of consideration within this review as to how the patient experience and view can be better incorporated into NICE's appraisal processes.

Consultation questions - valuing the benefits of health technologies	Comments
What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	
Do the proposals create any equalities concerns, particularly for NICE's legal responsibilities and the important need to eliminate unlawful discrimination and promote equality?	
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?	While we welcome the case for change to consider further the role of real-world evidence or non-RCT evidence, we would have liked to see more detail as to how this will be achieved, and how it will be incorporated into decision-making processes. We are extremely disappointed, however, that this consultation does not reference any detailed plans for improvement in how NICE incorporates evidence and information from patients themselves. We would require a much greater level of detail as to how NICE will achieve the proposal to make the impact of expert input, such as patients, clear within the decision-making framework. We cannot make any assessment of whether the proposals will achieve the aims of the review unless we understand exactly how NICE proposes to achieve this. As part of this review, NICE should set out a proposal as to how it will use quantitative patient preferences in decision-making and economic modelling. We welcome the case to include guidance on qualitative evidence use, and also the recognition that is particularly important for rarer diseases, which include many blood cancers. Greater clarification as to how this will impact decision-making is needed, and indeed, how patient evidence is considered in this context. To achieve this, NICE should develop a comprehensive evidence base on their approaches to

	Consultation questions - understanding and improving the evidence base	Comments
		involving patients and their representatives - with a focus on the difference it makes to decisions. We welcome the involvement of patients and their representative organisations as part of the technical engagement step, but request that the impact of patient engagement on decision-making be made more transparent, with full reporting on this. Generation of this evidence base should include not only patients and their representative organisations, but also bring in external independent researchers. This evidence base should encourage more patients and patient organisations to engage and participate in NICE activities in the future. We welcome the case for change for using surrogate outcomes where appropriate, but more detail is required.
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?	The proposals on extending the evidence base have the potential to capture additional benefits of treatments. Additionally, patient groups will feel more likely and better able to engage if they are clearer on the types of evidence permissible and how it will be taken into account in the decision-making process.

	Consultation questions - understanding and improving the evidence base	Comments
	• 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?	
3	What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	n/a
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?	n/a
5	General comments: If you have additional comments on this section please share them here:	n/a

Structured decision making

	Consultation questions - structured decision making	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 are disappointed by the absence of a case for change where a treatment in not cost effective at zero price. This is something that has occurred within blood cancer appraisals. A solution needs to be found to ensure that patients can access new and effective treatment. Challenges in this area are likely to increase with a greater number of combination therapies emerging for blood cancers. We appreciate that addressing the challenges that arise from combination treatments is not entirely in the purvey of NICE, as a component of the issue is multi-indication pricing. However, we urge NICE to consider its position on the case for change in this area as part of this review. We strongly urge NICE to use this review to also address the issue of treatments that are deemed not cost effective at zero price due to background, unrelated healthcare care costs. We believe there is a pressing need for clarity in this area, driven by new NICE guidance on when committees should depart from the reference case.
2	What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS?	The proposal to allow committees to optimise recommendations, even where the population is cost-effective, risks indirect discrimination. For example, patients with lower ECOG scores or co-morbidities are likely to be older patients.

	Consultation questions - structured decision making	Comments
	• 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?	
3	What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	n/a
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:	

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?	It is disappointing that this section is lacking a case for change for rare disease specifically, despite evidence that there are specific challenges for this group illnesses. The section on ATMPs does not provide sufficient detail to understand whether it will address the challenges that arose with previous ATMP appraisals. This suggests that the methods review will not be future-proofed, as it seems that the challenges already faced with ATMPs have not been fully considered, nor comprehensive proposals developed to overcome them. New technologies will provide further challenges, and we would like to see within this review a NICE commitment to work in partnership with NHS England and the pharmaceutical industry to better incorporate horizon-scanning into its processes, to ensure it is equipped to deal with future developments.
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?	If horizon-scanning and preparing for future developments is not central to NICE's practices, it will put future innovation at risk.

	Consultation questions - challenging technologies, conditions and evaluations	Comments
	• 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?	
3	What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	n/a
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?	n/a
5	General comments: If you have additional comments on this section please share them here:	n/a

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?	n/a
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?	n/a

	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?	n/a
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?	n/a
5	General comments: If you have additional comments on this section please share them here:	n/a

General comments

Please provide any other comments you may have here.

- In general, the proposals lack the requisite detail to allow the Blood Cancer Alliance to respond more fully. We hope there will be opportunity for patients and patient representative organisations to engage in the design of proposed guidance and changes. It is imperative that patient organisations remain well-represented in ongoing working groups. We would also welcome information on when details will be provided and whether there will opportunity for comment at that point.
- The consultation paper suggests that proposals are to benefit patients, and therefore it is very disappointing that little reference is made to how patients will be better engaged in appraisal processes. It may be that this will be worked out in the detail. We urge NICE to engage patient representatives in designing better opportunities for patient engagement and better consideration of patient-generated evidence.
- The Blood Cancer Alliance's recent report on Access to Medicine forms a comprehensive evidence base for some of the proposed changes we have made in this consultation response. It is available here.

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