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 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	Cancer52
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?	We welcome the inclusion of a severity modifier, something which was set out in Cancer52's letter to the NICE Chief Executive. For rare cancer patients who are not facing end of life, this is likely to have a significant impact on medicines being approved. Rare and less common cancers are often more severe than the more common cancers, due to fewer and/or less effective treatment options. We also welcome an increased flexibility around uncertainty. Uncertainties that cannot be resolved, such as due to size of population, are a key challenge for rare and less common illnesses. However, we reserve further judgement on this topic until further details are made available on how this will be implemented. However, we are deeply concerned about the decision to remove the end of life modifier. For many rare cancers, curative treatments are few and far between, and most new medicines coming through the pipeline only provide life-extending benefits. Thanks to personalised medicine, new medicines are for increasingly small patient numbers, and are expensive. However, the replacement of this end of life modifier with a severity modifier up to a value of 1.7x the

	normal ICER threshold could have a significant negative impact on cancer patients at the end of their life. Unless the full 1.7 modifier is guaranteed for all end of life medicines, terminally ill rare cancer could lose out on valuable extra time with their loved ones. Cancer52 was also disappointed that rarity has not been considered as a modifier. The consultation states that this is to "avoid including specific provisions for rare diseases as much as possible". Yet this is exactly what is necessary to achieve equitable outcomes between rare illness and less rare conditions; rare diseases face a number of specific challenges which could be overcome with a rarity modifier. While we appreciate that the Highly Specialised Technology stream is designed to take some of these challenges into account, the stringent entry conditions to this pathway mean that even medicines which will be used by a tiny number of patients are not able to use the increased ICER threshold. Indeed, to our knowledge, no cancer has gone through the HST process, showing that the criteria discriminate against rare cancers. We also welcome the move to reduce discounting to 1.5%. This could mean that curative treatments are not disadvantaged. We understand that there will be concerns from some stakeholders regarding the impact on affordability if this results in more approvals; however, this is mitigated by commitments made to reimburse the NHS in the voluntary scheme on brand medicines pricing and access agreement.

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?	The severity modifier has the potential to benefit rare diseases, which are often more severe. However, we are concerned about the risk of removing the end of life criteria, which benefits cancer treatments that historically had lower levels of approval by NICE, and ask that the risk of disadvantaging these treatments is lessened by including end of life as the maximum possible level of severity.
What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities?	n/a
Do the proposals create any equalities concerns, particularly for NICE's legal responsibilities and the important need to eliminate unlawful discrimination and promote equality?	The removal of end of life criteria is a potential equality issue. This modifier was designed to improve upon historically lower levels of approvals for cancer treatments, and it's removal may therefore discriminate against cancer unless properly incorporated into the severity modifier.
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?	We welcome the commitment to clarify how committees consider other, real world evidence in addition to randomised control trials. This is particularly important for rare cancers, for which small patient populations make gathering a high level of evidence via full trials increasingly difficult. However, we are concerned about the lack of mention of the input of patient group evidence, such as surveys or qualitative evidence in the decision-making process. Patient groups provide a vital link between the scientific evidence and the patients who will ultimately benefit from the medicines, and can give insights into experience of the medicine in question and/or comparators. Whilst the document refers to clarifying use of evidence from expert elicitation, patient groups provide quantitative and well designed and high quality pieces of evidence that should be considered alongside other real world evidence. It must not be forgotten that decisions made by committee ultimately have a human impact. There is also a lack of information surrounding a framework for combining the qualitative and quantitative evidence, which could ensure that patient experience would be given formal weight when making decisions.

		We also welcome commitments to consider alternatives relating to the EQ5D quality of life measure when it is shown to be unsuitable. For many rare cancer patients, this blunt instrument fails to demonstrate the nuances of quality of life. However, further 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? ● 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?	Considering a wider evidence base is likely to reduce uncertainties, especially where large randomised clinical trials are difficult to do due to the nature of the disease, and therefore this will benefit rare disease populations. Real world evidence will also show benefits beyond those captured during trials; patients often report that things they value aren't always captured within these trials. Not formally recognising the evidence generated by patient groups risks frustrating these groups. Significant time and resources go into a submission from a patient organisation and this should have a place in the decision making framework. Not knowing the impact of their evidence is also frustrating for individual patients participating as patient experts.
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:	

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?	A desire for clarity and consistency is at odds with the need to be flexible due to uncertainty from rare illnesses. We would like more information from NICE regarding these conflicting aims. We are concerned about proposals made to only approved treatments in groups which are most cost effective. For rarer cancers, this will only create further uncertainty where patient numbers are already small. Additionally, it risks only making treatments available in at certain lines of therapy; if not approved first line for rare treatments, patients may have to have inferior treatments before they can access something that would be more effective for them if had initially. For rare treatments where there may be no effective treatments, this is a major concern.
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	The proposal to allow committees to optimise recommendations, even where the whole population is cost-effective, risks discriminating against the sickest, by age or discriminating against chronic illnesses needing long term treatment if brought to NICE alongside a more acute subtype of the same illness. This is also at odds with NICE striving to achieve access for full licensed indications where possible and also against its aim to not make decisions on cost alone, which drives this decision. The opportunity cost of funding for the least cost effective groups is outweighed by policy mechanisms for

	patients, science, society and the life sciences industry?	medicines that prevent an increase in spending from impacting the overall NHS budget.
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?	If subgrouping was to be conducted on characteristics more likely to occur in older people (such as high ECOG score and more comorbidities), this has the potential to discriminate against older patients.
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?	We were disappointed that NICE does not consider there to be a case for change in how basket trials are assessed. Given the issues which arose as a result of the first histology independent treatments, and with more of these coming in the future, we expected more concrete changes to the appraisal methodologies available. We are disappointed that there is no case for change for ATMPs, given that these are more likely to There was a disappointing lack of case for change for rare disease, despite evidence of specific challenges for this group. It is more difficult for rare cancers to meet required standards of evidence to be appraised through STA, yet cancers are excluded from HST due to the criteria set. We again call for NICE to consider that rare cancers require special methods in order to achieve equitable access (rather than favour them over non-rare cancers). Overall, we are disappointed that this case for change has been largely based on existing evidence from previous appraisals. Cancer52 and others have regularly called for this review to "future-proof" the process, as well as this being an aim of the review. How can we future-proof if we only look at challenges seen in the past? Newer technologies, including some which we

		have already seen go through the appraisal process, pose challenges we have not yet tried to address in literature.
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?	Basket trials potentially could represent quicker access for patients where trials may not be viable in the small individual populations. Therefore, the methods need to be updated to account for the challenges they create.
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:	

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?	
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?	

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?	The review is designed to align methods across programmes, including the HST programme. However, it does not reference the fact that rare cancers are particularly challenged by the STA process, nor acknowledge the fact that cancers are excluded from HST due to the criteria, such as the need for a specialised centre for care. Rare cancers are falling into the gap between the evidence requirements of STA and HST and this should be further addressed in addition to the proposals outlined in this case for change.
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.

23 members of Cancer52 have also confirmed that they support this submission, in addition to any submission they provide themselves, including: Action Against Heartburn, Action on Womb Cancer, ALK Positive, Anthony Nolan, Blood Cancer UK, Brain Tumour Research, Braintrust, CLL Support, Fight Bladder Cancer, Heartburn Cancer UK, Kidney Cancer Support Network, Leukaemia Care, Lymphoma Action, MDS Patient Support, Mesothelioma UK, Neuroendocrine Cancer UK, OcuMel UK, Ovacome, Pancreatic Cancer Action, Sarcoma UK, Target Ovarian Cancer, The UK Mastocytosis Support Group, Womb Cancer Info

We are as yet unsure if these proposals will be sufficient to deal with the challenges for appraising rare and less common cancer medicines via the STA process. The system needs flexibility to ensure equitable outcomes for rare illnesses. If proposals are not sufficient, there will remain a need for a separate process to fill the gap between STA and HST, as called for in the Cancer52 recommendations for the NICE methods review.

Cancer52's report on recommendations for the Methods Review also called for a number of other changes beyond the scope of the review. We are pleased to see topic selection has been consulted on elsewhere; however HST criteria was not considered at same time. There also remains challenges with the patient involvement and impact, which we have outlined in the report. There a number of changes that need to be made to the process to improve patient involvement in the appraisal processes, alongside some methodological changes that we have called for above. We hope that the forthcoming process review considers these fully. There have been recommendations made following patient involvement workshops and patient involvement working groups previously that have not been implemented and we would like to see these changes made, both here where it applies to methods and also in future process reviews.

Finally, it is not clear about the impact of the changes to the handling of uncertainty on the use of CDF. We have raised concerns in our previous reports that patients and patient groups are not involved in the discussions around the use of the CDF to resolve uncertainty.

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