## 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 | The Haemophilia Society |
| 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?<br>• 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?<br>• Do you have any comments or feedback on how well the proposals will achieve the aims of the review? | We welcome the intention to review NICE's methods for valuing health technology particularly with a view of accepting and understanding a greater degree of uncertainty. As a charity representing people with bleeding disorders, a set of rare genetic conditions, we hope that these changes will make it easier for new technologies to be adopted which will benefit our members.<br><br>The proposals indicate that a specific modifier to reflect rarity in decision making was considered, however, that a decision was taken to not have "specific provisions for rare diseases as much as possible".<br><br>There are, however, particular challenges faced by treatments for rare diseases that could be ameliorated with changes to the processes through which rare disease technologies are considered.<br><br>We believe that NICE should consider the development of a specific pathway for rare disease technologies, in line with the approach to providing additional flexibility for 'orphan' technologies used by other HTA bodies in France, Germany and Scotland. |

| Consultation questions - valuing the benefits of health technologies | Comments |
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| What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS?<br>• What are the potential benefits of the proposed cases for change?<br>• Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them?<br>• 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 are happy with the proposal to remove the end-of-life modifier and welcome in principle a severity modifier. This should be used to prioritise treatments for conditions for which no effective treatment exists.<br><br>We also welcome an appreciation that a greater understanding and acceptance of risk is required in certain areas. The quality of data for the effectiveness of treatments in rare diseases has, by the nature of the rarity of the conditions, greater uncertainty. Similarly, novel therapies with new modes of action and substantial potentially long-term or life-long effects will carry greater uncertainty and need to be reviewed with this in mind.<br><br>Consideration of how a health inequality modifier could be used could be welcome. It could include a QALY modifier for new treatments for conditions that disproportionately affect historically underserved populations such as women and girls as well as treatments for conditions with higher incidence in, for example, BAME communities. |
| What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities? | Greater use of managed access agreements such as through EAMS could be an effective way of ensuring patient access to new treatments while allowing the collection of additional data and other evidence on the effectiveness of technologies. |

| Consultation questions - valuing the benefits of health technologies | Comments |
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| 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 presumption of the use of EQ-5D in measuring quality of life discriminates against rarer conditions.<br><br>In line with the proposal to consider more patient reported outcomes, NICE should promote the use of high quality, validated disease specific quality of life measures. In Haemophilia, for example, measures such as the Haem-A-QoL and Haemo-QoL-A have been tested and validated.<br><br>These would allow more sensitive comparisons of the outcomes of new technologies in patient populations where effective treatments already exist, but new technologies could improve outcomes, reduce the burden of treatment or lead to cost savings.<br><br>The EQ-5D reference cases do not work as well for rare diseases in general and may require adjustment, particularly if it is NICE's intention to continue to compare treatments from different disease areas. However, The Haemophilia Society believes that comparisons between disease areas are more fundamentally flawed and should be avoided.<br><br>Disease specific quality of life measures could help to better ascertain people's expectations, they should show not only what people can do but what they could do. |

| Consultation questions - valuing the benefits of health technologies | Comments |
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| | The existence of a 'Disability paradox' has been shown in haemophilia wherein people with haemophilia value a given health state more highly than the general population. (https://abstracts.isth.org/abstract/examining-the-hemophilia-disability-paradox/) This means that improvements in quality of life that new treatments present may be being undervalued by NICE analyses. |
| General comments: If you have additional comments on this section please share them here: | In general the focus of these proposals on seeking and reviewing more information and requiring more data could come into conflict with NICE's aims to review more technologies quicker. It is not appropriate for NICE to be the limiting factor in providing access to new technologies and adding additional delays to approvals. Questions of affordability are already decided by NHS England and the total pharmaceutical bill is controlled through statutory and voluntary schemes such as the VPAS.<br><br>The proposals also seek to consider the NICE HTE methods without considering the wider context in which they sit. We could welcome greater clarity on the purpose of the assessments. Are they designed to compare technologies with other new technologies and/or existing treatments? Or is the purpose of them to decide whether a given technology is value for money in its own right? |

| Consultation questions - valuing the benefits of health technologies | Comments |
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| | Without a clear statement of what an assessment means for commissioning decisions by NHS England it is not possible to fully comment on the proposals in the round. However, we are able to comment on aspects of the proposals in a vacuum and have done so in our response.<br><br>Finally, it is unclear if the proposals will be fit for purpose if the total number of technologies due for review increases substantially in the next few years. |

### *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?<br>• 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?<br>• Do you have any comments or feedback on how well the proposals will achieve the aims of the review? | NICE should make it clear how it plans to consider and value real-world evidence and patient-reported outcomes. Patient preference goes beyond clinical effectiveness to include reductions in pain and the burden of treatment as well as improvements in other aspects affecting quality of life such as ability to engage in education, work, hobbies and sports and the effect on mental health.<br><br>NICE should work with people with bleeding disorders to establish what outcomes are most important to them and ensure these outcomes are appropriately valued in technology evaluations. |
| 2 | What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS?<br>• What are the potential benefits of the proposed cases for change?<br>• Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them?<br>• Do you have any comments or feedback on how well the proposed methods will support innovation for | The proposals suggest a clearer and larger role for real-world evidence and patient-reported outcomes. NICE should explain what it sees as the strengths of this evidence and how it will ensure it is considered in their decision-making.<br><br>Currently, there is no effective framework for considering this evidence and valuing it alongside evidence from RCTs. For conditions where effective treatments already exist greater importance needs to be given to real-world evidence that compares new treatments to the current standard of care rather than to no treatment. |

| | Consultation questions - understanding and improving the evidence base | Comments |
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| | patients, science, society and the life sciences industry? | The proposals suggest that probabilistic analyses should be conducted rather than deterministic analyses for economic analyses. This can cause problems for rare conditions where variability may be higher and particularly for very rare conditions where a deterministic analysis could be better.<br><br>These changes will increase the burden on companies and patient groups to produce more data and conduct more complex, costly and time-consuming analyses. The cost of bringing a new technology to NICE for evaluation is now estimated to be around £1 million, including the production of submission and analysis as well as NICE fees. We are considered that this situation may deter smaller pharmaceutical companies, such as small UK-based gene therapy companies from launching new products in the UK. This feels at odds to the Government's Life Sciences Industrial Strategy. |
| 3 | What are the potential implications of the proposed changes for other NICE guidance and advice, and for other NICE programmes and activities? | NICE should ensure that companies with products in the pipeline are aware of its intention to value PROs more highly. It should help them ensure that such outcomes are included in their phase 2 and 3 clinical trial outcome measures. The proposals should be incorporated into guidance from NICE's Office for Market Access. |
| 4 | Do the proposals create any equalities concerns, particularly for NICE's legal responsibilities and the | The proposals intend to better capture and assess uncertainty. This is a laudable aim but may disadvantage treatments for rare |

| | Consultation questions - understanding and improving the evidence base | Comments |
|---|---|---|
| | important need to eliminate unlawful discrimination and promote equality? | diseases which will have higher levels of uncertainty due to the smaller populations of affected individuals.<br><br>NICE must be careful that, in making clearer consideration of uncertainty they are not disadvantaging technologies for small patient populations. |
| 5 | General comments: If you have additional comments on this section please share them here: | NICE should also consider how it can better use evidence from trials or real-world evidence from other countries. Instead of the current bias against evidence from outside the UK, NICE could work more closely with it's counterparts in other parts of Europe as well as ICER and CADTH in North America to broaden the range of evidence, including unpublished evidence, it considers. |

### *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?<br>• 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?<br>• Do you have any comments or feedback on how well the proposals will achieve the aims of the review? | More emphasis should be put on helping patients and patient groups to engage with NICE evaluations. NICE should provide more resources to patient groups and support them with more information and clarity on what input and evidence is most useful from them. |
| 2 | What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS?<br>• What are the potential benefits of the proposed cases for change?<br>• Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them?<br>• 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 have concerns about the proposal to restrict access to certain subgroups of patients in cases where the technology has been shown to be cost-effective for the whole population. This could lead to some patients being denied access to what NICE has determined is a cost-effective treatment. |

| | Consultation questions - structured decision making | Comments |
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| 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 |

### *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?<br>• 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?<br>• Do you have any comments or feedback on how well the proposals will achieve the aims of the review? | We welcome plans to review methods to ensure they are appropriate for new technologies such as gene therapies.<br><br>However, we believe there remains a risk that patients with rare diseases will continue to be disadvantaged if certain changes are not considered. There remains a gap between the STA and HST processes that many rare disease treatments fall into. NICE should consider appropriate modifiers to improve access to rare disease treatments. |
| 2 | What are the potential effects of the proposed changes on patients and their families, health technologies, the life sciences industry and the NHS?<br>• What are the potential benefits of the proposed cases for change?<br>• Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them?<br>• 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 - challenging technologies, conditions and evaluations | 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 |

### *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?<br>• 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?<br>• 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?<br>• What are the potential benefits of the proposed cases for change?<br>• Are there any risks that might arise from adopting the proposals? If so, how might we try to reduce them?<br>• 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: | It is unclear whether it is suitable to have one set of guidance that applies to all four of the processes considered in this review. NICE should keep the four processes distinct and only align the systems where it is appropriate to do so. |

### *General comments*

Please provide any other comments you may have here.

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| A reduction in the discount rate to 1.5% is broadly welcome as it would reduce the current undervaluing of longer-term benefits of treatments. However NICE should look again at whether differential rates could allow more appropriate treatment of the longer term cost compared to longer term benefits. |

## 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