NICE National Institute for Health and Care Excellence

Health technology evaluations: interim methods and process guide for the proportionate approach to technology appraisals

Process and methods

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1 Introduction

1.1 This guide describes the interim methods and process changes that NICE has implemented as a result of the proportionate approach to technology appraisals work done between April 2022 and March 2023. It should be read alongside the relevant sections of the NICE health technology evaluations manual.

1.2 Taking a proportionate approach to technology appraisals allows NICE to increase our capacity and produce more guidance while also ensuring time and resource savings for our partners and stakeholders, such as patient and public involvement groups. It also enables faster access to some medicines for patients. For those working in industry, it means a simpler, faster evaluation process for certain treatments.

1.3 This guide outlines the changes made to:

- scoping
- scheduling of topics
- handling of confidential information
- the cost comparison process
- topic progression after evidence critique
- committee decisions outside of formal meetings.

1.4 The changes outlined will be monitored and reviewed. The outcomes will be used to inform the final modular update to the NICE health technology evaluations manual.

2 Scope

2.1 Consultation on the draft scope: identifying cost comparison topics

2.1.1 At scoping consultation, stakeholders will be asked questions relating to the population, treatment pathway, benefit and clinical similarity to help establish the case for cost comparison. The aim is to establish if the intervention is likely to be clinically similar, so it can be compared using cost comparison methods with another intervention that NICE has previously recommended for the same indication. The chosen comparator must be established in practice and have substantial use in the NHS in England for the same indication.

2.1.2 The draft scope sent out at consultation will indicate if NICE is considering cost comparison as a possible process for the evaluation.

2.1.3 During scope consultation, NICE's medicines optimisation team will engage with medicines and prescribing associates to create a briefing report on the appropriateness of cost comparison. This report will be published alongside topic information on the NICE website.

2.1.4 The scoping consultation will enable NICE to decide if the cost comparison process is suitable, taking into account input from stakeholders. If it is established that cost comparison is appropriate, NICE will invite stakeholders to make a cost comparison submission. If cost comparison is not appropriate stakeholders will be invited to submit to a single technology appraisal. This decision will consider relevant risks associated with the appraisal and the decision to use cost comparison.

2.1.5 All other elements of the scoping phase remain as described in section 2 of the NICE health technology evaluations manual.

3 Developing guidance

3.1 Evaluation timelines

3.1.1 Scheduling of topics into the NICE work programme will be managed using information on expected regulatory approval dates and submission readiness which will be provided through horizon scanning and topic selection activities and directly to NICE by the company.

3.1.2 Topics may benefit from aligned internal processes when they are in the same disease area, follow similar regulatory timelines and are scheduled into the same (or closely aligned) committee meeting.

3.1.3 When appropriate, NICE may decide to hold a joint committee meeting covering more than 1 appraisal. A joint committee meeting is when 2 separate topics are discussed in 1 committee session. Confidentiality will be strictly preserved; the topics will remain as separate appraisals and recommendations will be made individually for each appraisal.

3.2 Handling confidential information

3.2.1 Section 3.2 of this interim guide supersedes section 5.4 of the NICE health technology evaluations manual on the handing of confidential information for medicines only. All other elements of handling confidential information remain as described in section 5.3 of the manual.

3.2.2 See the principles for confidential information marking and redaction for further information and best practice in marking and redacting documents. This also outlines the additional descriptive reporting requirements for data that is to be redacted, to ensure transparent reporting of committee decision making. It provides advice and information for stakeholders but is not a formal process guide.

3.2.3 To ensure that the evaluation process is transparent, it is essential that as much of the evidence as possible informing the committee's decision

making is made available to stakeholders and is publicly available. In some circumstances, NICE will accept evidence and information not in the public domain under agreement of confidentiality. The NICE health technology evaluations manual defines 3 categories of confidential information: commercial-in-confidence, academic-in-confidence and depersonalised data. Academic-in-confidence is not used for medicines evaluated through the technology appraisals or highly specialised technologies programmes.

3.2.4 There are broad categories of data and information which are redactable and non-redactable. Evidence and information not in the public domain that may be redacted includes:

- Evidence that is commercially sensitive, such as confidential price discounts and confidential information on market share. Also, data that allows back calculation of commercially sensitive data (see section 3.2.11 for more information on confidential price discounts).
- Clinical data that has not been made publicly available and for which there is no plan for the data to become publicly available. The reason for redacting this data should be explained and consideration should be given to the expected impact on NICE's ability to explain the evidence on which the committee's decisions are based to stakeholders and the public (see section 3.2.6).
- Data provided to the stakeholder submitting to NICE by a third-party organisation, if there are stipulations from the third-party organisation on how the data may be disseminated by the stakeholder. This may include, for example, registry data or data from a trial when the stakeholder is not the sponsor. In these cases, the redaction stipulations of the third-party organisation will be adhered to.
- Data that allows for subject identification, including depersonalised data (data that is stripped of direct identifiers) but is still at high risk of subject identification.

3.2.5 Categories of information that cannot be redacted include:

- Methods used to conduct a study or to analyse data from a study.
- Clinical data that is available in the public domain.

- Clinical data awaiting publication, including in a journal or in documents supporting authorisations by regulatory agencies that are released at the time of marketing authorisation.
- Data collected within NHS clinical practice as part of a managed access agreement cannot be considered confidential unless it meets other criteria (for example, allows for subject identification).
- Critical appraisal of clinical studies and indirect comparisons.
- Clinical opinion and assumptions (which are not based on empirical data).

3.2.6 Information marked as confidential should be kept to an absolute minimum and reasons for confidentiality must be stated clearly. Marking must allow evidence and information that is likely to be fundamental to the committee's decision making to be sufficiently explained to stakeholders and users of NICE guidance.

3.2.7 Data that is likely to be fundamental to committees' decision making includes:

- Cost effectiveness (incremental cost effectiveness ratio [ICER]) or cost comparison (incremental cost) estimates.
- Data informing the case for decision modifiers to be applied in technology appraisals and highly specialised technology evaluations.
- Evidence allowing consideration of items listed in section 6.2.28 of the health technology evaluations manual. Primarily, the generalisability, reliability and robustness of evidence informing an evaluation and plausibility of assumptions or model outcomes.

  NICE recognises that some of this evidence may fall under the categories of redactable data in section 3.2.4, such as:

- data allowing back calculation of a confidential price discount (for example, the price related to a patient access scheme [PAS], a commercial access agreement, or from the Commercial Medicines Unit).

- clinical data not intended for publication.

  In most instances in which the stakeholder considers it necessary to mark this data as confidential, as a minimum, an accompanying descriptive summary of what the data shows must be provided so that NICE can explain committee decision making to stakeholders and the public. There are instances in which numerical data rather than a descriptive summary is needed to explain committee decision making. This includes data informing the case for decision modifiers and health-state utility values, on-treatment utility increments or decrements, and utility decrements associated with adverse events. In these cases, numerical values should be shown. New flexibility on the redaction of ICERs has been introduced to prioritise the transparency of these numerical values.

3.2.8 There are instances in which the exact decision-making ICER, or incremental costs in cost comparison analyses, cannot be published in NICE documents or in public committee meetings. This includes when there are confidential patient access schemes for combination treatments, comparators and subsequent treatments. In these cases, NICE will state in its public committee meetings and post-meeting documents whether the values are above or below a level at which the technology may provide value for money. Given the high proportion of evaluations in which this is the case, NICE will consider this approach across all technology appraisals and highly specialised technologies evaluations. This means that there is flexibility allowing redaction of ICERs and incremental costs if:

- there are confidential patient access schemes for combination treatments used alongside the intervention under evaluation, comparators or subsequent treatments
- a new confidential price for the intervention under evaluation is expected or the confidential price is expected to change over the course of the evaluation, and reporting of results including different prices will allow calculation of the final confidential price
- a case for a severity modifier is being made

- it allows utility values to be transparent.

  When ICERs are redacted, incremental quality-adjusted life years (QALYs) should not be redacted.

3.2.9 If NICE wishes to publish or publicly share data regarded by the data owner as confidential, both NICE and the data owner will negotiate to find a mutually acceptable solution. This will recognise the need for NICE to support its recommendations with evidence and the data owner's right to confidentiality. The need for negotiation is considered exceptional when the principles for confidential information handling are adhered to. The data owner retains the right to make a final decision about the release of confidential information into the public domain.

3.2.10 NICE could be challenged that confidential information it has received should be publicly released in the interests of fairness during an evaluation, at appeal or resolution, through judicial review or otherwise. If this happens then data owners must, on request, promptly reconsider whether it is necessary to maintain confidentiality. If disclosure is not possible, the data owner must be prepared to assert publicly that the information is confidential and must submit evidence justifying why NICE should maintain that confidentiality. Without such assertion and evidence, NICE is entitled to conclude that the information is no longer confidential.

3.2.11 Details of a PAS, once referred to NICE for consideration in an evaluation, are not confidential except when NHS England has agreed that a simple discount PAS is confidential. All other types of commercial access agreements, once referred to NICE for consideration in an evaluation, are confidential. In these cases (as outlined in section 3.2.4), the discount and any data that could lead to back-calculation of the discount will not be shared with stakeholders or released into the public domain.

3.2.12 When the details of the PAS are not published in final NICE guidance, the NHS must have access to the details. This is so providers and commissioners can properly account for the PAS and commercial agreement. Details of commercial access agreements will not be published in final guidance. When elements of a commercial access agreement need to be known to the NHS for the agreement to be

operationalised, the NHS must have access to the details.

3.2.13 NICE will not share details of confidential price discounts for other medicines with the company for a new technology being evaluated. For each medicine, and for each indication included in the treatment pathway, the company must include a 'discount' field in its economic model. This should allow the user to input any value between 0% and 100%, which is then applied as a percentage discount to the list price of the medicine. By providing this feature in its model, the company will be responsible for the initial programming, which the external assessment group (EAG) will check. All parties should then be confident that the discount is programmed correctly. The EAG will be authorised to know the exact level of discount for commercial arrangements in the evaluation.

3.2.14 The EAG will use the list price, or an alternative publicly available price such as the eMIT price, for any other technologies with confidential price discounts in its external assessment report when reproducing the company's analyses and for any exploratory analyses. To allow the committee to explore the effect of using the actual cost of the technologies in the analyses, the EAG will also create a confidential appendix to its report. This will reproduce all analyses from the external assessment report using the exact level of discount. When the results of the EAG analyses are classed as confidential because of existing confidential commercial mechanisms, including PAS and commercial access agreements, NICE will state whether the ICERs are above or below a decision-making threshold in its public committee meetings and post-meeting documents (see section 3.2.8).

3.2.15 Executable economic models used in the evaluation will be made available on request to stakeholders who have signed a confidentiality agreement.

3.2.16 Committee and EAG members attending the committee meeting will be provided with all confidential information submitted.

3.2.17 The clinical and patient experts who attend the committee will be provided with all confidential information submitted, except confidential

PAS for combination treatments, comparators and subsequent treatments, and commercial access agreements (or other similar confidential price arrangements).

3.2.18 In committee meetings, confidential information will be redacted from the slides. Committee and EAG members, clinical and patient experts and company representatives will also be given an unredacted version of the slides presented in the public part of the meeting. When necessary, for appraisals in which more than 1 technology is being evaluated, NICE may agree with the relevant data owners additional arrangements for handling clinical data not intended for publication during public meetings, to allow effective and transparent discussions.

3.2.19 If a technical engagement happens, all information marked as confidential will not be released to stakeholders even though they have signed a confidentiality agreement. Patient, clinical and commissioning experts will be able to see unredacted documents.

3.2.20 If an evidence submission or a statement from a non-company stakeholder contains confidential information, it is the responsibility of the submitting organisation to provide 2 versions:

- A version for NICE, the committee, the EAG and the NHS England clinical leads and commissioning experts with all the confidential information marked with turquoise highlighting and underlined.
- A version in which all the confidential information is redacted.

3.2.21 The stakeholder must complete a confidential information checklist at the time of submission. This should list all confidential information included in the submission or statement and the reason for its confidentiality. If NICE does not receive a completed checklist with a document, none of the information will be considered confidential.

3.2.22 Data owners may be asked to check that confidential information is correctly marked in documents created by others in the evaluation before release.

3.2.23 NICE releases all documents that are presented to committee in

committee papers to stakeholders during the evaluation. NICE publishes these documents on its website within 7 days after they have been sent to stakeholders. After NICE has published these documents on its website, they are no longer confidential. Confidential information within published documents is redacted.

3.3 Evidence submission: cost comparison

3.3.1 For topics identified as cost comparison, submissions should be made using the cost comparison submission template. Submissions made using the standard single technology appraisal template after cost comparison has been referred will be rejected and the topic may be delayed.

3.3.2 For cost comparison appraisals, the deadline for receipt of the evidence submission is 28 days from the invitation to participate.

3.3.3 During a cost comparison appraisal, the selected clinical and patient experts will not be asked to complete a formal statement or attend a committee meeting. Experts are requested to be available to answer questions that may arise during the appraisal process.

3.4 Evidence review: cost comparison

3.4.1 For cost comparisons, the EAG prepares an evidence assessment report based on a proportionate review of the company's evidence submission.

3.5 Topic progression: single technology appraisal

3.5.1 After receiving the external assessment report, NICE will assess the evidence submissions and report and decide how the appraisal will progress. At this stage an appraisal can:

- Continue as a single technology appraisal and progress to preparation for a committee meeting.

- Continue as a single technology appraisal and progress to technical engagement before preparation for a committee meeting.
- Be appropriate for a streamlined committee decision process in selected low-risk circumstances, with committee decision outside of a formal meeting.
- Pause while NICE considers the most efficient and appropriate course of action (see section 5.5.30 of NICE's health technology evaluations manual).

3.5.2 Technical engagement will only be included if NICE considers that it is appropriate, helpful and proportionate. NICE will take into account whether the technical engagement process is likely to resolve key issues before the committee meeting.

3.5.3 If technical engagement is included timelines will be amended to allow for engagement time with stakeholders.

3.5.4 NICE will make decisions about whether to streamline topics into a committee decision outside of a formal meeting.

3.5.5 When deciding on the suitability for streamlined decision making, NICE will take into account the risks associated with the appraisal and the decision to streamline. This may include:

- The likelihood of decision error in the guidance, and its consequences.
- The complexity of the technology, clinical pathway or evidence, and associated uncertainties.
- The potential impact of the decision to streamline on:
  - resources for NICE, committees and stakeholders
  - service readiness
  - consistency and predictability of NICE decision making
  - openness and transparency in decision making.

3.5.6 The progression decision and relevant timelines will be communicated to stakeholders within 2 weeks of receipt of the external assessment report. Information will also be published on the NICE website once

stakeholders have been informed.

3.6 Evaluation

Changes to committee decision making outside of formal meetings

3.6.1 For cost comparison appraisals and those streamlined for committee decision outside of a formal meeting, a subset of the committee will review the evidence. It will be able to make a recommendation outside of a full committee meeting.

3.6.2 Experts who have been selected to take part in the appraisal or other members of the committee may be invited to contribute on a case-by-case basis. This will happen if, in the opinion of the committee subset or the NICE team, they are needed to address specific questions.

3.6.3 If the subset of the committee concludes that it cannot make a recommendation this will result in a full committee meeting. This will not alter standard governance or appeal processes and maintains the independence of the committee as a decision-making body.

3.6.4 If a full committee meeting is needed then clinical experts, patient experts and non-company stakeholders will not normally be invited to take part in the committee meeting discussion. In some circumstances, the committee chair and NICE may agree to invite clinical, patient or NHS commissioning experts to the meeting to help address specific uncertainties.

3.6.5 Processes following a recommendation decision are unchanged. For information on consultation, appeals and guidance publication, see NICE's health technology evaluations manual.

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