Proportionate approach to technology appraisals: final report 2022–23

April 2023

Applying a proportionate approach

The number, range and complexity of medicines evaluated by NICE is increasing – but not all need the full technology appraisal process. By developing a proportionate approach to technology appraisals, we can meet increasing demand while maintaining a robust, evidence-driven approach.

Through robust, predictable and proportionate evaluations, NICE can continue to support rapid access to clinically and cost-effective technologies for patients and the NHS, while making it easier for stakeholders to contribute to evaluations and focusing on what is most needed.

Using proportionate approaches allows different evaluations to follow paths that match their specific needs. The new approaches continue to include a value signal and access recommendation, and use the same decision-making frameworks for clinical and cost effectiveness as our existing processes.

NICE's proportionate approach to technology appraisals project aimed to increase the capacity for publishing appraisals by 20% from 2023–24 onwards. We developed new approaches using test-and-learn principles, exploring ideas and developing them through direct experience in active health technology evaluations. NICE engaged with stakeholders throughout the project, and incorporated real-time input and feedback throughout.

This final report presents the conclusions from the 2022–23 proportionate approach project, outlines the proportionate approach that will be implemented from April 2023, and identifies key next steps. It is accompanied by an interim methods and process guide.

Streamlined approaches

Recognising that not all medicines need the same depth of evaluation, NICE has identified opportunities to introduce simpler and faster processes for some topics. These streamlined approaches have allowed us to shorten the time taken for evaluation and reduce the overall workload by 25% to 45% for some technology appraisals.

The proportionate approach to technology appraisals project considered 2 different streamlining opportunities: cost comparison appraisals and streamlined decision making.

Cost comparison

The streamlined approach initially focused on new treatments that are similar to treatments already recommended by NICE (for example, new drugs in an existing class). With these appraisals, NICE and the committee already have a good understanding of the disease and the technologies, and the economic analysis can be based on cost comparison methods. NICE's existing cost comparison approach (previously called 'fast-track appraisals') provided a helpful framework, but it was not as efficient as it could be. So, NICE has developed a new streamlined approach for cost comparison appraisals.

The streamlined approach to cost comparison appraisals was piloted in 2 technology appraisals: NICE's technology appraisal guidance on somatrogon for treating growth disturbance in children and young people and vutrisiran for treating hereditary transthyretin-related amyloidosis. In both cases, the technologies were recommended substantially quicker than would otherwise have been possible, while identifying opportunities to improve the process. These opportunities for improvement related to:

- scoping, clarification and guidance production
- approaches for primary care-commissioned technologies
- obtaining additional advice when necessary.

Using feedback and lessons learnt from the pilots, NICE's streamlined approach for cost comparison appraisals shortens the timeline by 45%, to 23 weeks. The main features of this streamlined approach are:

- NICE identifies evaluations that are suitable for cost comparison during the scoping stage, with input from the company, patient groups, clinicians and NICE's medicines optimisation team. This helps ensure early and robust selection of appropriate topics for cost comparison.
- Evidence submissions and academic review are simplified. Patients, patient experts, clinicians and the company provide input, but this input is more proportionate to what is needed to support a recommendation.
- Recommendations are made by a subset of the committee outside of formal meetings, with the option to seek relevant advice from experts or other committee members. This streamlines the decision-making process and allows the committee time for topics that need further deliberation, while maintaining sufficient input to ensure that a robust decision is made.

Streamlined decision making

We identified opportunities to use a streamlined decision-making approach for more technology appraisals beyond those suitable for cost comparison. This applies to evaluations that are considered lower risk for patients, the NHS, stakeholders and NICE. When the risks associated with a technology or its evaluation are lower, a streamlined approach is more proportionate.

Unlike cost comparison appraisals, a full view of the clinical and economic evidence is needed before a technology can be identified as being suitable for this streamlined decision-making approach. This needs to take into account any uncertainties, the patient and clinical perspectives, and the risks associated with the appraisal.

We take a broad view of the risks associated with the appraisal at a new topic-progression decision stage. After this, a streamlined decision-making process is used, which follows a similar pattern to cost comparisons; that is, a subset of committee members deliberate and decide outside of a formal meeting.

This approach creates efficiencies for both NICE and external stakeholders, while remaining robust, retaining proportionate stakeholder participation and speeding up the evaluation and access to the technology.

This streamlined decision-making approach has been piloted in 3 technology appraisals: NICE's technology appraisal guidance on nintedanib for treating idiopathic pulmonary fibrosis, eptinezumab for preventing migraine and nivolumab for neoadjuvant treatment of resectable non-small-cell lung cancer. These technologies were recommended 8 to 9 weeks faster than through the usual process. The streamlined decision-making approach should reduce the time in evaluation by 25% to 30 weeks.

The main features of this streamlined approach are:

- Assessment of evidence submissions and academic review.
- NICE considers the risks and decides on a topic's suitability for streamlined decision making, with a built-in fail-safe mechanism when needed.
- A subset of the committee, with appropriate advice as needed, decide on any recommendations outside of a formal meeting.
- Less time spent in appraisal, rapid guidance publication and faster access for patients.

We are exploring through another pilot whether this approach is suitable for technologies likely to have a managed access recommendation and which are otherwise suitable for streamlined decision making.

Operating efficiently

Alongside the streamlined processes, the project identified additional efficiency improvements relating to how NICE manages evaluations. These improvements are within the scope of established methods and processes, so minimal changes are needed to the published manual for health technology evaluation.

We have identified 3 areas for efficiency improvements:

- technical engagement

- paired appraisals
- handling confidential information.

Technical engagement

The technical engagement process is a helpful tool for some evaluations but is not always necessary or proportionate. The updates to the health technology evaluation processes published in 2022 emphasised that technical engagement is optional. But in practice, all evaluations scheduled since 2022 have included technical engagement by default, and this step has only been removed in a small number of cases. This means that some evaluations may have included this step when it was not necessary or proportionate. Time spent in evaluation would have been longer than necessary, and patients would have had to wait longer for access.

So, we have refined our processes so that technical engagement is included only when it is helpful to committee decision making. The decision to include technical engagement will be made by NICE, based on evidence and academic review, and made during the topic-progression decision stage used in the streamlined decision-making process. Technical engagement will be added when it can improve rapid and efficient decision making for that evaluation, which will mitigate the modest timeline extension that will be needed to accommodate it, thereby retaining rapid access to clinically and cost-effective medicines. In this way, NICE will ensure the technical engagement process step is used proportionately, retaining it where it is valuable but shortening the length of appraisals and reducing the accompanying burden on NICE, committees, academic groups, companies, stakeholders and experts where it is not needed.

Paired appraisals

NICE sometimes works on multiple appraisals in the same disease area with similar timelines (most often in pairs). In such cases it may be appropriate to align the evaluation timelines. In the past this has been managed informally, but this has led to additional challenges and missed opportunities for efficiency.

So we have developed improved internal approaches to manage topics in the same disease area with similar timelines when they appear within the work programme. These include, for example, changes to how resources are allocated and internal project timings. Each evaluation continues as an individual, standalone single technology appraisal, following the processes in NICE's health technology evaluations manual.

This paired appraisal approach is being piloted in empagliflozin for treating chronic heart failure with preserved or mildly reduced ejection fraction [ID3945] and dapagliflozin for treating chronic heart failure with preserved or mildly reduced ejection fraction [ID1648]. We have noted concerns from stakeholders about these evaluations, although many of the concerns relate to inherent issues associated with appraising 2 technologies with similar timelines. These issues would have arisen even using previous informal approaches. We have refined our approach based on the pilot and stakeholder feedback, and further refinements will be made over time. Overall, the pilot topics showed that approaching paired appraisals in this way provides small but valuable improvements to efficiency.

Confidential information

NICE must ensure that evaluations are transparent and that confidential information is redacted when necessary. This creates a substantial administrative burden for all parties involved. Also, updated guidance from the International Committee of Medical Journal Editors (ICMJE) states that presenting information in health technology evaluations does not preclude publication in affected journals. This means that academic-in-confidence marking for data awaiting publication is not necessarily needed. This provides an opportunity to refine how confidential information is handled.

NICE has partnered with the Canadian Agency for Drugs and Technologies in Health (CADTH) and the Institute for Clinical and Economic Review (ICER) to develop a joint position statement on confidentiality of clinical evidence. NICE has also explored opportunities to improve efficiency in the confidential marking process while maintaining efficient access to data with appropriate confidentiality safeguards.

Having applied the principles in the joint position statement and streamlined the confidential marking process, NICE's interim principles for marking confidential information provide greater clarity on appropriate confidentiality marking and remove the need to use the academic-in-confidence category of redactable data for medicines evaluated through the technology appraisal and highly specialised technology programmes. Our approach retains the critical importance of appropriate confidentiality protection and timely access to data while reducing the administrative burden for NICE and its stakeholders.

Exploratory findings

The project explored 2 other aspects of a proportionate approach to technology appraisals: pre-specifying assumptions and pathway appraisals.

Pre-specifying assumptions

Given that NICE committees often have considerable past experience that is relevant to evaluations, we explored whether particular assumptions could be pre-specified at the start of an evaluation. This would save unnecessary repetition for both stakeholders and NICE. But a retrospective pilot rapidly identified that this would not be feasible: there was not enough consistent precedent to establish pre-specified assumptions, and assumptions that might theoretically be suitable for pre-specification would not save significant time in the evaluation. Furthermore, significant resource would be needed to establish and maintain pre-specified assumptions, which could have been contrary to the aims of the proportionate approach. So, NICE did not develop a pre-specified assumptions approach but noted several recommendations for future consideration.

Pathway appraisals

Around 40% of NICE's technology appraisal guidance relates to only 10 disease areas. This presents an opportunity for substantial economies of scale. Furthermore, by consolidating appraisals in a disease area, NICE can improve how it presents guidance, reflect disease pathways more dynamically and make innovative use of real-world evidence.

NICE is developing an approach based on ongoing evaluations spanning several technologies across a disease area, using a pre-built economic model. This approach represents a significant departure from the current single technology appraisal model and requires longer-term development. NICE is piloting this approach for technologies in renal cell carcinoma and non-small-cell lung cancer. These pilots will continue in 2023–24.

Measuring performance

Until 2022–23, NICE reported on the performance of the technology appraisal and highly specialised technologies programmes using 3 key performance indicators (KPIs). These were reported directly to the Department of Health and Social Care, and to stakeholders and the public in the integrated performance report in the public board papers. But the KPI framework restricts how transparently NICE can report on the work programme for stakeholders and the public, because many guidance publications are affected by factors outside of NICE's control and are excluded from the reporting.

Introducing a proportionate approach provides an opportunity to rethink how NICE structures and reports on the programmes. The KPI framework for topics for which final guidance publishes in 2023–24 will provide more informative data on how the programmes are performing. This will give stakeholders and the public a clearer picture of how NICE is supporting rapid, evidence-based access to clinically and cost-effective technologies.

Project outcomes

Current predictions suggest that by applying a proportionate approach, NICE will increase capacity for health technology evaluations by approximately 17%. This represents a substantial efficiency improvement, both in terms of capacity and the time spent in evaluation. The efficiency improvement is expected to be sufficient to cover the increasing demand for technology appraisals. Efficiencies include:

- Shorter timelines and reduced resources for streamlined cost comparison appraisals (forecasted 15 to 20 appraisals over 2023–24).

- Reduced workload for evaluations that use streamlined decision making (a further 15 to 20 appraisals over 2023–24).
- Smaller operational efficiencies in paired appraisals of technologies in the same disease area with similar timelines.
- Additional operational efficiencies through changes to technical engagement and confidential information handling (unquantified).
- Longer-term efficiencies through pathway appraisals.

These efficiencies will ensure that we can continue to deliver a full programme of high-quality guidance and rapid access as demand grows.

Stakeholder feedback throughout the project has been broadly supportive and positive. Companies have welcomed opportunities to engage with the project and individual pilots, and NICE has rapidly acted on feedback, taking learnings forward to shape and improve future processes. Further engagement and feedback is ongoing and will inform implementation of the approaches.

Next steps

Implementation

These proportionate approaches will be rolled out for technology appraisals and highly specialised technologies evaluations starting after publication of the interim methods and processes guide (April 2023). When appropriate, streamlined decision making may be considered for topics that started before this point, on a case-by-case basis and in discussion with relevant stakeholders. Rollout will be accompanied by a broad range of implementation activities.

The future

Following interim implementation of these proportionate approaches, NICE will formally incorporate them into the manual for health technology evaluations through a modular update. This process will include appropriate engagement and consultation with stakeholders.

In anticipation of further demand for NICE technology appraisal guidance and as part of continuous improvement, NICE will continue to explore further ideas and proportionate approaches to health technology evaluations. These may include:

- Continuing the pilots for pathway appraisals.
- Completing the streamlined decision making for managed access pilot, and exploring in partnership with NHS England whether there are further opportunities to expand on ambitions for rapid entry to managed access for medicines that are highly likely to require this route.
- Further opportunities for streamlined and proportionate approaches (such as process options for evaluating technologies with multiple indications).