National Institute for Health and Care Excellence

Review of the health technology evaluation processes

This report gives details of the proposals for change to the processes used for health technology evaluation at NICE.

The Board is asked to review the report and approve the proposals for public consultation.

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Introduction

This document is the 3rd in a series of consultations regarding the future of topic selection, methodologies and processes for health technology evaluation within NICE.

Ensuring rapid access to clinically and cost-effective health technologies is critically important to patients and their families, the NHS and the life sciences industry. For more than 20 years, NICE’s health technology evaluations have played a key role in supporting access to medicines, medical technologies and diagnostics, guided by the [NICE charter](https://www.nice.org.uk/about/who-we-are/our-charter) and [NICE principles](https://www.nice.org.uk/about/who-we-are/our-principles). To continue to support the needs and objectives of all parts of the healthcare and life sciences ecosystem, NICE must ensure that its processes of health technology evaluation maintain and improve upon key objectives regarding quality, dependability, speed, flexibility and cost.

NICE guidance both informs shared decision making and shapes how companies put forward their technologies. NICE has an opportunity to continue supporting a productive relationship between the NHS and the life sciences industries and to help align their ambitions with the expectations that the public has of the NHS. In this way, NICE can help ensure that the NHS gets the best value out of the products it uses while supporting the life sciences industry in its journey through regulation, health technology evaluation and patient access. Changes to NICEs processes in isolation will not overcome all the challenges for accessing new health technologies in the NHS, in terms of how they are developed, evaluated and supported for adoption. But they present an important opportunity to build on NICE’s success to date, and further support patients, the life sciences industry and the NHS now and in the future.

Following the end of the Transition Period after having left the European Union, the UK is developing a new regulatory and access environment with the life sciences sector. As events unfold, it is important to clearly show how NICE’s processes can continue to support early patient access and encourage companies to launch their products first in the UK.

A process can be described as a sequence of steps required to take an input and transform it into an output. NICE formally publishes process guides and other documentation so that stakeholders and users of NICE guidance can have confidence in our ability to consistently provide products and services that conform to agreed standards and requirements.

It is important that we continue to build upon the success of the health technology evaluation programmes over the past 20+ years. We continue to support real patient access by developing positive recommendations for the majority of the technologies we appraise (>80% for 979 individual recommendations since 2000) and we provide answers close to when the health service needs them (within 90 days from marketing authorisation for pharmaceuticals, and often much closer to it). We can only continue to achieve these outcomes in the future if our stakeholders are willing to work with us to speed up processes, make them more efficient and responsive to the needs of all involved.

The current processes, on which the proposals for change build, can be found on the NICE website, in the:

* [guide to the processes of technology appraisal 2018](https://www.nice.org.uk/process/pmg19/chapter/foreword)
* [medical technologies evaluation programme process guide](https://www.nice.org.uk/process/pmg34/chapter/introduction)
* [highly specialised technologies guidance](https://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-highly-specialised-technologies-guidance): the interim process guide (PDF) can be found under ‘useful documents’
* [diagnostics guidance](https://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-diagnostics-guidance): the programme manual (PDF) can be found under ‘how we develop guidance’.

Why review the processes?

Our overarching goal for considering changes to NICE’s health technology evaluation processes is to improve the health of people using the NHS and social care system, and in doing so support equitable access for people in greatest need and secure good value to the NHS.

NICE’s health technology evaluation processes should be fast, flexible and responsive, to support rapid patient access to clinically and cost-effective health technologies in the ever-changing health and care landscape. Flexible and adaptable processes should provide the opportunity for stakeholders to embrace the variety of options to drive faster decision making. Achieved through less resource intensive processes supporting rapid access and implementation of beneficial new technology.

Changes to NICE’s health technology evaluation processes should:

* support patients and the NHS in accessing clinically and cost-effective health technologies,
* align with changing Medicines and Healthcare products Regulatory Agency systems and processes
* support the attractiveness of the UK as a first-launch country for important and promising new health technologies, to ensure that people can access them as early as possible,
* facilitate equitable consideration of commercial and managed access flexibilities, and recognise and respond to experience with the Cancer Drugs Fund and other commercial and managed access arrangements,
* enable the technological change and innovation that the UK wants to encourage and support, by ensuring fair, robust and predictable evaluations across new and existing technology types,
* sustain NICE’s reputation as a world leader in health technology evaluation.

The proposals included within this paper reflect NICE's emerging organisational strategy. One of the four key priorities of this strategy is the development of 'rapid, robust and responsive technology evaluation'. Implementation of this strategy will require us to focus on investing in the path towards creating final guidance as an output of a collaborative process. A comprehensive package of support for stakeholders is provided through the Office for Market Access, NICE Scientific Advice, Commercial and Managed Access and research facilitation programmes.

These activities are manifestations of health technology assessment moving towards ‘health technology management’, where in an effective HTA ecosystem support for early development, early advice, adoption, real world performance and reassessment and disinvestment are targeted to ensuring that the needs of the patients are front and centre in our activities. A focus on health technology management, rather than just assessment, will allow us to tap into learning health systems, providing for the necessary alignment with the movement to living guidelines in NICE.

COVID-19

The recent COVID-19 pandemic has not only an impact on our ability to maintain planned levels of guidance production, it has also affected the ability of stakeholders to contribute and input. We intend to build on the learning and experience of the NICE Centre for Guidelines and their flexible approach to producing the COVID-19 rapid guidelines.

This encourages further exploration of opportunities within the areas of automation, faster evaluations and a diversification of CHTE outputs.

Regulatory processes

NICE plays a key role in ensuring the UK remains a destination of choice for the life sciences sector to invest, research and launch their new and innovative products. We are working closely with the MHRA to make sure we can continue to publish our guidance for medicines, medical technologies and diagnostics in a timely way following the Transition Period. This includes collaborative development of alternative licensing routes for medicines such as the Accelerated Assessment, Rolling Review or Project ORBIS in order to closely align regulatory and health technology assessments to benefit patients and the life sciences sector by facilitating faster access.

To help ensure continuity of supply of medicines and medical devices as of 1 January 2021, the UK will unilaterally recognise certain EU regulatory processes for a time limited period. These measures include automatic recognition of the decisions of the European Commission to authorise products under the Central Authorised Procedure (CAP) for two years. This will apply to most novel products and means companies who hold an EU Marketing Authorisation will be able to have their licence recognised by the MHRA and be provided with a UK licence.

However, alternative licensing routes for medicines such as the MHRA's Accelerated Assessment, Rolling Review or Project ORBIS are expected to be utilised.

NICE are also working with the MHRA and the Scottish Medicines Consortium (SMC) on an Innovative Licensing and Access Pathway for medicines. Work is also being initiated by NICE, MHRA and NHSx on exploring new pathways and support for digital health technologies. Although these pathways are important developments in the assessment of new technologies, they are in early stages of development and therefore are not subject to this specific consultation.

The UKCA (UK Conformity Assessed) mark for Medical Devices has recently been introducted and we are also aware of the forthcoming changes to the regulatory system for the UK medical devices market. We will collaborate with the MHRA on these important developments to ensure that we continue to publish our guidance in a timely way in accordance with the appropriate regulatory legislation (UKCA mark or CE mark).

The objective, through joint working between the MHRA, and other partner organisations in the UK, is to develop approaches to reduce the time to patient access for new technologies that will benefit patients. In order to facilitate delivery of this key objective, organisational partners must have the ability to be able to share confidential operational information regarding planning and scheduling in order to synchronise activities effectively and efficiently.

Highly Specialised Technologies

There is an important societal interest in rare diseases which raises the importance of our work in this area across all of the CHTE guidance producing programmes, none more so than the Highly Specialised Technologies (HST) programme.

Our overarching goal for considering a review of the HST criteria, including adding an overarching narrative of the purpose of the HST programme, is to make them clearer and more specific, and the outcome easier to understand and more predictable for our stakeholders. The number of highly specialised technology topics is not expected to change as a result of the revised wording.

The current criteria were developed in April 2013. This review considers particular challenges associated with the increase in technologies being developed for rare diseases being considered for the HST programme, challenges in the criteria interpretation and the changing disease therapies landscape for the latest health technology innovations (such as advanced therapy medicinal products (ATMP)s, histology-independent cancer treatments and other emerging technologies), to ensure that NICE can select the most appropriate technologies to be evaluated by the HST programme. This will secure rapid assessment and access to such valuable innovations, supporting the priorities and themes outlines in the recently published [UK Rare Diseases Framework](https://www.gov.uk/government/publications/uk-rare-diseases-framework).

Digital health technologies

We have successfully completed two evaluation pilots within the Digital Health Technologies space with important lessons learnt for the continuous improvements. As it stands, we expect to develop guidance on digital health technologies primarily through the medical technologies evaluation and diagnostics assessment programmes. As this field of technologies is evolving rapidly, NICE will continue to be flexible in its approach to these technologies and adapt its processes accordingly.

Diagnostics and genomic testing

Diagnostic and genomic testing continues to evolve rapidly. We will work closely with the NHS Genomic medicine service, Genomics England and other system partners to identify those technologies which are the greatest strategic priorities for the NHS on which to develop guidance. The COVID-19 pandemic has further amplified the need for radical change in the provision of diagnostics. Our processes must be flexible and responsive to enable us to respond to the needs of the healthcare system and new emerging technologies in this field.

NHS England & NHS Improvement commercial interactions

NICE works closely with NHSE&I to support commercial activities and enable timely commercial discussions between the NHS and the company. This partnership between NICE and NHSE&I is key to ensuring the production of timely guidance and patient access to cost-effective technologies. NICE acknowledges the principles and concepts outlined in the draft NHS Commercial Framework for New Medicines.

Innovative Medicines Fund

In an era of personalised medicine, pharmaceutical and biotechnology companies are developing more sophisticated and targeted approaches to developing novel treatments. At the same time there is increasing focus on finding a solution early in the product lifecycle, which exacerbates clinical uncertainties when decisions are required. Managed access is one potential option available to help address this situation.

The 2019 Conservative Party’s election manifesto included a commitment to introduce an Innovative Medicines Fund which will extend the successful operation of the Cancer Drugs Fund (CDF) to give patient access to the most promising, life-saving treatments. From NICE’s perspective, this call for an expanded fund with a broader focus presents an opportunity to determine the policy principles which guide how managed access operates beyond the Cancer Drugs Fund, clarify roles and responsibilities and review how our processes and methods align with this expanded scope for managed access within NICE health technology evaluation programmes.

NICE and NHSE&I are working jointly with the DHSC to consolidate policy principles underpinning the operation of managed access as part of the extension of this approach under a new Innovative Medicines Fund.

What approach was used?

Governance

An internal working group from across the organisation met regularly to ascertain difference in approaches across all of the guidance producing programmes and consider how and if the processes could be aligned.

Stakeholder insight group

A stakeholder insight group (SIG) comprising of external stakeholders representing industry, patient and carer organisations and academic groups was created in order to ensure proposals for process change were developed with continued input from the stakeholder community and enable a more interactive involvement in the change process. This stakeholder insight group will be an important forum for further developing the principles for change contained within this consultation document.

In addition to the stakeholder insight group, a formal patient working group was set up to help influence the future of patient and public involvement and ensure the vision was co-designed with the patients, carers and organisations we work with. The output of the work from the patient working group is available here: Improving meaningful public involvement in NICE medicines and technologies guidance.

This review comprises the work of many people in the task and finish groups, the stakeholder insight group and working group. This consultation sets out the key proposed changes to the process and provides an essential step in testing out these proposals with our stakeholders.

This consultation document considers processes for the evaluation of medicines, medical technologies, diagnostics and digital health technologies. It applies across all 4 of NICE’s health technology evaluation programmes (technology appraisals, highly specialised technologies, medical technologies and diagnostics), and seeks to align them when possible and appropriate. This will also remove the interim nature of the current HST process and will provide additional information as to the direction of travel regarding the criteria that will be used for selection of appropriate technologies for evaluation within the Highly Specialised Technologies Programme.

The key considerations and rationale for the changes is summarised in [appendix 1](#Appendix1), alongside the judgements NICE has made to come to these proposals. It is informed by the conclusions of the task and finish groups, prepared by NICE taking into account input from the stakeholder insight group.

Summary of proposals

Proposed changes are categorised into 4 themes:

* Alignment of the current guidance development processes
* Opportunities for new process improvements and ways of working
* Commercial and Managed Access processes
* Highly Specialised Technologies - vision and principles

Alignment of the current guidance development process

We intend to align all phases of the health technology guidance development process where possible. This includes adjustments to timelines to create efficiencies and removing inconsistencies and inefficiencies between programmes.

Proposals

* Develop a simplified singular process for all Centre for Health Technology Evaluation (CHTE) programmes.
* Align terminology used across all CHTE programmes.
* Scoping consultation length will be flexible from 5-20 days dependant on the needs of the topic.
* Scoping workshops will take place virtually.
* Companies will provide a 'Summary of Information for Patients' with their evidence submission.
* Patient and carer organisations can provide written submissions for consideration by the Committee in all guidance programmes.
* All stakeholders will have the same opportunities for participation in evaluations across CHTE. The individual terms ‘consultee’ and ‘commentator’ will be removed.
* NICE should provide designated account / stakeholder relationship managers for stakeholders to support their involvement in topics.
* The highly specialised technologies committee and medical technologies advisory committee shall develop and make recommendations on technology appraisals when required.
* The option of developing a final guidance document after the first committee meeting will be extended to diagnostics and medical technologies guidance.
* All guidance programmes will have the option of a long consultation (20 working days) or short consultation (between 10 and 20 working days).
* Assessments of multiple technologies within one piece of guidance shall also be an option for highly specialised technologies and medical technologies guidance.

Opportunities for new process improvements and ways of working

 The healthcare system environment continues to evolve, and we must both ensure we are providing outputs which this changing environment needs and take the opportunities this creates to improve our process for developing guidance.

Proposals

* Guidance on the growing field of Digital Health technologies will be developed as medical technologies and diagnostics guidance.
* We will use Experts who have been recruited to provide expert advice on other NICE topics (including guidelines) in the same clinical area in addition to those nominated.
* Technical engagement processes need to change to enable a high proportion of guidance to be developed using one committee meeting. Technical engagement shall be an option available within all guidance development programmes but will only be used for technologies that require additional support before committee.
* The option of a fast-track appraisal for technologies with a low ICER will be removed.
* We will have the option to terminate guidance development where the company submission is putting forward a base-case incremental cost-effectiveness ratio ICER significantly higher than the standard threshold.
* Establish a transparent process for NHS England & NHS Improvement to include draft treatment eligibility criteria within their submission to inform managed access arrangements.
* Develop a process to rapidly review guidance as a result of the introduction of biosimilars.
* Ensure transparency at final guidance stage on whether a company had obtained NICE scientific advice on the topic.
* Ensure our processes are focussed on and make a positive difference in reducing health inequalities.

Commercial and Managed Access processes

The following proposals are designed to enable NICE to facilitate commercial discussions to establish the cost effectiveness of a technology and/or to address uncertainties in key components of the evidence base.

Proposals

* All commercial and managed access proposals should include details of the Patient Access Scheme (PAS) which will form the core of all submissions to NICE.
* Request that companies provide a summary of data gaps to enable exploratory discussions about managed access.
* Develop NICE commercial and managed access processes to provide a mechanism for the identification of the need for routes to patient access.
* Develop criteria to assess the exceptional needs outlined by companies which warrant commercial and managed access options.
* Provide greater flexibility for commercial discussion to be concluded after the first committee meeting once the committee’s preferred assumptions are known.
* Allow a limited time period for commercial negotiations to conclude where a BIT breach is significant.
* Develop the process and criteria for making recommendations on and entry into a managed access arrangement.
* Explore opportunities to route promising technologies directly into managed access, without requiring a full health technology evaluation.
* Develop a process for the establishment and oversight of data collection agreements.
* Develop the approach for technologies to be re-assessed at the end of a period of manged access.

Highly Specialised Technologies programme

Proposals

Confirm the vision and underlying principles for the Highly Specialised Technologies programme in order to further develop the criteria for topic selection and routing.

Assessing deliverability of the changes

Workload analysis

The scale and impact of the proposals on both the workload of NICE and its stakeholders are currently unknown. It is anticipated that some of the proposals will be resource releasing whereas others will be resource incurring. The impact of the proposals be analysed further following receipt of responses to consultation.

Charging

It should be noted that calls for additional input, engagement, outputs may increase the charge to companies for Technology Appraisal and Highly Specialised Technologies guidance.

Appendix 1 – Summary of proposed changes

Alignment of the current guidance programme processes

A simplified singular process for all

The different CHTE guidance programmes have evolved separately over time resulting in different terminology and approaches to scoping, stakeholder involvement, technical engagement, consultation, committee consideration and process options. Stakeholders have different experiences, roles and requirements dependant on the guidance programme they are involved in.

We intend to align all phases of the health technology guidance development process where possible. This includes adjustments to timelines to create efficiencies and removing unwarranted inconsistencies and inefficiencies between programmes.

Proposals

Create a simplified singular process for all CHTE programmes (a diagram is available in appendix 2). The diagram indicates the alignment of major milestone steps within the CHTE guidance development programmes. It is important to note that management of exceptional circumstances and warranted differences between value proposition and technology type remain necessary.

Terminology

The NICE guidance programmes currently use different names for the main participants, documents and processes throughout the development of guidance. This can be confusing for stakeholders involved in developing guidance on different types of technologies. In future all programmes will use the same terms to describe participants, documents and processes. Currently, the general terms used within documentation to simply describe the process of guidance development/production are appraisal, assessment or evaluation. In the future, this will be aligned and the term 'evaluation' will be used universally.

Scoping

The current approach to scoping topics for guidance development differs across programmes. A universal process that removes inefficiencies and creates flexibility for both stakeholders and NICE is required.

The optimum approach to managing the scoping exercise should be based on the needs of the technology and would include evaluating the requirement to consult on the scope and determining if a workshop is required. This assessment will be based on existing corporate knowledge and experience of both the technology type and disease area.

Proposals

All NICE guidance programmes will have the option of a long consultation (20 working days) short consultation (between 5 and 20 working days), and/or the requirement for a scoping workshop in order to obtain relevant stakeholder input to the guidance scope. NICE should determine at the beginning of the scoping process the length of scoping consultation or requirement for a workshop which is proportionate and best suited to determining the final scope.

Some topics may not require a formal scoping consultation and NICE will release what will be the ‘final’ scope with the invitation to participate. This approach may need to be implemented when accelerated regulatory processes are utilised, or for topics where NICE has built a significant amount of corporate knowledge over time.

Any scoping workshops that are held will primarily take place virtually (using video conferencing software) as opposed to in person to ease the impact of travel and make participation more convenient for interested stakeholders.

Stakeholders

Stakeholders are a vital component of NICE's work. Keeping relevant stakeholders informed of our work, using stakeholder expertise, and representing stakeholder interests in NICE guidance is vital, and is underpinned by the NICE charter and NICE principles.

Creating a positive stakeholder experience and engaging with stakeholders in a timely, seamless manner, is crucial for the success of NICE guidance now and in the future.

Stakeholder engagement processes throughout all programmes must ensure accurate stakeholder identification and continued involvement is consistent and meaningful. Communicating both timelines and key developments are imperative to stakeholder investment and knowledge of an individual topic in which they have chosen to invest time and resource.

We are aware of the desire from our stakeholders, particularly patient and carer organisations and individuals to provide further support to help navigate and understand committee documents. Exploration has been ongoing to pilot the creation of a lay summary of industry’s submission to NICE, known as the 'Summary of Information for Patients' (SIP). Its purpose is to help patients understand industry’s submission so that they can better engage in an appraisal; primarily to help them understand the evidence more easily to write their submission, but of course it will go considerably beyond this and provide a better understanding of the evidence for technical engagement and the committee meeting. The SIP is written by the company and included with the rest of their submission and sent on to the other participating stakeholder organisations by NICE and can be used to inform later submissions and statements. The company may also be asked to provide a SIP to support their response to technical engagement, or submission of additional evidence in response to additional consultations during guidance development.

Stakeholders are categorised using different terminology across guidance programmes in CHTE. In TA, HST, and MTEP, the terms consultee and /or commentator are used to define which stakeholders can submit evidence, nominate experts and appeal (including resolution in MTEP), and the terms mean different things in TA and HST versus MTEP. In other programmes in CHTE, these terms are not used.

We acknowledge that stakeholders have limited capacity to contribute to NICE assessments, and therefore we must look for opportunities to ease the burden of participation where possible, and at least, not to increase the burden.

Proposals

Stakeholders will receive regular informative communications as to the status and development of topics that are subject to guidance development. Where topics are delayed, paused or rescheduled, NICE will work with companies to provide clear rationale for changes and timescales to the wider stakeholder group.

Companies will be asked to provide a 'Summary of Information for Patients' as a standard element to their evidence submission.

Patient and carer organisations will have the opportunity to provide written submissions for consideration by the Committee in all guidance programmes.

NICE should explore mechanisms to provide feedback to patient and carer organisations. The benefits of the feedback letter process in the IP and HST programmes should be reviewed and, if appropriate, the practice replicated throughout CHTE. This could involve input from the lead team committee members.

The terms ‘consultee’ and ‘commentator’ should be removed to allow stakeholders the same level of participation across the CHTE guidance producing programmes. Some sub-categories of stakeholders such as the 'comparator manufacturers' would not be invited to submit evidence, nor appeal / request resolution on the draft final recommendations.

 NICE should provide designated account / stakeholder relationship managers to work across the wide stakeholder community, building on the highly respected work and achievements of the existing Public Involvement Programme. This would allow the support all organisations and individuals through the evaluation processes from start to finish.

Committees

To increase flexibility, we intend to better utilise the capacity of committee members and meetings. Meeting virtually is more convenient for committee members and other participants. It also reduces the travel burden for attendance for the public and stakeholder organisations. It has also led to improved attendance and reduced quoracy issues, particularly towards the end of meetings. This approach reduces the amount of time attending committee meetings takes participants away from their NHS and other activities.

As referred to within the Topic Selection consultation document issued in October 2020, NICE intends to use the skill and expertise of the medical technologies evaluation committee to develop and publish technology appraisals on medical devices. Forthcoming hybrid technologies that combine the use of different technology types will be discussed by the most relevant committee with the expertise in the area of the technology that has the greatest uncertainty.

The involvement of company representatives, professional experts, patient experts and external academic groups will be aligned across all CHTE guidance committees ensuring they have the same options and level of participation in the committee discussion irrespective of the type of guidance being developed.

Proposals

The highly specialised technologies committee shall develop and make recommendations on technology appraisals when required.

The medical technologies advisory committee shall develop and make recommendations on technology appraisals of medical devices.

Committee meetings will primarily be held virtually with the option to hold meetings in person where appropriate.

Guidance consultation requirements

It is important to ensure that committees have the same options available for developing guidance in the shortest timescale and use their limited capacity to develop guidance on the maximum number of promising technologies. All programmes will have the option of publishing a final evaluation guidance document without consultation, where the first committee meeting has produced a clear outcome where the recommendations are unlikely to be changed by consultation.

Proposals

Akin to the processes already used within technology appraisal and highly specialised technologies guidance; for diagnostics guidance and medical technologies guidance there will be the option of developing a final guidance document after the first committee meeting where the recommendations are clear and unlikely to be materially changed by public consultation. Stakeholders will still have the right to raise factual inaccuracies or comment on a breach in process during the resolution stage before final guidance publication.

All NICE guidance programmes will have the option of a long consultation (20 working days) or short consultation (between 10 and 20 working days), in order to obtain relevant stakeholder input to draft guidance. NICE should determine at the beginning of the consultation process the length of consultation which is proportionate.

The suspension and termination process

There are different approaches and terms used when CHTE guidance development is stopped before publication. As per the process already utilised within the Technology Appraisals programme, all guidance programmes should have the opportunity to formally terminate evaluations and formally publish this as a guidance output. The termination process should be utilised when the guidance development process cannot be concluded and will clearly explain the reasons for termination of the guidance on the NICE website.

Proposals

Where the development of guidance has started but cannot be concluded due to non-submission of evidence from the company, or NICE do not agree that an existing submission is fit for purpose and the technology has UK regulatory approval, the topic will be published as Terminated guidance. NICE will state the reason for the termination. The term 'cancelled' will no longer be used.

Where the development of guidance has started but cannot be concluded and the technology does not receive UK regulatory approval, the topic will be formally regarded as 'discontinued'.

Where the development of guidance is paused with the expectation that it will restart, the guidance status will be regarded as 'suspended’.

Multiple Technology Assessments for Highly Specialised Technologies and Medical Technologies

The option of carrying out a multiple technology assessment currently only applies to technologies being considered within the technology appraisals and diagnostic assessment programmes.

Proposals

To increase flexibility, maximise resource, align working practices and provide timely guidance for the NHS, NICE proposes introducing the option of a multiple technology assessment for Highly Specialised Technologies.

NICE proposes to determine if there is demand from the healthcare system for multiple technology assessment of cost saving medical technologies.

Opportunities for new process improvements and ways of working (adapting to the changing healthcare environment and addressing key challenges)

Expert identification, selection and involvement

Patient, clinical and commissioning expertise is vital to the guidance development process. Therefore, great importance should be placed upon how experts are identified, selected for participation and supported through the process. As the number of guidance topics per year has grown it has become increasingly difficult and resource intensive to identify and recruit sufficient independent experts. This has also placed a greater burden on patient/carer organisations and professional societies who are asked to nominate many experts.

An exercise to align and provide clarity for expert involvement across the different guidance programmes has already taken place, but the process of expert involvement should be enhanced. Often nominations and selection of experts are made very close to the committee meeting leaving little preparation time for both NICE and the experts selected. This also places additional pressure onto NHS Trusts for short notice requests for leave/absence to take part in NICE guidance development.

Expert involvement remains an integral part of health technology guidance development. NICE will explore how and when the patient evidence is presented to the committee.

Proposals

We propose to start the expert nomination process earlier in the guidance development process, capitalising on the engagement with stakeholders during the scoping process.

Patient/carer and Professional organisations will still be asked to nominate experts for all guidance topics in their field. NICE will explore opportunities to ensure there are sufficient potential experts for all guidance development topics.

In order to broaden adapt the process for recruiting experts to a much-increased volume of topics, Experts that have been nominated and selected to participate in other related guidance development activities across NICE (including NICE Guidelines) will be considered eligible to act as experts on related topics. NICE may request their participation in the guidance development of other related topics.

The Diagnostics Assessment programme will continue to use Specialist Committee Members in their development process instead of patient and clinical experts that the Medical technologies, Highly Specialised Technologies and Technology Appraisals will use.

Working in parallel with the regulatory process

A key driver for technology appraisal and highly specialised technologies programme is the speed of NICE decision making (referred to as timeliness) in relation to the regulatory approval of a new medicine. This is evident in the 2016 arrangements for the CDF and the 2019 Voluntary Scheme for Branded Medicines in which the commitment is made for NICE to publish final guidance for new medicines within 90 days of receipt of regulatory approval.

The fundamental approach used by NICE to facilitate timely decision-making is to develop NICE guidance alongside the regulatory approval processes. In order to be able to publish guidance on new medicines within 90 days of the marketing authorisation, NICE will hold the first committee meeting ideally at a point in time close to when a formal regulatory signal has been made for a new technology. It is crucial that this approach is is aligned with the updated MHRA regulatory approaches.

As more information becomes available on new UK regulatory approaches for all technologies following the end of the EU Exit transition period, it is evident that some technologies will be able to access expedited regulatory procedures. Companies currently hold a position that it would not be feasible to submit an evidence dossier to NICE ahead of the submission of an evidence dossier to the formal regulatory body.

It will be important to also consider parallel working in the context of the forming regulatory pathway for medical devices, diagnostics and digital health technologies in order to promote equity across the CHTE programmes and provide fast, timely access for patients to these technologies.

It is important that the NICE processes can keep pace with the expedited regulatory procedures and further consideration as to how this can be achieved is required. Alignment of the timing and synchronisation of evidence submissions are key factors to consider, alongside the potential to reduce the time spent in the scoping and consultation phases of evaluation.

Proposals

On occasion, there may be a delay in the regulatory process. In order to minimise the impact on the appraisal process and committee availability, a short delay to regulatory approval is not expected to adversely affect the existing timing of the committee meeting. Because the technology will not, at this stage, have received a formal regulatory signal, the committee meeting will be held in private (without the public gallery) to preserve the confidentiality of the data submitted by the company. A significant regulatory delay would require the committee meeting to be rescheduled to an appropriate future date. A parallel HTA and regulatory development approach should not be exclusive to medicines and can also be utilised for medical technologies and diagnostics where appropriate.

Develop an expedited variation to the standard evaluation process to facilitate alignment with accelerated UK regulatory processes.

Technical engagement

In April 2018, an updated Guide to the processes of technology appraisal was implemented to manage the increasing number of topics being routed through the 4 technology appraisal committees and to enhance engagement with stakeholders.

The new process provided an early engagement step with companies to resolve key technical and commercial issues ahead of the first appraisal committee meeting. The aim was to maximise the opportunity to go straight to final draft guidance publication after the first committee meeting, avoiding the need for consultation, a second committee meeting and utilisation of additional resource from NICE and all stakeholders.

After two years the technical engagement step has been modified to adapt the process and to retain the elements that have proved popular (for example, increased engagement). One of the key benefits from the new process is a move to developing a focused 'issues-based' technical report, and to clearly ask stakeholders for input into these specific issues. This has been achieved by re-framing the ERG report so that it is presented in an issues-based style. The final ERG report is circulated for stakeholder engagement and feedback. Allowing the company this ‘right to reply’ addresses one of the major criticisms of the old process, where feedback was restricted to correction of factual inaccuracies and there was no opportunity to air and address differences of opinion.

Between 2006 (following the introduction of the straight to final draft guidance option via the Single Technology Appraisal process) and 2016 the decision rate was only 19%. However, following the introduction of the new arrangements for the CDF in 2016, the decision rate rose to 33%, with cancer topics doubling in the rate of straight to FAD decisions. Since the introduction of the technical engagement step in 2018, only 45% of topics (both cancer and non-cancer) result in a straight to final draft guidance decision. It is unclear as to whether the rate is positively affected by the number of cancer topics that NICE appraises and the opportunity for the committee to make a recommendation within the CDF or whether the technical engagement stage has made a positive impact (albeit small).

We have not been able to capitalise on the idea to work earlier with companies in the technology appraisal programme on the evidence submission, through technical engagement, in order to extract a more acceptable value proposition earlier in process. Investing in support for early discussions focussing on commercial and managed access should allow NICE to revisit how we can improve throughput to continue to meet the expectations for timely and relevant guidance.

The digital health technology assessment pilots also incorporated the use of a technical engagement step within guidance development. The pilots concluded that a less resource intensive, and flexible approach to technical engagement which resolves uncertainties around the digital nature of the technology at an early stage – particularly its interaction with the system and reliance on human intervention – would be more appropriate moving forwards. Evidence gaps should also be discussed at this stage. It was also thought appropriate that the technology sponsor is invited to resolve uncertainties relating to, for example, the design, intended use, available evidence. This could also play an important role in the development of guidance on other Medical Technologies.

Proposals

It is imperative that the value gained from the inclusion of the technical engagement stage of the process is proportionate to the level of effort and resource utilised, for NICE and all stakeholders. To resolve the escalating challenge we propose that the technical engagement step shall be an option available within all guidance development programmes, but will only be used for technologies that require additional support (i.e. commercial, managed access). The technical engagement step should be used to note and consider any evidence gaps and potential resolution ahead of a committee meeting.

Fast Track Appraisals

The Fast-Track Appraisal (FTA) process was introduced within the technology appraisals programme in 2016. FTA was predominantly designed to deliver an equally robust but less resource-intensive decision than a standard single technology appraisal. NHS England and commissioners committed to provide funding for technologies recommended via the fast track process within 30 calendar days of guidance publication.

The FTA process is comprised of two methodological elements:

Cost comparison - where a case can be made that the technology to be appraised is expected to provide similar or greater health benefits, at a similar or lower cost, compared with technologies that have been previously recommended in published NICE technology appraisal guidance for the same indication and an estimate that the budget impact of the technology would fall under NHS England and NHS Improvement’s budget impact level for the full patient population relevant to the appraisal.

Low ICER case where the aim is to make available, more quickly, those technologies that NICE can be confident would fall below £10,000 per quality adjusted life year (QALY) and its budget impact below the level set by NHS England and NHS improvement.

The current FTA process for technology appraisals appears to be significantly underutilised as few technologies have been considered via this route since its introduction. As of January 2021, only 6 topics have been formally appraised using the FTA process, all of which have made a case for cost comparison. There have not been any successful cases presented of a low ICER.

NICE is responsible for the decision to allocate and schedule an appraisal as a single technology appraisal or a multiple technology appraisal. A company can request to convert the STA into an FTA, although NICE will make the final decision on whether the standard or fast track process will be used. The life sciences industry have reported that the current approach to the format of the evidence submission and timing of the NICE decision to confirm the FTA process is accepted in a cost comparison case presents a barrier to their considerations and appetite for an FTA in this setting.

Proposals

To create a simplified process and balance the needs of other changes to types of health technology evaluation options, NICE proposes removing the low ICER FTA option for technology appraisal.

In efforts to recover from the COVID-19 pandemic emergency the Scottish Medicines Consortium reviewed their work programme and encouraged sponsors to use the abbreviated submission process for new medicines where alternatives within the same therapeutic class have previously been accepted for use by the Scottish Medicines Consortium. There may be an argument for NICE to consider a similar approach and to allocate and schedule appraisals as a single, multiple or fast cost comparison appraisal type, following completion of topic selection. This would retain the option of a cost comparison FTA but will not allow companies the discretion to decide to convert from the STA to an FTA. There may also be an opportunity to review whether a full technology appraisal committee would be required to make the recommendations within the FTA, or whether an alternative decision maker (for example NICE Guidance Executive) could be considered.

A simpler approach to evaluation for technologies with significant numbers of indications and applications

Drug development can now see exploration of multiple indications for new drugs. Companies are applying for marketing authorisations for more than 10, and sometimes up to 20, therapeutic indications, where in the past it would have been exceptional to receive more than 5. This applies to cancer drugs in particular and can already be clearly seen by the amount of guidance that is either published or in development for immune-oncology drugs such as pembrolizumab, nivolumab, atezolizumab.

This issue is also prevalent in the evaluation of medical devices where these technologies can be used in multiple populations such as urine collection, vascular access and US/CT/MRI imaging.

The scale of individual drug-indication pairings or medical technologies that are in the development pipeline for regulatory approval over the coming years could rapidly absorb a significant proportion of capacity within the NICE evaluation work programmes.

Proposals

We must consider if the current approach to conduct a full technology appraisal for every new indication is proportionate or if an alternative simpler approach to the evaluation, or alternative NICE product, may be more appropriate for technologies that are evaluated multiple times. We seek stakeholder views and input during the consultation about the different approaches and principles that may need to be applied in order to deliver an alternative solution.

Managing company submissions

The technology appraisal programme receives company submissions that include a base case ICER that is significantly higher than the standard threshold (£20,000 - £30,000 per QALY) upon which the committee will base their decision. Some companies have presented an ICER of over £200,000 per QALY. In some scenarios, this issue may be able to be addressed through commercial opportunities although this may not be the case for all. The current process requires that all next steps are completed in order to present such a case to the appraisal committee for a decision. Consideration must be given as to whether this remains a proportionate use of committee capacity and investment of both stakeholder and NICE resource or whether there is an alternative approach that can be taken in this regard.

Proposals

Where a company presents a value proposition that includes a base case ICER that is significantly higher than the standard threshold, and this level of ICER cannot or has not been sufficiently addressed through commercial discussions between the company and NHS England and Improvement, NICE will reserve the right to assess the feasibility of presenting the topic to committee for a decision. If NICE do not believe it appropriate to present this topic to committee, the company submission will be formally rejected and the evaluation will be terminated. The termination will be published on the NICE website. It is expected that this process would apply to all CHTE guidance producing programmes that consider cost effectiveness.

Combination treatments

NICE fully supports the Association of the British Pharmaceutical Industry (ABPI) in its ongoing efforts to find solutions to enable companies to engage with one another to ensure that the combined cost of combination treatment can be developed for evaluation, at the standard NICE threshold, in line with competition law. While we anticipate that any approach identified for specific combinations will require an agreement in principle before the formal evaluation process begins, we recognise that the companies may need to understand the NICE appraisal committee's preferred assumptions before finalising the details of the combined cost for the combination.

Proposals

To ensure that there is an opportunity post committee for companies to agree on the combined cost of combinations once the committee’s preferred assumptions for the economic modelling are known. We will work with the committee chairs, vice-chairs and wider members on how they can support us in minimising the need for more than one formal committee meeting.

The introduction of Biosimilars

When an originator technology loses its market exclusivity, it is anticipated that there will be a reduction in the price of the technology as a biosimilar or generic product enters the market. This reduction in price is generally significant. Normally, if the originator has a NICE positive recommendation, the recommendation applies across new market entrants. However, if there is no recommendation or a negative recommendation for the originator, the biosimilar or generic product can struggle to achieve market access.

The standard route to access would be through a technology appraisal and a positive recommendation from NICE. However, it is unlikely that an individual biosimilar or generic company would have the relevant clinical trial evidence for all licensed indications to be able to construct a submission dossier. Nor would it be willing to pay the cost recovery charge for a new technology appraisal as its potential revenue will be significantly limited as a positive recommendation could open the market up to competitors.

This is particularly challenging when there remains a clinical need for the technology, the decrease in price has the potential to make the treatment cost effective and there are companies willing to enter the market but unwilling to pay for a technology appraisal.

This also presents challenges where an originator product is under appraisal or is part of a combination that is under appraisal by NICE and where the originator or combination is not cost-effective but it is known that loss of market exclusivity, and subsequent price decrease, is likely to make them cost-effective.

Proposals

Develop a process that allows the rapid review of guidance where loss of market exclusivity and the subsequent price decrease of an originator could result in the a biosimilar product being cost-effective where neither the pathway nor the evidence has changed significantly.

NHS treatment eligibility criteria

In the technology appraisal and highly specialised technologies evaluation programmes, NHS England & NHS Improvement use treatment eligibility criteria for some high-cost drugs and within the context of Managed Access Agreements (MAA) to ensure that clinicians are aware of the evidence that demonstrates which patients will benefit from treatment. The interface between this NHS England & NHS Improvement process and NICE's health technology evaluation processes varies for different categories of treatment and is not reflected in the existing published process documentation.

The current process lacks transparency as there is no clear guidance as to when stakeholders will see and be able to comment on proposed criteria.

Proposals

Create a standardised process for NHS England & NHS Improvement to include draft treatment eligibility criteria within their submission to NICE, along with a rationale explaining the need for these additional criteria, including where they may subsequently arise from commercial negotiation associated with either a MAA or a Budget Impact Test. This process will enable engagement with wider stakeholders.

Recording when scientific advice has been sought

Companies can seek early scientific advice from NICE on their clinical and economic generation plans. This is a fee-based service. Currently there is no indication in the final guidance for products on whether the company sought scientific advice or not.

The EMA now publishes whether companies sought scientific advice when they publish the European public assessment reports. This development was introduced after a European Ombudsman review of the EMA scientific advice process in which there was a call for more transparency.

The publication of which products have received early scientific advice in the final NICE guidance would allow an open assessment of the value of NICE scientific advice.

The fact that early scientific advice has been sought should not be disclosed to committees or the NICE teams supporting the guidance production during the deliberations, to avoid any potential influence over the decision making.

Proposals

Publish whether a product received NICE scientific advice in the final guidance. This would only report standard, express, parallel and medtech advice and would not apply to the gap analysis service Medtech Early Technical Assessment (META) or the preliminary independent model advice (PRIMA) service.

Reducing Health Inequalities

It is key that our guidance development processes actively work to reduce health inequalities in the population and healthcare system. NICE is committed to using its guidance to improve the opportunities for everyone to lead a healthy life.

Proposals

NICE wants to hear from our stakeholders on what changes we can make to our processes help to reduce health inequalities in the way we develop our guidance, how stakeholders participate in the process and how health inequalities are identified and considered in making recommendations.

Commercial & Managed Access processes

Commercial and Managed Access (CMA) processes link specifically to technologies being evaluated by the NICE technology appraisal and highly specialised technologies programmes.

Commercial proposals and managed access proposals

Patient Access Schemes are the longest running and default option for companies to use. There are two types of PAS;

* 1. Simple PAS (confidential)
	2. Complex PAS (transparent)

The simple PAS proposal is the fastest option available to companies. Companies should always include a simple PAS when submitting commercial proposals to NICE.

More recent commercial options are those that fall under the definition of Commercial and Managed Access Agreements outlined in the anticipated NHS England & NHS Improvement Commercial Framework for New Medicines.

To ensure company-led proposals for either commercial and/or managed access are received in a timely manner and are available to both NICE and NHS England & NHS Improvement, a new process for submitting these proposals will be outlined. Commercial and/or managed access proposals provide a route for companies to identify early commercial and managed access needs for their technology.

A key objective is to enable NICE to proactively facilitate commercial discussions between NHS England & NHS Improvement and companies concerning the most plausible options available for a deal to establish the cost effectiveness of a technology and/or to address uncertainties in key components of the evidence base submitted to NICE that cannot be managed by a simple PAS alone.

Proposals

All commercial and managed access proposals should include details of the Patient Access Scheme (PAS) which will form the core of all submissions to NICE. If companies require a commercial flexibility over and above a PAS, this request will need to be justified and rationalised as part of the proposal.

Where evidence gaps or significant clinical uncertainties are likely to impact on the ability of NICE committees to make a recommendation for routine commissioning, companies will be asked to provide a summary of the data gaps to enable exploratory discussions about managed access. NICE will share appropriate information with NHS England & NHS Improvement in line with organisational principles and confidentiality arrangements.

Develop NICE commercial and managed access processes to provide a mechanism to assess the potential need for routes to patient access in addition to PAS including commercial flexibility and managed access.

Develop criteria to assess the exceptional needs outlined by companies which warrant commercial and managed access options.

Consider how to help companies identify and make preparations for the most appropriate commercial mechanism, including a PAS, to enable their best value offer ahead of the first appraisal committee meeting.

NICE will provide greater opportunity after the first committee meeting for companies whose value proposition has been sufficiently developed using early commercial engagement opportunities, to allow commercial discussions to take place once the committee’s preferred assumptions for the economic modelling are known. NICE will work with the committee chairs and vice-chairs and wider members on how they can support us in minimising the need for more than one formal committee meeting.

The Budget impact test

The current budget impact test process was introduced in 2017. It was designed to achieve a balance between the requirement for timely guidance and any negotiation NHSE &I need to undertake to manage affordability. Maintaining the overall 'tension' between these aspects after the first evaluation committee meeting is desirable, however 'learnings' from the first phase of the BIT has highlighted opportunities for further refinement.

Proposals

NICE will re-consider the timelines and steps after the appraisal committee meeting where a FAD has been agreed, to allow a limited time period for commercial negotiations between the company and NHS England & NHS Improvement. This will only be offered in exceptional cases where a BIT breach is significant. Stakeholders will be fully informed of the application of the additional commercial negotiation timeframe.

Clarifying the status of a recommendation for managed access

The status of a recommendation for managed access is unclear. While a recommendation for managed access has been represented as a third option for a recommendation from NICE's committees, the National Institute for Health and Care Excellence (Constitution and Functions) and the Health and Social Care Information Centre (Functions) Regulations 2013, only provides for a recommendation for routine commissioning (or not).

Topics recommended for managed access via the Cancer Drugs Fund (CDF) are more clearly the subject of a conditional recommendation for patient access within the context of the fund. Outside of the CDF, for Highly Specialised Technologies and other Technology Appraisal topics recommended for managed access the position is less well established.

Proposals

To work with all stakeholders and our partners at NHS England & NHS Improvement and the Department of Health and Social Care to confirm the status of recommendations for managed access and the rationale for this position with reference to the funding requirement outlined in sections 7 and 8 of the NICE Regulations.

Managed access entry

We propose to confirm the criteria that NICE's health technology evaluation committees take into consideration when making a recommendation for use with a Managed Access Agreement (MAA). In particular, to review the existing criteria for entry into the Cancer Drugs Fund and to consider whether and how these principles could be extended to guide decision making for any technology.

Additionally, we will facilitate committee decision by ensuring they have access to relevant information to:

Assess whether the research questions can be addressed via an ongoing study, real world data collection or a combination of both

Understand whether further data collection is possible – i.e. is the data currently being collected, or is there a realistic prospect of initiating a new data collection?

Determine that the acquisition cost of the technology during the period of the MAA is commensurate with the level of uncertainty determined by the committee.

Currently, NICE's processes require a full technology appraisal for our committees to provide a recommendation for use subject to a time limited MAA. Experience is demonstrating that a full technology appraisal is required to evaluate whether the technology can be recommended for routine commissioning after a period of managed access as well. The time, resources and capacity requirements for topics recommended for managed access is twice that of topics recommended for routine commissioning. We must explore opportunities to develop a more streamlined market access entry process that is aligned with the MHRA's Innovative Licensing and Access Pathway, as well as for topics identified from an integrated horizon scanning function for the NHS in England. In principle, the objective is to explore options to enable earlier patient access to promising new technologies, and supporting NICE to make recommendations for managed access where immature clinical outcome data and / or significant clinical uncertainties are likely to preclude a recommendation for routine commissioning. Any new process will need to strike the right balance to ensure there is sufficient probity and rigor, while at the same time facilitating transparent and robust decision making.

Proposals

Explore opportunities to route promising technologies with significant evidence gaps directly into managed access, without requiring a full health technology evaluation.

Develop a process for assessing company-led proposals for managed access to ensure NICE has access to all relevant information required to make a recommendation for use subject to a managed access agreement.

Develop a single list of considerations for use by NICE technology evaluation committees to guide the development of recommendations for use in managed access.

Data collection agreement development and oversight

NICE takes the lead in developing Data Collection Agreements, while NHS England & NHS Improvement leads in drafting the Managed Access Agreements and Commercial Access Agreements. Each Data Collection Agreement is developed collaboratively with the company, data provider, NICE and NHS England and Improvement, via a short-term working group, which has the specific task of operationalising the committee's recommendation with reference to the significant uncertainties identified. The operation of these groups varies between cancer topics (where the process is highly standardised and streamlined) and non-cancer topics, which due to their inherent complexity follow a different process, which is partly standardised and partly tailored to the needs of each technology.

During the period of the Managed Access Agreement, NHS England & NHS Improvement ensures that there are appropriate monitoring systems to review that the data collection and evidence generation elements are being implemented faithfully. NICE works with a range of stakeholders (including NHS England & NHS Improvement, the company, data providers, clinicians, patient groups and academics) as part of a Managed Access Oversight Group for each technology to ensure high quality data is being collected, arrangements for statistical analysis are in place and robust, patient access is enabled in line with the recommendation for use in managed access and that any issues that emerge are addressed effectively.

During a managed access agreement, it may be necessary to review interim data reports to assess whether the data collection is providing the required outputs and to assess whether the deliverables required for the re-assessment will be available. Currently there is neither a formal process to highlight if a data collection exercise is not delivering the outputs expected, nor clarity on the corrective actions that NICE might require to maintain the viability of the Managed Access Agreement.

From time to time, there may be a need to update the Data Collection Agreement (for example, when marketing authorisations are updated) to reflect changes in the licensed patient population and implications for the data collection exercise. The process for these amendments and the role of the Oversight group in reviewing changes for each Data Collection Agreement needs to be clarified.

Proposals

Processes for the development and operationalisation of MAA will be documented, CAA and DCA following a recommendation for use subject to a MAA. This will include the role and purpose of a data collection working group and opportunities for various parties to participate in this process

Document processes for monitoring the performance of active data collections, including the various parties who might be involved in this process and develop a transparent process for interim reviews, which may produce recommendations for corrective action and amendments to the MAA and/or an early re-evaluation of the evidence if the rationale underpinning the original data collection are found to have altered.

Develop a process for the amendment of treatment eligibility criteria within a Managed Access Agreement, for example, when a marketing authorisation is extended to a new age group of patients.

Managed access exit

Experience with MAA guidance reviews and the reassessment process for multiple topics following a period of managed access have identified the need for a single process across all health technology evaluation programmes. Variation between processes for CDF, HST and non-cancer TAs reassessments are no longer reasonable. Additionally, the shorter CDF guidance review process has not delivered significant efficiencies, while at the same time reducing the flexibility that NICE committees, and external stakeholders would like when topics come back for review.

The longer than anticipated average duration of MAAs is another factor that suggests a standard reassessment process is needed. During any period of managed access these is the potential for significant changes to occur in treatment pathways. The current fixed approach to MAA guidance reviews does not provide an opportunity for the implications of any changes to be reflected. Therefore, we propose to consider whether to conduct all managed access reviews as a full reassessment (e.g. STA) under the process and methods current at the time of the invitation to participate for the guidance review. This will provide greater clarity and certainty to all stakeholders concerning the process for the reassessment of topics following a period of managed access.

Proposals

Develop a single approach to MAA guidance reviews, for technologies to be re-assessed at the end of a period of manged access.

 Objectives & vision of the Highly Specialised Technologies programme

In 2013, the highly specialised technologies (HST) programme was launched following the transfer of evaluating drugs to treat very rare diseases from the Advisory Group for National Specialised Services (AGNSS) to NICE.

The relevant regulations are made under s. 237(1) of the 2012 Act are the National Institute for Health and Care Excellence (Constitution and Functions) and the Health and Social Care Information Centre (Functions) Regulations 2013 (the“2013 Regulations”). Among other things, the 2013 Regulations establish the twin processes (HTA or HST) for the recommendation of “health technologies” for NHS use by NICE.

A “highly specialised health technology” is defined as “a health technology intended for use in the provision of services for rare and very rare conditions provided for in regulations under section 3B(1)(d) of the 2006 Act”. This is a reference to s. 3B(1)(d) of the National Health Service Act 2006. The relevant regulations made under that provision are The National Health Service Commissioning Board and Clinical Commissioning Groups (Responsibilities and Standing Rules) Regulations 2012 (the “2012 Regulations”). The 2012 Regulations provide at regulation11 (entitled “Specified services for rare and very rare conditions”) that “the Board must arrange, to such extent as it considers necessary to meet all reasonable requirements, for the provision as part of the health service of the services specified in Schedule 4". Schedule 4 to the 2012 Regulations sets out over 150 services for rare and very rare conditions.

The vision for Highly Specialised Technologies

The objective and intent of the HST programme is to provide fair and equitable access to treatments for patients with serious and severe ultra-rare conditions where there is vulnerability, substantial unmet needs, or very limited, not very effective treatment options, who would be disadvantaged by an appraisal undertaken via the standard appraisal process.

The HST programme is necessarily exceptional and a separate programme from the standard appraisal programme. It is a deliberate departure from the principle of a “level playing field” for the evaluation of all treatments. This is because it is recognised that a simple utilitarian approach in which the greatest gain for the greatest number of patients’ approach would most likely result in small numbers of patients with rare conditions not likely to get access to new treatments. Therefore, it must be seen in the context of that process and should not be used for most new treatments.

The HST programme operates in the acknowledgment of the significant challenges associated with ultra-rare conditions, including barriers to evidence development and the opportunity to make reasonable returns for research and development. However, it was not set up with the intention of appraising all rare disease technologies and it would be inappropriate for the programme to do so.

The landscape of rare disease technologies has evolved and changed since the HST programme’s inception. There has been an increase in the number of treatments in development across the spectrum of rare disease.

Purpose of the HST Topic Selection criteria

The programme was intended to consider a very small number of treatments for very rare conditions. The number of technologies that can be evaluated through HST will be responsive to the pipeline and there is no fixed limit on the number that can be evaluated in the programme. To qualify, topic selection decision makers must be satisfied all the HST criteria have been met in full.

The HST topic selection criteria are used to support decision makers to make clear, consistent, and transparent decisions on which topics are the most appropriate to route to the HST programme. Stakeholders should be provided with clarity and the relative ability to predict a routing decision (based on the criteria).

The criteria are not a tool to manage affordability or budget impact in the NHS. This is managed within other measures such as the budget impact test assessment and via commercial conversations.

NICE’s HST topic selection criteria should be transparent, clear and robust, to support decision makers to apply and stakeholders to interpret, taking into account the ever-changing health and care landscapes.

This consultation considers all relevant factors affecting the selection of topics for the HST programme. Key considerations include the wider objectives and needs of patients, families and carers, the NHS, government, the life sciences industry, and other stakeholders. Although an independent body, NICE does not operate in isolation and must recognise the needs of all stakeholders.

Review of the existing HST criteria

The objective of the review of the HST criteria is to:

* 1. Provide predictability and clarity to decision makers and stakeholders.
	2. Increase transparency and make decision making efficient.

The final routing recommendation should be made by deliberation, consensus, and the application of judgment by an appropriately selected group of relevant disciplines/experts. This should be in the form of an expert decision-making group who can apply expert knowledge to the information presented to make topic appropriate routing decisions.

The HST topic selection criteria aim to acknowledge and understand the challenges associated in generating evidence and making a case for such small populations. Importantly, it recognises the significant benefits that can be achieved for these ultra-rare patient groups when these new technologies are recommended. It is acknowledged, in these cases, even if the technology were to be provided with significant discounts or other economic mechanisms it would not succeed in a standard technology appraisal.

The HST topic selection criteria have been designed to recognise and make provision for the many different scenarios and situations that have occurred and that may occur in the future. Past and future topics and scenarios have provided an opportunity to reflect and consider where the HST criteria needed more clarity to meet the vision and objectives of the programme.

Key principles of Highly Specialised Technologies

The HST programme is governed by a number of key principles that help guide and support the vision and objectives. These principles have been developed in response to stakeholders seeking clarity and better understanding of how the HST programme selects topics for evaluation.

* 1. A technology may only be considered for the HST programme if the condition for which it is for is listed in the Manual of prescribed services (or is expected to do so at the time of guidance publication) and if it meets all of the selection criteria. There is a recognised highly specialised commissioned service formally recognised by NHS England or recognised as Rare Disease Collaborative Network or Highly specialised technology services.
	2. The criteria should be precise, carefully defined, ‘yes/no’ criteria (where possible, but worded such that flexibility and judgement is permitted when appropriate.
	3. The HST programme should consider only technologies for which it is biologically plausible that the use will be restricted to an ultra-rare condition – for the duration of the guidance.
	4. The condition is life-threatening in that it severely limits length and/or quality of life.
	5. The condition must have a significant unmet need. Current treatment options should be limited and not very effective. There is evidence to support no satisfactory method of prevention or treatment exists, or there is a very significant limitation to current treatment (e.g. side effects, burden of therapy).
	6. It is accepted there may be exceptionally high prices for the technologies that are for exceptional circumstances where there is substantial unmet need. Therefore, technologies that can be used for wider populations and have been restricted to a narrow population for commercial reasons are not appropriate for the HST programme.
	7. To confirm suitability for HST, it is expected that available research and evidence generation will demonstrate that a technology was only ever intended for use in the very small ultra-rare population with a high unmet need.
	8. The intended group of patients should be distinguishable from other patients by characteristics other than their observed or expected response to the technology.
	9. Repurposed medicines should not normally be considered eligible except in exceptional circumstances where the research/trial burden undertaken was in an exceptionally rare condition and without this approach the technology would not have been made available for the specific very rare population.
	10. Any technology that is already licensed in another indication will be excluded from being selected for HST. If another indication is reimbursed then it should be a small ultra-rare population. Or only when there is evidence to support that the financial burden of researching in the specific condition is such that it could not be reimbursed by STA type funding.
	11. The HST programme will consider all types of technology.

Some of the current HST topic selection criteria may be considered redundant and not reflective of the current landscape and therefore it is appropriate to consider removing these as part of the decision making.

Existing criterion that requires the technology to have the potential for life-long use” and that “the condition is chronic” should be removed to enable one-off treatments such as gene therapies to be considered in the HST programme.

The cost of the technology is not normally known at the topic selection stage therefore, the cost of the technology is better considered during the evaluation stage and should not be relevant criteria for the topic selection stage.

Clinical Commissioning Group (CCG) commissioned technologies should be excluded from selection for evaluation within the HST programme.

Appendix 2 - Single Flexible Evaluation Process – TA/HST/DAP/MTEP

*Only When Beneficial*

Patient Org

Submissions

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Professional Org/ Commissioner

Submission

Independent

Assessment

Committee Meeting

Committee

Meeting

Or

Scoping Workshop

Technical Engagement

Expert

Advice

Scoping Consultation

5-20 days

Company

Submission

Guidance Published (TA/HST/MTG/DG)

Draft Final

Guidance

Public

Consultation

Glossary

This glossary provides an explanation of words and phrases used within the consultation document. For a glossary of all words and phrases used in guidance development please access the links to the programme process manuals provided in section 7 of the paper.

Carer

The term 'carer' refers to a person who provides unpaid care by looking after a relative, friend or partner who needs support because of ill health, frailty or disability.

Cancer Drugs Fund

A modified  process for cancer drugs which was implemented in line with the new operating model of the Cancer Drugs Fund introduced in April 2016. Information on the new Cancer Drugs Fund operating model is available on NHS England and Improvement's website.

CE mark(ing)

The CE mark is a mandatory conformity mark on medical device products placed on the single market in the European Economic Area. The CE mark certifies that a product has met EU safety, health or environmental requirements. CE marking will continue to be needed for devices placed on the market in Northern Ireland.

Commentator

An organisation that engages in the guidance development process but is not asked to prepare a submission. Commentators are invited to comment on the draft scope, the technical/assessment report and the consultation document. These organisations include relevant comparator technology companies, Healthcare Improvement Scotland, relevant National Collaborating Centres, related research groups and other groups as appropriate.

Commercial Arrangement

Companies who are members of the 2019 Voluntary Scheme for Branded Medicines can submit proposals for commercial arrangement as part of an ongoing or published technology appraisals or highly specialised technologies. The diagnostics programme can also receive and consider commercial proposals from companies. NICE can only consider commercial proposals after NHS England and NHS Improvement has formally approved them.

Committee

A standing advisory committee of NICE. Includes people who work in the NHS, lay members, people from relevant academic disciplines and the pharmaceutical and/or medical technology industries.

Company

The company that manufactures or sponsors either the technology being evaluated, or the comparator technology.

Comparator

The standard intervention against which the intervention being evaluated is compared. The comparator can be no intervention, for example best supportive care.

Consultation

The process that allows stakeholders to comment on draft versions of NICE guidance and other documents (for example, the draft scope) so that their views can be taken into account when the final version is being produced.

Consultee

An organisation that takes part in guidance development of a technology in the Technology Appraisal/Highly Specialised Technologies programmes. Consultees can comment during the consultation processes. Consultee organisations can nominate professional, commissioning and patient experts to present their personal views to the committee. All consultees are given the opportunity to appeal against the final appraisal document (FAD).

Cost effective(ness)

How well a technology works in relation to how much it costs.

Decision problem

A clear description of the interventions, patient populations, outcome measures and perspective adopted in a health technology evaluation, relating specifically to the decision(s) that the evaluation is designed to inform.

Department of Health and Social Care (DHSC)

The Department of Health and Social Care is responsible for standards of healthcare in the UK, including the NHS. The Department sets the strategic framework for adult social care and influences local authority spending on social care. The Department is also responsible for promoting and protecting the public's health, taking the lead on issues such as environmental hazards to health, infectious diseases, health promotion and education, the safety of medicines, and ethical issues.

Economic modelling

An explicit mathematical framework that is used to represent clinical decision problems. It incorporates evidence from a variety of sources so that the costs and health outcomes can be estimated.

European Medicines Agency(EMA)

A decentralised agency of the European Union responsible for the scientific evaluation of medicines developed by pharmaceutical companies for use in the European Union.

Evaluation

The process of evaluating the clinical, economic and other evidence about the use of a technology and to formulate guidance on its use.

Evidence

Information on which a decision or guidance is based. Evidence is obtained from a range of sources, including randomised controlled trials, observational studies and expert opinion .

Final appraisal document (FAD)

The FAD sets out the Committee's final recommendations to NICE on how the technology should be used in the NHS in England.

Guidance Executive

A team comprising the executive directors and centre directors at NICE who are responsible for approving the final guidance before publication.

Health technology

Any method used by those working in health services to promote health, prevent and treat disease, and improve rehabilitation and long-term care. Technologies in this context may be drugs, diagnostics, devices, digital technologies, and combinations of these or other technology types.

Highly Specialised Technologies (HST)

Highly specialised technologies (HSTs) are medicines designed for very rare conditions, currently guidance on HSTs are only produced on single technologies in a single indication.

Incremental cost-effectiveness ratio (ICER)

The ratio of the difference in the mean costs of a technology compared with the next best alternative to the differences in the mean outcomes.

Indication

The defined use of a technology as licensed by the Medicines and Healthcare products Regulatory Agency (MHRA) or the European Commission.

Lay member

A lay member is a committee member with a patient, service user, carer or community background. The lay member's role is the same as other committee members, and additionally includes contributing a lay perspective and highlighting patient and carer issues.

Managed Access

The process by which patients can be given access to promising medicines where the evidence base is insufficient/ too uncertain to make a final recommendation. Access is provided under strict conditions which include the requirement to gather the evidence required for NICE to make a standard recommendation on its cost effectiveness.

Marketing authorisation

An authorisation from the Medicines and Healthcare products Regulatory Agency (MHRA) to market a medicinal product.

Medicines and Healthcare products Regulatory Agency (MHRA)

The Executive Agency of the Department of Health and Social Care. It protects and promotes public health and patient safety by ensuring that medicines, medical devices, equipment and diagnostic technologies meet the appropriate standards of safety, quality, performance and effectiveness, and are used safely.

National Institute for Health Research – Health Technology Assessment Programme

The National Institute for Health Research – Health Technology Assessment (NIHR HTA) is part of the NIHR Evaluation, Trials and Studies Coordinating Centre (NETSCC) based at the University of Southampton. The NIHR HTA coordinates the Health Technology Assessment Programme on behalf of the NIHR. The aim of the programme is to ensure that high-quality research information on the costs, effectiveness and broader impact of health technologies is produced in the most efficient way.

NICE Scientific Advice

NICE Scientific Advice provides paid-for advice to companies and other organisations on how to develop the evidence base that demonstrates the value of technologies to patients and the healthcare system.

Outcome

A measure of the possible results of a treatment with a preventive or therapeutic intervention. Outcome measures can be either intermediate or final end points.

Patient expert

Acts as an expert witness to the Committee. Patient experts have used the technology either personally or as part of a representative group. Patient experts attend as individuals; they may be either somebody with personal experience of the condition, and if possible, the technology, or a member of a patient and carer organisation for the condition being appraised.

Professional expert

Professional experts act as expert witnesses to the Committee. They are selected on the basis of specialist expertise and knowledge of the technology and/or other treatments for the condition. They provide a view of the technology within current clinical practice, and insights not typically available in the published literature.

Public Involvement Programme (PIP)

The PIP is the team at NICE that supports and develops public involvement across NICE's work programme. A PIP public involvement adviser is assigned to each appraisal and supports patient and carer consultee organisations, their representatives, and individual patients or carers throughout the appraisal. The PIP public involvement adviser also supports the lay members of the appraisal committees.

Scope

Provides a detailed framework for the evaluation and defines the disease, the patients and the technologies that will be covered by the evaluation. The questions the evaluation aims to address are also part of the scope.

Stakeholder

An organisation that takes part in guidance development.

Technical engagement

When the technical report is sent to stakeholders and experts to seek their views on the judgements made by the technical team and to specify any remaining clinical uncertainties.

Technical team

A team comprising members of the NICE  staff, who are responsible for considering submissions and providing preliminary scientific judgements and advice to the appraisal committee.

Technology appraisal

The process of developing recommendations on the use of new and existing health technologies within the NHS in England (also see 'Evaluation'

Technology assessment

see 'Evaluation'.

Terminated guidance

The standard guidance development process relies on companies submitting evidence, in line with NICE's specification. Occasionally, they do not make a submission or the submission does not meet the required specification. The evaluation is therefore terminated and NICE asks NHS organisations to take into account the reasons why the company did not make an evidence submission when making local decisions on whether to make the technology available.

Topic Selection

The process by which NICE determine which technologies it should develop guidance on.

UK conformity assessment (UKCA) mark

A UKCA mark indicates that medical device is fit for its intended purpose and meets legislative requirements. It also allows for the device to be marketed in Great Britain.

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