Briefing and messages

March 2021

NICE Process Review

Executive Summary

- NICE must ensure there is a robust health technology assessment (HTA) approach in place for making decisions about which new medicines represent value-for-money and should be paid for on the NHS.
- NICE has launched a 10-week consultation on proposed changes to its processes for evaluating health technologies (closing date 15 April). NICE's aim is to have faster, flexible and more responsive processes to evaluate new health technologies and bring them to patients sooner[1].
- This consultation is a critical opportunity to consider how NICE's processes link into the Government's ambition for the UK to be a leading regulator of new medicines approvals now we have the left the European Union, including the MHRA's new Innovative Licensing and Access Pathway (ILAP). A well joined-up system for licensing and reimbursing medicines will send a powerful signal about the UK's status as a science superpower.
- ABPI welcomes proposals to make NICE's processes more flexible and adaptive but this must be done in a way which includes companies in decision making. There are positive ambitions to improve stakeholder input, including better communications to patient and carer organisations and the introduction of a Summary of Information for Patients.
- ABPI is highly concerned by the proposed principles on which to update the Highly Specialised Technologies (HST) programme criteria, which evaluates medicines that treat very rare diseases. NICE should simplify the criteria so the HST programme is available for all treatments used in ultra-rare patient populations. This will help meet the ambitions of the Government's recently published Rare Disease Framework.
- We have concerns about the proposals to shorten consultation timeframes and make the technical engagement step available for only some appraisals. We do not think this will provide stakeholders with sufficient time to input their views and to ensure critical issues in the evidence base are resolved early in the appraisal process. Increased speed must not be at a cost to appropriate levels of stakeholder input.
- Separately, NICE must take forward and implement the proposed changes to appraisal methods, which are also critical in moving the dial for access to innovative medicines. We want to see NICE delivering on its proposals to introduce modifiers, accept greater uncertainty in some circumstances (including when treatments are developed for rare

[1] Reviewing our process for health technology evaluation: consultation | NICE guidance | Our programmes | What we do | About | NICE

diseases), and change the discount rate used to be in line with the latest evidence and Treasury's guidance[2].

- These changes will also send a strong signal to global life sciences companies that the UK is supporting innovation and is an attractive market for early launch of new medicines.

Context

- NICE must ensure there is a robust health technology assessment (HTA) approach in place for making decisions about which new medicines represent value-for-money and should be paid for on the NHS.
- NICE is currently undertaking a review of the methods and processes it uses across its technology evaluation programmes to ensure that its work remains cutting edge and able to deal effectively with innovative technologies as they emerge.
- Through 2019-20 there has been a strong focus on the Methods Review and a consultation on NICE's proposals for changes to its methods completed at the end of last year. Work is ongoing to consider the response to this consultation and determine how the proposals will be taken forwards and implemented.
- NICE has now published its Process Review consultation (a ten week consultation closing on 15 April)[3]. There are important proposals in this consultation which will have an impact on the overall success of NICE's Methods and Process Review.
- The Methods and Process Review is critically important because it will set the framework for how the UK will provide access to new and breakthrough medicines for NHS patients for years to come.

NICE must do more to support access for patients with very rare diseases through the HST programme

- NICE evaluates most medicines through its Single Technology Appraisal (STA) programme. This process is not suited to support the evaluation of very specialist medicines for rare diseases.
- In recognition of this challenge, in 2013 NICE launched the Highly Specialised Technologies (HST) programme. It provides a more flexible approach, with a higher cost-effectiveness threshold than the TA programme and has enabled several very specialist rare disease medicines to reach patients.
- There remains however a significant challenge for new medicines that treat very rare diseases which do not meet all the HST criteria but are not suitable for the TA programme. Addressing this 'gap' between TA and HST has been a key issue for several years. Under the current Process Review, the HST 'criteria' - used to determine whether a medicine is evaluated using the HST programme instead of the TA programme - are being reviewed. The current criteria are convoluted and open to misinterpretation and do not facilitate transparency in how HST/TA routing decisions are made.

[2] For further information please visit What is the NICE Methods Review? | ABPI

[3] Reviewing our process for health technology evaluation: consultation | NICE guidance | Our programmes | What we do | About | NICE

- In the consultation, NICE has recognised the HST criteria need to be clearer to stakeholders. However, the principles proposed will not meet the stated objective of providing *"fair and equitable access to treatments for patients with serious and severe ultra-rare conditions…who would be disadvantaged by an appraisal undertaken via the standard appraisal process".*
- **NICE should simplify the criteria so the HST programme is available for all treatments used in ultra-rare patient populations.** This will help support the ambition set out in the Government's recently launched Rare Disease Framework[4], which states that "ensuring continued development and improves access to specialist expertise, treatment and drugs will require innovation".
- The outcome of the Methods Review which contains key proposals, including introducing modifiers, changing the discount rate, and accepting greater uncertainty for rare disease medicines, is critically important to support access to rare disease medicines which do not meet the HST programme criteria and must be evaluated via the TA programme.

NICE should create a more joined-up approach that does not shorten consultation timeframes unnecessarily

- The consultation is seeking views on ways of working to adapt to the evolving post EU Exit environment. ABPI believes this is a key opportunity to consider how NICE's appraisal processes can map onto the MHRA's new accelerated pathways, including the Innovative Licensing and Access Pathway (ILAP)[5], Project ORBIS and the ACCESS Consortium.
- A joined-up approach is needed that provides an aligned system view on the risks and opportunities for early access to innovative new medicines, with processes that enable a streamlined access pathway. Further clarity is needed on how its processes will link into and meet the ambition of the ILAP.
- NICE has demonstrated its ability to be agile in response to the COVID-19 pandemic. Working virtually to develop guidance has proven to be successful and provides opportunities to better support stakeholder input, particularly for clinicians and patients/patient representatives. It is important that NICE is clear on how patient and carer input impacts on decision making.
- To produce rapid guidance to support treatments for COVID-19, NICE has had to shorten process timelines, particularly for stakeholder consultation. ABPI disagrees with taking this approach forwards into NICE's 'business as usual' processes. Proposals to shorten consultation timings during the appraisal process will not provide stakeholders with sufficient time to input their views and will be particularly challenging for patients, the clinical community and companies. Such engagement is important to help NICE

[4] UK Rare Diseases Framework - GOV.UK (www.gov.uk)

[5] The Innovative Licensing and Access Pathway (ILAP) aims to accelerate the time to market, facilitating patient access to medicines. The ILAP awards a new "innovative medicine" designation (Innovation Passport), utilises tools from a toolkit and a "Road Map" (Target Development Profile). The pathway brings together innovative approaches to support the safe, timely and efficient development of innovative products Innovative Licensing and Access Pathway (ILAP) for medicines - GOV.UK (www.gov.uk)

understand, for example, the impact of the disease and current clinical practice. Shortening parts of the process at the critical scoping stage of an appraisal also risks introducing bigger issues and delays further on in the appraisal.

- ABPI welcomes NICE's proposals to introduce a Summary of Information for Patients and improve communications to patient and carer organisations, including finding a mechanism for providing feedback following their input.

NICE should be sufficiently resourced to deliver its appraisal programmes without needing to reduce stakeholder input

- Pharmaceutical companies pay a substantial amount of money to NICE for every appraisal undertaken on their medicines. The premise for introducing fees for appraisals was that NICE could operate the TA and HST programmes in a sustainable and efficient way that allowed it to be more responsive to developments in the life sciences sector. It is therefore important the Process Review does not remove/reduce time for important components of the appraisal process.
- When NICE reviewed the TA programme process in 2017/18, a "technical engagement" step was added into the process to enable key technical issues to be discussed (and where possible resolved), ahead of the Appraisal Committee meeting. This is a highly beneficial part of the process and ABPI considers it should be prioritised and utilised for all appraisals. Introducing an option to remove it for some appraisals may create unfairness and will not support resolving technical issues earlier on in the process. NICE needs to adequately resource this process so that is properly implemented as originally envisaged.

ABPI welcomes clarity on NICE's Commercial and Managed Access processes and supporting the implementation of the Innovative Medicines Fund

- The Process Review provides the opportunity to clarify how NICE and NHSE&I's processes are joined up to provide a clear, coherent route to patients for medicines which require commercial and/or managed access arrangements. These processes need to be clearly described and set out.
- Expanding the approach taken for the Cancer Drugs Fund so that funding is available to provide early access to promising non-cancer medicines, as well as cancer medicines, will enable the implementation of the Government's commitment for an Innovative Medicines Fund. This is critical to ensure an equitable system for treatments in all disease areas. Clear and flexible arrangements for managed access will also be essential to capture the benefits of rapid licensing pathways.

For more information please contact Vicky Whitehead vwhitehead@abpi.org.uk