NICE Methods and Process Review Consultations: Key Messages

August 2021

Executive summary

- The three consultations on NICE's proposals for changing its methods and processes for health technology evaluation are the final step in a two year programme of work. They are highly significant as they will set the framework for how NICE decides which new medicines will be made available to NHS patients for years to come.
- Overall, the consultations set out many positive changes which will help support timely access to new medicines. In particular, ABPI is pleased to see that the proposals to introduce a severity modifier and to accept greater uncertainty in some circumstances, including for rare disease medicines, have been taken forwards following broad support in the first Methods Review consultation. However, there is room for NICE to go further with more ambitious proposals.
- There are two priority areas of concern in the consultation that will restrict patient access to medicines, and hold back the UK's ambition to be a global life sciences hub:
    - **The proposed criterion that medicines can only be routed to the highly specialised technologies (HST) evaluation programme if they have an eligible patient population of less than 500 across all of their licensed indications**. This is arbitrary and could prevent patients with very rare conditions having access to the medicines they need.
    - **The decision to retain the current discount rate at 3.5%** despite NICE's view that there is an evidence-based case for change, because of perceived policy and system barriers. This means that the long term value of many types of new medicine, such as cell and gene therapies, will be inadequately assessed.
- In the consultation, NICE calls for further discussion with wider system stakeholders on changing the discount rate. ABPI urges DHSC, NHSE&I and others to work constructively with industry and utilise the guarantees afforded by the Voluntary Scheme for Branded Medicines Pricing and Access to enable NICE to change the discount rate, aligning to the Treasury Green Book and latest evidence.

What is the new Programme Manual and why is it important?

- NICE ensures we have a robust, transparent and inclusive framework in place for making decisions about which new medicines represent value-for-money and should be paid for on the NHS.
- The proposed changes to NICE's methods and processes and the resulting Programme Manual bring together several years of work and will set out how NICE will evaluate medicines and other healthcare technologies for years to come, ultimately deciding what treatments will be made available to patients.
- Change is needed because the medicines the pharmaceutical industry is researching and developing have dramatically changed over the last ten years. Medicines are being developed to treat patients earlier on in the disease pathway, with novel mechanisms of action that in some cases are potentially curative. In addition, these medicines have gone from being predominantly treatments for long-term chronic conditions and late-stage cancers, to more targeted therapies for complex, sub-diseases with small patient populations.
- These advances and the way evidence about them is generated can bring complexity and challenges in NICE appraisals. The new manual aims to overcome some of these challenges, provide more guidance to companies, and enable NICE to appropriately evaluate innovative medicines as they emerge.

What are the key proposals?

- The consultation papers build on the proposals made in previous consultations. Many of the proposals are very positive and, when taken in the round, will support the evaluation of medicines in the future.

Methods

- Of note, proposals to introduce a severity modifier and accept greater uncertainty in some circumstances (for example when evaluating innovative technologies and rare disease medicines), have the real potential to benefit patients by supporting access to life changing medicines. NICE will also be doing further work on a health inequalities modifier, to ensure the value of reducing health inequalities is more clearly considered within decision making.
- Helpful proposals have been made to provide more guidance and flexibility on the evidence NICE considers in its evaluations, including the use of real-world data. In providing more flexibility, NICE will increasingly be able to answer key questions and address gaps in the evidence that is available from clinical trials. This is really important for the ambition to make new medicines available to patients as quickly as possible.

- New methods are being introduced to help with challenging technologies, conditions and evaluations. These positive changes will support companies with their submissions and how to best present evidence from innovative trial designs, such as those being used for histology independent cancer medicines.
- Positive steps are also being made towards a more pragmatic approach to NICE's decision making framework in complex scenarios where, for example, a medicine cannot be considered cost effective even if it is priced at zero, or there are circumstances when broader system costs (for example, panel genetic testing and platform set up) are included but should be adjusted in the evaluation.
- Several proposals which we considered unhelpful such as the inclusion of overly complex technical analysis which did little to support the decision making process of appraisal committees have not been taken forward, e.g. the complex topic of "expected value of perfect information".

Severity modifier

- Modifiers are factors that affect NICE's decisions on health technologies. The consultation proposes a new severity modifier to replace the current end of life modifier. This is in general terms a positive change which provides a broader definition of severity. It will benefit patients with a wider range of conditions, for example musculoskeletal, inflammatory and mental health, in addition to cancer (which the current end-of-life modifier mostly focusses on).
- NICE has proposed the severity modifier is implemented in a way that is cost neutral, prior to additional research being done to understand by how much society attributes more value to severe conditions. We consider the guarantees on spending afforded by the Voluntary Scheme for Branded Medicines Pricing and Access (Voluntary Scheme), and other cost containment policies already in place, should allow for greater ambition than this, and for the new modifier to benefit more medicines than is currently being proposed. This would better align with the Prime Minister's Life Sciences Vision and make a really positive impact for patients with the most devastating diseases.

Managing uncertainty

- Earlier licensing approvals and changes to the type of medicines coming to market - for instance with medicines being developed for more targeted and smaller patient populations - create challenges in the associated level of uncertainty in their evidence base at the time of their NICE appraisal.
- In recognition of this, NICE will apply more flexibility when considering evidence in circumstances concerning medicines and patient populations for which evidence generation is particularly difficult. This will support patients with rare diseases and

companies developing medicines in areas of scientific breakthrough and is very welcome.

- NICE will work on a visualisation framework to better characterise and present uncertainty to its committees for implementation as part of the appraisal process. This will help support decision making.

Discount rate

- NICE maintains their "view that there is an evidence-based case for changing the reference-case discount rate to 1.5% for costs and health effects", but due to wider policy and system implications the decision has been made to retain the current discount rate at 3.5%.
- ABPI is very concerned that this will mean that the long term benefits of treatments such as cell and gene therapies will not be appropriately valued. The current discount rate undervalues the longer-term benefits that medicines offer patients and their families and makes it difficult for innovations like cell and gene therapies to be recommended by NICE.
- Without a change to the discount rate, we do not believe the aim of the review – "to support the ambition of the NHS to provide high quality care that offers good value to patients and to the NHS" - will be met.
- ABPI calls on system stakeholders to do more work together with industry, ensuring there is full consideration of the guarantees afforded by the current Voluntary Scheme, to enable NICE to make the proposed change to the discount rate with the implementation of the new manual from January 2022 onwards.

Processes

- ABPI supports NICE's decision to maintain the rigour and inclusiveness of its technology appraisal processes. A good balance has been met in retaining consultation timings and important process steps such as technical engagement, whilst opening up flexibilities when these can be applied to support faster access to the most promising new medicines.
- A comprehensive level of process detail around commercial and managed access arrangements is welcome and will support all companies in understanding how to best engage early and develop these agreements to prevent unnecessary delays for patients.
- Beyond this current consultation, there are some new ways of working that need to be developed to create efficiencies and ensure NICE's work programme is sustainable. For example, more streamlined approaches are needed for developing guidance for medicines which have many indications; a mechanism should be available to fast track some medicines into managed access agreements ahead of a full NICE appraisal; and a

more efficient process is needed for reviewing guidance when biosimilars are launched. ABPI stands ready to work with NICE on these additional components so that they work for industry.

Topic selection - HST criteria

- NICE evaluates most medicines through its Single Technology Appraisal (STA) programme. This programme is not suited to support the evaluation of very specialist medicines for rare diseases. In recognition of this challenge, NICE has a highly specialised technologies (HST) evaluation programme that provides a more flexible approach and a higher cost effectiveness threshold than the TA programme.
- The topic selection and routing part of the consultation proposes four new criteria to assess whether a medicine can be routed to the HST evaluation programme. ABPI is concerned that one of these criteria – that the eligible patient population must normally be no more than 300 people in England for the indication and no more than 500 people across all of the medicine's indications – is both arbitrary and unnecessarily restrictive and could prevent many medicines being developed for very rare conditions from being able to benefit from a HST evaluation. We do not think this criterion should be included in the new topic selection manual.

How does this consultation link with the Innovative Medicines Fund (IMF)?

- Alongside the changes being made to NICE's methods and processes, a new Innovative Medicines Fund (IMF) is being implemented following a commitment made by the Government.
- Some innovative medicines struggle to be recommended by NICE because of the limited evidence available at the time of the evaluation. This challenge is amplified for medicines breaking into new therapeutic territories, treating very small patient populations and that offer the potential for long-term survival or even a potential cure for life-threatening diseases.
- The IMF will complement the Cancer Drugs Fund, enabling patients with conditions outside of cancer to benefit from early access when further data is needed to support NICE in making a recommendation for their use in the NHS.
- The proposals NICE has made around their processes for managed access are important in defining how the IMF will be implemented.
- To ensure the fund is sustainable and the right medicines go into it, the IMF must be introduced in addition to wider changes being made in NICE's new manual, particularly those which help NICE manage and accept greater uncertainty in some circumstances.

Conclusion

The three consultations taken in the round present a significant opportunity to ensure NICE's methods and processes are cutting edge and able to support NHS patients having timely access to life changing medicines, whilst delivering on the ambition set out in the UK's Life Sciences Vision. We look forward to all stakeholders engaging with the consultation process to ensure this can be the case.

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