NICE Methods and Process Review Outcome: Key Messages

Executive summary

- In January 2022, the National Institute for Health and Care Excellence (NICE) published a new Manual, describing the methods and processes it follows to evaluate health technologies.
- This Manual followed an extensive two-year review, which was initiated to consider the latest evidence and ensure NICE's methods and processes remain fit for purpose against the backdrop of the scientific advances being made by the life sciences industry.
- The new Manual introduces several positive changes, including introducing a severity modifier and some welcome flexibilities such as accepting greater uncertainty in some circumstances, including for rare disease medicines, and better consideration of evidence sources beyond randomised controlled trial data.
- However, the ABPI is concerned that overall, the outcome of the review has not met the level of ambition that was anticipated, and clearly set out in the Government's Life Sciences Vision. This, coupled with the implementation of the new Manual within a cost neutral envelope will restrict patients' access to some types of new medicines and hold back the UK's ambition to be a global life sciences hub.
- The priority areas of concern are:
    - **The decision to retain the current Discount Rate.** This is not in line with the Treasury Green Book and latest evidence, despite NICE being clear that the evidence base supported making a change (from 3.5% to 1.5%). This means that the long-term value of many types of new medicine, such as cell and gene therapies, will be inadequately captured.
    - **The new criterion for access to the Highly Specialised Technologies Programme.** The new criteria will restrict the number of medicines for very rare diseases being evaluated using the additional flexibilities this programme provides. This will slow down, or even potentially halt, the introduction of new medicines for very rare conditions where patients will often have no alternative treatment available.
    - **The introduction of the severity modifier in an 'opportunity cost neutral' way.** This means some end-stage cancer patients may miss out on last resort treatments, due to the retirement of the end-of-life modifier.
- NICE is taking a new "modular" approach for updating the new Manual, which could help support continuous improvements. This needs to be implemented quickly - with input from stakeholders - and a sharp focus and ambition to make progress on access to medicines

for all NHS patients. Future changes must address the significant missed opportunities in the recent review and the feedback NICE received from stakeholders across the life sciences and patient sector.

What is the new 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 changes to NICE's methods and processes set out in the new Manual, bring together several years of work and set out how NICE evaluates medicines and other healthcare technologies, ultimately deciding what treatments will be made available to patients.
- Continuous improvement is needed because the medicines the pharmaceutical industry is researching and developing are advancing at a fast pace. 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 for NICE appraisals.
- The new Manual may help overcome some of these challenges and provides more guidance to companies in some areas.
- However, there are significant missed opportunities and changes that have been made which do not reflect the feedback from industry and other stakeholders made to the consultation.

What improvements have been made?

- Some of the updates in the new Manual are positive and, when taken in the round, will help support the evaluation of medicines in the future.
- These include:
    - NICE's independent Appraisal Committees accepting a higher degree of uncertainty when evidence generation is particularly difficult, for example in rare diseases and for innovative and complex medicines.
    - Supporting the use of more comprehensive evidence base for decision making, including real-world evidence sources and the lived experiences of patients.
    - Clarifying that NICE accepts health effects of carers as well as patients in the evaluation of a medicine is a welcome step forward.

- Better flexibilities for challenging or unusual scenarios. For example, NICE being unable to recommend a medicine even if it were priced at zero and when there are costs to the healthcare system that should not be fully borne by one individual medicine alone.

- The implementation of these flexibilities in practice needs to be closely monitored.

Areas of concern which require ongoing focus as a priority:

Severity Modifier

- Modifiers are factors that affect NICE's decisions on health technologies. The Manual introduces a new severity modifier, replacing the end of life modifier. This is in general terms a positive change because it provides a broader definition of severity and will benefit patients with a wider range of conditions, for example musculoskeletal, inflammatory and mental health, in addition to cancer (which the end-of-life modifier mostly focussed on).
- However, it has been introduced in an "opportunity cost neutral" way, limiting its impact and meaning some end-stage cancer patients may miss out on last resort treatments.
- Monitoring the application of the modifier in practice and progressing the additional work NICE has identified as necessary to inform further broader evolution of the modifier needs to be a high priority.

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%".
- The ABPI is very concerned that this change has not been able to be made. It 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, the ABPI does 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" - has been met.
- The ABPI calls on system stakeholders to do more work together with industry, to enable NICE to make the proposed change to the discount rate.

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 revision of the Highly Specialised Technologies (HST) selection criteria risks preventing some medicines treating very rare diseases from being able to enter this evaluation programme. The impact of this change needs to be closely monitored to consider whether further adjustments are needed.
- There remains a particular challenge for rare disease medicines which will not meet the HST entry criteria nor benefit from the new severity modifier.

Conclusion

- Swift implementation of the Manual is now needed, with a fair approach taken to managing the transition from previous to new methods, for example so those patients currently benefiting from end-of life medicines in the Cancer Drugs Fund do not lose out.
- The ABPI welcomes NICE's commitment to closely monitor and review the impact of the changes to ensure they are working in practice as intended.
- Finally, the Life Science Vision set out to make the UK a leading global hub for life sciences. The role of NICE and the methods it uses to evaluate new medicines are observed internationally, signalling to global investors the extent to which the UK values innovation. Currently the UK has one of the lowest levels of investment in medicines[1]. This funding gap versus comparable countries needs to be addressed by government for NICE and the NHS to be seen as world leading.
- We look forward to working together to make this happen so that patients in the UK can benefit from early and sustainable access to new medicines.