# SHCA position paper on the NICE review

## Introduction

The Specialised Healthcare Alliance (SHCA) represents patients with rare and complex conditions, many of whom require sometimes-sophisticated medical treatments on a short term or ongoing basis. For many of these conditions, there are few, if any, treatment options available. Patients therefore rely on the health service to make new treatments available to them whenever they can, to give them a chance of a better life.

In England, the process for taking decisions on which new treatments should be made available has evolved gradually over an extended period of time. The creation of NICE by the Government in 1999 represented an acknowledgement of the need for expert independent advice and national standards in these decisions. NICE was tasked with ensuring that recommendations were based on the best scientific evidence and that patients were treated equitably, in line with the founding principles of the NHS.

The establishment of NICE was a welcome step for patients in England, who had too often faced 'postcode lotteries' in relation to access to both treatments and services, due to high levels of variation in approaches taken by their local NHS organisations. Patients' right to medicines approved by NICE is now a cornerstone of the NHS Constitution, underpinned by the legal requirement for NHS organisations to fund recommended medicines.

However, the NICE that exists today is markedly different from that which was created 20 years ago. While the core principles of its approach have remained unchanged, NICE's process and methods have undergone a series of updates over time. These have primarily been driven by changes in the nature of the medicines that it has been tasked with assessing. From medicines used at the end-of-life, to targeted cancer treatments, to treatments for ultra-rare conditions, NICE has gradually developed a range of different approaches to the assessment of new treatments that are designed to provide more flexibility in evaluation.

While the establishment of these flexibilities has been a hugely welcome step for patients, the SHCA believes that further reform is now needed to ensure that similar arrangements are introduced for patients with rare diseases. As advances in science unlock increasingly personalised medicine, the proportion of targeted treatments for small populations is likely to continue to grow. Without further change, there is a risk that patients will face unfair barriers to new treatments.

The SHCA therefore welcomes the launch of NICE's review of its methods as an opportunity to update its approach to ensure that patients with rare and complex conditions are treated fairly, and to safeguard against the denial of access to medicines simply because a condition happens to be rare.

This paper sets out:

- Background to the NICE review
- Key issues that the SHCA believes must be addressed by the review
- Principles that should guide NICE in developing solutions to these challenges
- Conclusions

**Background**

In July 2019 NICE confirmed that it had launched a review of its methods for health technology evaluation.[i] The review will consider changes to the ways in which NICE makes decisions on which medicines, technologies and diagnostics the NHS should make routinely available to patients.

The scope of the review is therefore wide-ranging; however, the methodology that NICE uses to assess treatments for rare and ultra-rare diseases is expected to be a key area of focus. In part, this is due to a number of recent high-profile cases of medicines not being made available to patients due to disagreements between pharmaceutical companies and the NHS about the way in which they should be assessed.

In addition, there are several other factors that make a re-evaluation of NICE's approach to rare disease treatments timely, as follows:

- First, NICE is due to take on responsibility for the assessment of all rare disease medicines from 2020. Currently, many rare disease treatments are not assessed by NICE, with NHS England instead evaluating them through its policy development process. This function is due to transfer to NICE in 2020 and NICE therefore needs to ensure that its methods are suitable for these medicines

- Second, the impact of the changes made to the evaluation of treatments for ultra-rare conditions in 2017 is becoming clearer, with a growing number of treatments being rejected or facing extended delays in assessment. There is therefore a need for NICE to consider whether further changes are necessary to ensure that patients do not lose out due to standoffs between the NHS and pharmaceutical companies

- Third, other UK nations have introduced processes that provide greater flexibility in the assessment of rare disease treatments and more involvement for patients in the decision-taking process. There is therefore a need for NICE to assess whether similar changes should be put in place to ensure that patients in England are able to benefit from a comparable approach

Encouragingly, the Government has indicated that rare diseases will be a specific area of attention within the review, with Baroness Blackwood telling the House of Lords in May 2019, "*I have asked [NICE's Chief Executive] to ensure that the review takes into account the benefits offered by new treatments for severe life-threatening and rare diseases, so this area will not go untested*".[ii]

NICE has since confirmed that it will be considering a number of areas in detail that are particularly important for the assessment of rare disease medicines, including:

- The **'modifiers'** that can be applied to calculations of cost-effectiveness to provide more flexibility than NICE's standard 'threshold' of cost effectiveness – currently, such modifiers exist for the assessment of medicines used at the end of life and for ultra-rare disease medicines

- How it approaches **uncertainty** in assessing the benefits of a new treatment – due to the low numbers of patients affected by rare diseases, it is harder to gather evidence on the impact of a treatment, creating challenges for NICE's exacting approach to

decision-taking, which relies on levels of data that often do not exist for rare disease treatments at the point at which they are assessed

- How it assesses **health-related quality of life** – NICE's current approach is based on being able to accurately measure how any given treatment affects an individual's quality of life, but the way in which it does this has been criticised for not being suitable for rare diseases, especially those that affect infants and children[iii]

The SHCA and its members look forward to contributing to the review and hope that it will result in proposals that provide NICE with the tools it needs to assess rare disease medicines appropriately and ensure patients with rare diseases can have confidence in the system moving forward.

To deliver this outcome, we believe that there are a series of key issues that the review must address.

**Key issues**

Through our work with our member organisations, the SHCA has identified several areas in which rare disease patients and the charities that represent them believe reform is needed:

- Flexibilities available to NICE in assessing rare disease medicines
- NICE's inconsistent approach towards uncertainty in clinical evidence
- Patient involvement in decision-taking
- Funding for rare disease treatments

Each of these issues is explored in detail below.

*Flexibilities available to NICE in assessing rare disease medicines*

The current system for the appraisal of medicines for rare diseases in England is highly complex. The level of flexibility that is available to decision-takers depends on the process through which it is assessed:

- For ultra-rare disease treatments assessed through NICE's Highly Specialised Technologies (HST) programme, NICE is able to take a more flexible approach to cost-effectiveness than its standard programme. However, a medicine must meet a set of very narrow criteria to enter the HST programme, meaning that only a very small number of treatments qualify. In addition, the introduction of a cost-effectiveness threshold in 2017 has resulted in an increasing number of negative recommendations and lengthy delays to decision-taking – this contrasts with the approach recently introduced in Scotland, where a new pathway providing rapid conditional access for a time limited period has recently been introduced for ultra-rare disease treatments[iv]

- For rare disease treatments assessed through NICE's standard Technology Appraisal programme, additional flexibilities are only available if the treatment will be used at the end of a patient's life or is a cancer medicine – this is different to the approach taken in Scotland and Wales, where additional flexibilities are applied both to end-of-life medicines and medicines for rare diseases

- For rare disease medicines assessed through NHS England's commissioning policy development process, the 'relative prioritisation' stage of the process is inherently challenging for treatments with small patient populations as it prioritises investment in treatments with the lowest costs per patient and the most substantial evidence bases

NICE's own Citizen Council, intended to represent wider societal views on NICE's approach, has also demonstrated support for greater flexibility in the assessment of rare disease medicines in the past, particularly where the condition in question has a severe impact on quality of life or where the treatment is life-saving.[v]

**89%** of respondents to a recent SHCA survey highlighted a need for greater flexibility in assessment processes to reflect the small patient population sizes of rare diseases[vi]

While the transfer of responsibility for assessing rare disease medicines from NHS England to NICE is a welcome step in reducing the complexity in the system, it is vital that this transfer of responsibility is accompanied by changes to the methods of assessment to ensure that NICE is able to fairly assess these treatments. There is therefore a need for NICE to introduce a new formal mechanism through which greater flexibility could be applied in the assessment of rare disease treatments, while also taking steps to address the challenges that have arisen in the HST programme since the introduction of the threshold in 2017.

*NICE's inconsistent approach towards uncertainty in clinical evidence*

At its core, NICE's approach to assessing whether a new medicine should be made available to NHS patients is based on weighing whether the benefits that it brings, based on an evaluation of the clinical evidence, are justified when set against the cost to the NHS.

Traditionally, NICE has set very high standards for the type and volume of evidence that it will accept to inform its decision-taking. Its preference has been for strictly controlled large-scale clinical trial data, to ensure that the evidence is as clear as possible on the benefits of the treatment in question, independent of any other factors.

**67%** of respondents to our survey said it was not very fair, or not fair, to have a system where rare disease treatments are assessed by different methodologies and budgets depending on the route they are scoped into[vi]

However, as more treatments for smaller numbers of patients have been developed, challenges with NICE's traditional approach have arisen. For many of these treatments, it is simply not possible to generate the types of data that NICE has requested, due to the very low numbers of patients involved and the absence of other effective medicines to compare against. As a result, there are much higher levels of uncertainty about the effects of these new medicines at the point at which NICE evaluates them.

This challenge has been recognised by NICE and steps have been taken to enable it to make a recommendation for a time limited 'conditional' period of access to medicines where there is a high level of uncertainty, while further evidence is gathered, after which time a follow up evaluation is carried out. During the conditional period, the NHS pays a lower price than it would for routine access, reflecting the uncertainty about the effectiveness of the treatment. From the patient perspective, this is a welcome and

pragmatic approach, as it prioritises access, while ensuring that the NHS does not overpay in the short- or long-term.

However, this option to address the uncertainty challenge is only available to NICE in certain circumstances, particularly in respect of cancer medicines, which are eligible to receive interim funding through the Cancer Drugs Fund or treatments assessed through HST, where Managed Access Arrangements can be put in place. There is therefore a need for steps to be taken to address this inequity and ensure that NICE is able to apply the same approach to all medicines for rare diseases moving forward.

*Patient involvement in decision-taking*

Due to the challenges involved in carrying out traditional appraisals of rare disease medicines, the patient voice is a hugely important resource for decision-takers. Patients are able to help decision-takers fill gaps in the data and understand what the condition in question is like for those living with it on a daily basis. They can also provide a unique perspective of the benefits of the proposed treatment in practice, based on their lived experience of receiving the treatment through a clinical trial.

NICE has a strong track record in patient engagement. Patients and patient organisations are invited to provide both written and verbal evidence to NICE committees in the course of technology appraisals, while NICE's Patients Involved in NICE coalition of patient organisations and Public Involvement Programme provide forums for NICE to hear directly from patient organisations and patients about the issues that matter to them.

In the case of the HST programme, patient organisations have on occasion been more closely involved in the formation of NICE's recommendations, including through being signatories to the Managed Access Agreements setting out the terms on which patients will be able to receive the drug.[vii]

However, the extent to which patients and patient organisations are involved in the development of NICE's recommendations on rare disease treatments varies significantly. There is no standard process that gives formal weight to patient evidence or opinion, unlike in Scotland where 'Patient and Clinician Engagement' meetings bring experts together to provide advice on factors that may not have been captured by the data analysis, or Wales, where 'Clinician and Patient Involvement Group' (CAPIG) meetings fulfil a similar function.

The SHCA believes NICE should take the opportunity of the methods review to evaluate how it could strengthen the patient voice in decision-taking, particularly in the assessment of rare disease treatments.

**Over 60%** of respondents to our survey told us that the changes introduced in Scotland had improved access to treatment

Survey respondents also highlighted the constructive nature of engagement, and the willingness to engage with patient organisations as key characteristics of the Scottish system[vi]

*Funding for rare disease treatments*

As treatments for rare conditions are, by definition, used in small numbers of patients, their per-patient cost tends to be higher than for treatments for more common

conditions. The relatively high per-patient costs of treatments for rare diseases creates challenges both for NICE, whose traditional approach to cost-effectiveness is not well equipped to assess these medicines and to the wider health service that has to find the funds necessary to pay for a growing number of rare disease treatments.

To date, the NHS has sought to manage cost pressures by introducing additional controls into the NICE process, including the creation of the 'Budget Impact Test' and the establishment of a threshold above which the NHS is unwilling to pay for ultra-rare medicines through the Highly Specialised Technologies programme.

While the SHCA understands that challenging financial pressures on the NHS have been the driving cause behind these changes, in practice they could result in patients facing delays to receiving new treatments or in the worst case being denied access altogether. We therefore believe that NICE should work with NHS England and the Government to explore alternatives that do not put patient access to treatment at risk.

This should include reviewing the approach pursued in Scotland, where the Scottish Government has established a 'New Medicines Fund' to help finance the cost of new rare disease medicines. The Fund is resourced through rebates paid by pharmaceutical companies under the terms of the UK-wide agreement between the pharmaceutical industry and government, which effectively acts as a cap on NHS spending on medicines.

In England, there is currently no mechanism in place to ensure that these rebates are utilised to support patient access to medicines, so this is an area that merits further consideration. By utilising these rebate payments, the NHS could both safeguard access to treatments for patients and wider NHS resources.

**Guiding principles for NICE methods reform**

In light of the issues set out above, the SHCA believes that there is a pressing need for reform of NICE's methods for the assessment of rare disease medicines. In consultation with our 120 patient organisation members, we have set out below the wider principles that we believe should guide NICE, NHS England, and the Government in developing a system that works for patients with rare diseases:

1. <u>There must be a 'premium for rarity'</u>

   NHS England and NICE must ensure that all treatments of equivalent clinical effectiveness are made available in a fair and equitable way whether they treat people with more common conditions, or people with rare conditions. Given treatments for rare conditions tend to be more expensive than treatments for more common conditions, this requires offering a 'premium' for rarity (which could involve accepting that a clear patient benefit is proven in the case of an 'orphan medicine', as happens in other countries).

   However, at present, existing NICE processes offer a significant premium only for treatments for ultra-rare conditions – not for treatments for rare conditions. Treatments for rare conditions are thus caught in the gap between the standard NICE process, which was designed for common conditions, and the HST process, which is only open to a small subset of ultra-rare disease treatments. With the transfer of responsibility for assessing medicines currently considered by NHS

England through its policy development process, this challenge will only grow unless action is taken to enable NICE to employ additional flexibility in these cases.

2. In the event of uncertainty, the presumption should be in favour of access

   At present, except in certain cases (and particularly in respect of cancer treatments), when NICE faces uncertainty in whether a treatment will be effective, it errs towards denying access to patients. This discriminates against treatments for rare conditions in particular, where information about their effectiveness tends to be less comprehensive than for treatments for common conditions simply because fewer patients can be treated with them. It is also the opposite approach to that recently introduced in Scotland where treatments are made 'conditionally' available whilst further information about their effectiveness is gathered. NICE should therefore be able to adopt a 'conditional' approach when assessing treatments for all rare conditions where there is significant uncertainty. As part of this, NICE should review the cost-effectiveness threshold for the HST programme, as such thresholds can create rather than solve challenges when uncertainty is very high, as is the case for almost every treatment that qualifies for HST.

3. The patient voice must be heard

   Although NICE's processes offer clear points when patients can contribute to decisions about the treatments that should be made available on the NHS, there is scope for NICE to offer better support to those charities who rarely engage with their processes and ensure that their views are formally taken into account in decision-taking. The PACE system in Scotland and CAPIG system in Wales provide practical examples of steps towards more patient involvement, though these are not the only models and we believe NICE should explore how it could facilitate even greater levels of involvement in decision-taking.

4. The NHS must work with pharmaceutical companies to ensure sustainable funding is in place for rare disease medicines

   Pharmaceutical companies and the NHS must work together to address the funding challenges created by the increased number of rare disease treatments. This should include identifying how commercial flexibility can be offered by both sides and exploring the opportunities to utilise the rebate payments paid by the pharmaceutical industry under national pricing agreements. This could involve identifying a separate funding stream for rare disease medicines, and enabling funding allocations to change in line with the demands for it (perhaps using the approach which has been adopted through the New Medicines Fund in Scotland).

**Conclusion**

In the years since its creation, NICE has evolved in step with changes in the nature of the treatments it has been tasked with assessing. NICE's ability to adapt to scientific progress, while maintaining high standards of transparency and patient engagement, has been a major source of the high regard in which it is held by patient-focused organisations such as the SHCA.

As NICE now takes on additional responsibilities for the assessment of rare disease medicines, there is a need for it to once more adjust its approach to ensure that patients

with rare diseases are treated fairly and equitably. There are several areas in which relatively minor changes, expanding upon NICE's existing approach, could make a significant difference to the lives of those living with rare diseases.

We hope that NICE, NHS England and the Government will consider the issues set out in this paper and the principles that we have proposed to guide reforms. We look forward to continuing to engage with all sides and championing the cause of patients with rare and complex conditions as the review progresses.

**SHCA, September 2019**

**Appendix – about the Specialised healthcare Alliance**

The Specialised Healthcare Alliance is a coalition over 120 patient-related groups and 8 corporate supporters, which campaigns on behalf of people with rare and complex conditions.

The secretariat to the Specialised Healthcare Alliance is currently provided by Incisive Health.

**References**

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