Specialised Healthcare Alliance
FOR EVERYONE WITH RARE AND COMPLEX CONDITIONS

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 medicines, 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.

Guiding principles for NICE methods reform

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. **There must be a 'premium for rarity'**

   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 very 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 that could be taken towards more patient involvement, though these are not the only models and we believe NICE should be bold in exploring 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 opportunities to utilise the rebate payments paid by the 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 demand (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, November 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.