Specialised Healthcare Alliance
FOR EVERYONE WITH RARE AND COMPLEX CONDITIONS

A backwards step?

Assessing the impact of recent reforms to NICE on patients in England

Introduction

The Specialised Healthcare Alliance (SHCA) represents patients with rare and complex conditions. For many of these conditions, there are few, if any, treatment options available. Patients therefore rely on the NHS to make new treatments available to them whenever they can, to give them a chance of a better life.

In recent years, scientific breakthroughs have allowed the development of an increasing number of new treatments for conditions that were previously too difficult to treat. For patients, these new treatments can represent anything from a literal life-saver to the chance to be able to live independently – carrying out the everyday tasks that most people take for granted.

However, for the NHS, new treatments often create additional pressures on its finances. As the number of new treatments has grown, so has the challenge for the NHS in finding the funds to provide a service to patients of the highest quality.

As a result of these pressures, in 2017 the NHS in England took the unprecedented step of introducing controls that gave it the power to request a delay the introduction of new and high-cost treatments, through the creation of a 'budget impact test (BIT)'. It also introduced new controls on the approval of new ultra-rare disease treatments (or 'Highly Specialised Technologies (HST)), by the adoption of a new 'cost effectiveness threshold'.

At the time, the SHCA and charities raised concerns about the potential impact of these changes. We were motivated to do this by our members, who expressed fears that they could lead to a backwards step for people living with rare and complex conditions. In a survey we carried out in April and May this year, for example, 81% of respondents reported that they were either very concerned (47%) or concerned (34%) about the potential impact that the changes would have on patients.[i]

More than a year on from these changes, we sought to assess the extent to which these concerns had been realised.

Frustratingly, we encountered significant obstacles that restrict the ability of those outside of the NHS to make a comprehensive assessment of the impact of the changes. However, it is clear from our findings that changes are required to current practice, at the very least to deliver increased transparency and accountability in decision making.

The SHCA is therefore calling for the Government and NHS to bring forward scheduled reviews of the changes to the end of this year, to coincide with wider discussions about the future of the system of deciding how new treatments should be made available on the NHS.

This paper sets out:

- Our approach to assessing the impact of the changes

- Our assessment of the impact of the BIT
- Our assessment of the impact of the new HST thresholds
- A summary of our conclusions and recommendations

Our approach to this work

- To carry out this work, we reviewed all Single Technology Appraisal (STA), Multiple Technology Appraisal (MTA) and HST evaluations carried out by the NICE between April 2017 and September 2018

- These are the different processes through which NICE makes recommendations on which new treatments should be made available to NHS patients, with STAs and MTAs used for the majority of treatments and HSTs used for treatments for very rare diseases

- For STAs and MTAs, Final Appraisal Determination (FAD) documents, setting out the detailed reasoning behind NICE's recommendations for each treatment that it has assessed, was reviewed to seek to identify references to the BIT

- For HSTs, Final Evaluation Determination (FED) documents (similar to FAD documents) were reviewed to identify whether the cost-effectiveness threshold was used in decision-taking. Due to the much smaller number of FEDs published since the changes were made, we also looked at NICE's earlier Evaluation Consultation Determination (ECD) documents, which set out draft recommendations for HSTs

Our assessment of the impact of the BIT

About the BIT

The BIT was created in response to concerns within NHS England about the affordability of the 'mandatory funding direction', which protects access for patients to cost-effective medicines by placing a legal requirement on the NHS to fund new treatments recommended by NICE. The mandatory funding direction had been created to safeguard consistent access across England and Wales to new treatments recommended by NICE, thereby avoiding 'post-code lotteries' based on local decision making on access.

By 2017, NHS England had become particularly concerned about the impact of the mandatory funding direction on its medicines budget. This was due to the rising proportion of new treatments that were being developed for rare and complex conditions, which NHS England is directly responsible for funding as part of its specialised commissioning responsibilities.[ii]

How the BIT has worked since being introduced

The BIT is applied to all new treatments assessed through the STA and HST programmes mentioned above. For each treatment considered through these programmes, NICE assesses the expected financial impact of expected use in the NHS. This takes into account the cost of the treatment itself, the costs for hospitals of providing it to patients, and any savings from reduced usage of existing

medicines.[iii] If the NHS expects to spend more than £20 million on the treatment in any of the first three years following its consideration by NICE, the test is deemed to be 'met' and NHS England is allowed to invite the pharmaceutical company concerned to negotiate a lower price.

If NHS England and the company are unable to reach an agreement, NHS England can ask NICE for permission to delay or even pause the use of the treatment in question, weakening patients' rights to treatment.

For NICE to agree to NHS England's request[iv], it must be of the view that:

- the budget impact test has been met, ie the NHS is likely to spend more than £20 million on the treatment in any of the first three years
- NHS England has attempted to reach a deal with the pharmaceutical company but has failed to agree one
- the level of delay (or pause) requested is justified given how much NHS England would otherwise have to spend
- the request takes account of the severity of the condition under consideration
- the decision does not exacerbate inequalities (as defined in law)
- the NHS has clearly set out how it will phase in the introduction of the treatment over the longer-than-usual period

However, although the above list sets out some broad criteria, there is no further guidance available to NICE to help it take these decisions. There are also no set rules on how NICE should report when the test has been engaged.

<u>The impact of the BIT on patients</u>

When the BIT was introduced, NICE estimated that it would be triggered by one in five treatments assessed through its processes.[v] It is therefore surprising that NHS England first announced in June 2018, well over a year after the BIT was introduced, that it had been triggered for the first time.[vi] As part of this announcement, NHS England disclosed that a confidential commercial agreement had been reached with the company for the treatment in question which meant that there would be no delay to its introduction to the NHS.

The absence of any discernible and adverse impact on patients in this case is welcome. However, the announcement did highlight issues with transparency in relation to the implementation of the BIT, insofar as NICE does not routinely report on the BIT as part of the publication of its guidance. Even in the sole case where it has been announced that the BIT has been triggered, this was not reported in the documentation published by NICE.[vii] This presents a major barrier to patients and patient organisations in trying to understand the impact of the BIT.

In papers for its July Board meeting, NICE noted "S*ince implementation, 65 appraisal and HST topics have been assessed for the budget impact test at the company submission stage of the process, and 14 (21%) have been identified as potentially meeting the budget impact test criteria. One of these topics has resulted in a successful commercial arrangement between the company and NHS England, and final NICE guidance has been published. The remaining 13 topics are still going through NICE's processes, awaiting the outcome of value assessment*."

Based on the board paper, NICE's original estimate of one in five treatments triggering the test would seem to be accurate. However, due to the lack of routine reporting, it is very challenging to assess the full impact of the BIT in the 13 topics, or even which topics were affected.

No definition was provided by what was meant by "*potentially*" meeting the test or what process is followed to determine whether the test itself is triggered. It is possible that the process that is followed when a budget impact test is "*potentially*" met, could result in delays to the publication of guidance, even if the test itself is not formally triggered, if there is a need for additional discussions between NICE, NHS England and/or the company involved.

NICE's relative lack of transparency on the BIT is unusual, because NICE is otherwise typically very clear about factors which may have a material impact on its recommendations. Furthermore, it is inconsistent with NICE's approach to transparency on the cost-effectiveness threshold for the HST programme, which was introduced at the same time as the BIT and which is routinely reported on in HST guidance, even in cases where it has not been met.

It is not clear why NICE has taken this very opaque approach on the BIT, although matters of commercial confidentiality may conceivably be a factor. However, in our view it should be quite possible for NICE to include a simple statement in each new piece of guidance regarding the status of the BIT.

There is therefore a clear need for NICE and NHS England to review its approach to transparency on the BIT to help patients better understand how it has been used in practice. A full review of the process is scheduled for 2020 and the SHCA believes that this should be brought forward to provide assurances regarding its impact to date and to clarify future arrangements to deliver accountability.

Our assessment of the impact of the HST cost-effectiveness threshold

About the HST threshold

It had long been recognised by NICE that its traditional method of assessing the 'cost-effectiveness' of a treatment, based on its use of a threshold of cost per 'Quality Adjusted Life Year' (QALY) to assess value for money, was not suitable for the assessment of ultra-rare diseases.

This was based on a consensus that the calculation of cost per QALY estimates is often fraught with uncertainty and never more so than with treatments for ultra-rare conditions where, by definition, the evidence available is extremely uncertain given the very low numbers of patients involved. The QALY measure has also been criticised for failing to reflect the health and quality of life issues that are important to people with very rare diseases and their families. [viii]

The HST programme was therefore set up in 2013 to provide an appropriate method by which NICE could evaluate treatments for ultra-rare conditions, using an approach which was not based on analysis of cost per QALYs.[ix] Decisions were instead taken based on a consideration of other factors, including the nature of the

condition, the impact of the new treatment, the cost to the NHS, value for money and the impact of the treatment on families, carers and society.

Prior to April 2017, the programme had some success in delivering access to treatments for ultra-rare conditions, with six new medicines approved under the system as originally introduced. This represented very welcome progress for patients with these conditions. However, the rate of publication of guidance was very slow, with those six pieces of guidance spread over period of four years following the introduction of the process.

In part, this was due to protracted commercial negotiations between companies and NHS England over the terms on which treatments were to be made available. Unlike in the STA programme, the original HST process did not set a clear timeframe in which decisions should be made: this meant that patients were often caught in the middle of these negotiations, with each new delay a source of frustration and distress.

The introduction of a cost-effectiveness threshold was proposed by NHS England as a solution to this issue, to provide a concrete signal for when a treatment should be funded. On the face of it, introducing a new signal was a logical step. However, charities and patient organisations were highly concerned about both the proposed use of the QALY measure and the level at which the threshold had been set (described below), not least following the publication of a welcome analysis carried out by the Genetic Alliance that suggested that none of the four HST treatments that had been approved by NICE at the point at which the proposals were first made would have been approved had they been assessed under the new system.[vii]

How the threshold works

Under the new HST process, a 'sliding' cost effectiveness threshold was introduced, whereby the more effective a treatment is judged to be, the higher the cost effectiveness threshold at which the NHS is prepared to fund it. If the incremental cost of a treatment is found to be below £100,000 for each additional QALY that it generates, it will receive automatic funding by NHS England, regardless of the total health gain that it delivers. However, if the treatment delivers more than 10 QALYs, additional weighting is applied to this threshold, up to a maximum of £300,000 per QALY for a treatment that delivers 30 QALYs in total. [x]

The impact of the threshold on patients

As highlighted above, these changes raised significant challenges to the approval of new treatments – and led to almost half of our members responding to our members' survey to say that they were not confident in the decisions made through NICE's HST process. The SHCA therefore sought to understand the impact that the changes have had on patients in practice.

Since the changes were introduced, however, just two treatments that have been affected by the reforms have received final guidance. Three other treatments received final guidance during the same period, but their reviews had all been initiated prior to the introduction of the threshold so were appraised under the previous system. In addition, two further treatments have received draft guidance,

while NICE ruled that it did not have sufficient evidence to make a decision on another. The outcomes of these evaluations are set out in table 1 below.

Table 1: NICE HST evaluations which have incorporated the cost-effectiveness threshold[xi]

Treatment	Indication	Date	Weighting applied to the standard £100,000 HST cost per-QALY threshold	Decision
Metreleptin	Lipodystrophy	Jul-17	Not able to quantify	No decision made (draft guidance)
Strimvelis	Adenosine deaminase deficiency–severe combined immunodeficiency	Feb-18	1.4 (setting the threshold at £140,000 per QALY)	Recommended
Cerliponase alfa	Neuronal ceroid lipofuscinosis type 2	Feb-18	Did not qualify for additional weighting	Not recommended (draft guidance)
Afamelanotide	Erythropoietic protoporphyria	May-18	Did not qualify for additional weighting	Not recommended (appealed)
Burosumab	X-linked hypophosphataemia (XLH) with radiographic evidence of bone disease in children aged 1 year and over, and in young people with growing skeletons	Oct-18	1.36 (setting the threshold at £136,000 per QALY)	Recommended (draft guidance)

The table above shows that out of the four treatments to which the threshold has been applied, two have received either draft or final positive guidance, and three have not. This stands in contrast to the HST evaluations which were completed before the reforms came into effect, all of which resulted in positive recommendations. Although the sample size is too small to draw robust conclusions, the emerging evidence indicates that concerns about the impact of the introduction of the threshold may prove well founded.

Notwithstanding the limited sample, it is clear that the threshold has not solved the issues with the pace at which HST guidance is published. This issue does not seem to be a 'teething problem' – well over a year after the introduction of the changes, of the nine HSTs that are currently listed on NICE's website as in development, seven have an expected publication date of 'TBC'.

In light of these challenges, the SHCA believes there is a need for a reconsideration of the HST threshold. The establishment of a new system of conditional access for ultra-rare diseases in Scotland from October 2018 provides an example of how NHS England and NICE could seek to address challenges in the current system.

Summary of our conclusions and recommendations

The full impact of the introduction of changes to NICE's process and methodology in April 2017 is challenging to assess. However, from publicly available evidence it appears that:

- The BIT has not yet resulted in restrictions on patients' access to new treatments, although it could have led to delays in decision-taking – it is difficult to discern the impact of the BIT with certainty on account of the relative lack of transparency

- The new HST thresholds appear to be impeding patients' access to treatments for ultra-rare disease

- The new HST thresholds have not coincided with any improvement in the time it takes for NICE to issue guidance

Our findings with respect to the HST process are highly concerning, particularly given that patients and their families continue to bear the ultimate cost – in terms of anxiety, stress, and in some cases the denial of the opportunity for improvements in health.

We therefore recommend that:

- The process for applying the BIT is reviewed so that patients and their representatives understand better when it is triggered, including by noting this in NICE's guidance

- The operation of both the BIT and the HST thresholds should be reviewed as early as possible – and ideally by the end of the year – with patients and their representatives fully engaged in the process

- The thresholds used in the HST process should be lifted in the meantime to ensure patients' access to new treatments is not impeded

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Appendix – about the Specialised Healthcare Alliance

The Specialised Healthcare Alliance is a coalition over 120 patient-related groups and 10 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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[vi] NHS England, NHS England strikes deal on new NICE recommended lung cancer immunotherapy drug, June 2018
[vii] NICE, Pembrolizumab for untreated PD-L1-positive metastatic non-small-cell lung cancer, July 2018
[viii] Genetic Alliance (2017), *Changes to the HST Programme,* available via: http://www.geneticalliance.org.uk/news-events/news/changes-to-the-hst-programme/
[ix] HM Government, 2012 Health and Social Care Act, 2012
[x] National Institute for Health and Care Excellence (2018), *Rare Disease Day,* available via: https://www.nice.org.uk/news/blog/rare-disease-day
[xi] SHCA analysis of NICE HST evaluations