

How will NICE's budget impact test affect new drug availability?

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NICE's budget impact test – in which new drugs that will have a net impact of £20 million or more in any one of their first three years of use will trigger commercial negotiations between NHS England and the drug company – was introduced in April, amid much controversy. Many argue the threshold is too low and will have an unacceptable impact on the availability of new medicines.

NICE and NHS England have been defending themselves from widespread criticism by healthcare charities, politicians and patients, following their announcement of changes that could lead to delays of up to three years to the introduction of expensive new drugs to the NHS.

NICE and NHS England, facing mounting pressure on NHS finances, announced a 'budget impact test' in March following a three-month joint public consultation,¹ beginning in October 2016. The test, which came into force on 1 April, assesses the potential extra cost of new drugs for the NHS in an attempt to manage their introduction and make sure their funding does not adversely affect other NHS services or the long-term financial stability of the health service. When NICE identifies a drug that is likely to cost £20 million or more in any of its first three years' use, it will trigger commercial negotiations

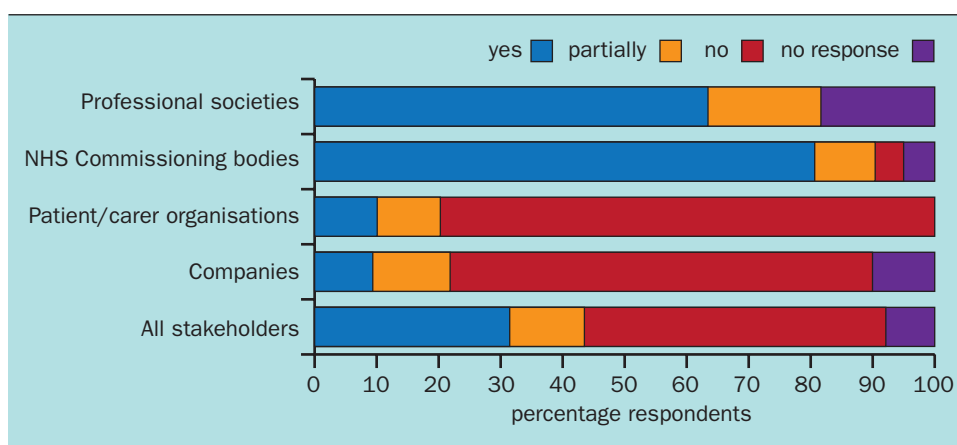


Figure 1. Stakeholders responses to the question "Do you agree that NHS England should set a budget impact threshold to signal the need to develop special arrangements for the sustainable introduction of cost-effective new technologies?" From: NICE and NHS England. Consultation on changes to technology appraisals and highly specialised technologies. Analysis of responses to the consultation, February 2017²

between NHS England and the drug company.

If this does not resolve the situation, NHS England can ask NICE to phase in the new treatment gradually, over a longer period than the statutory 90 days, up to a maximum of three years, to make it more affordable to the NHS and minimise its effect on services. The NHS must spell out the length of the proposed extension and set out its plans to phase in the product, based on clinical advice, up to full implementation by the end of the specified phasing period, says NICE.

NICE, which is keen to stress that £20 million is a threshold not (as portrayed by the media) a 'cap', adds: "There can and will be circumstances where it is appropriate for the NHS to pay more in order to make new treatments available for patients."

Too low a threshold?

Large pharmaceutical companies and industry representative bodies strongly challenged the proposal to introduce a net budget impact threshold during the

consultation. According to the consultation analysis, nearly half of the 151 stakeholder respondents (48%) did not agree with the proposal to set a budget impact threshold (see Figure 1).² They felt questions of affordability were valid but already addressed through the Pharmaceutical Price Regulatory Scheme (PPRS), which reduces the amount the NHS pays for new drugs. And many, including academics and patient groups, questioned why the PPRS had not been referenced.

There was also concern among the stakeholder respondents about the proposed level of the net budget impact's threshold. Only 13% agreed it should be set at £20 million, while 22% said "partially", 53% said "no", and 13% – who did not explicitly state "yes/no/partially" and their response did not appear to answer the question – were recorded as "no response" (see Figure 2). Many stakeholders "felt that the £20 million figure had been arbitrarily selected, with a lack of rationale provided."²

The Association of the British Pharmaceutical Industry (ABPI) was one respondent to the consultation that said the £20 million threshold was too low. ABPI value and access director Dr Paul Catchpole says: "Everyone was taken aback when such a low threshold was set because the consultation was supposed to be aimed at dealing with situations that occur more infrequently. As, for instance, when the transformational

direct-acting antivirals for hepatitis C came along a few years ago, which are curative within a matter of months and where the budget impact was between £100 and £200 million.

"Our position is very clear. NHS England and NICE need to get better at managing and planning – three to four years in advance – for the introduction of new technologies and new medicines. They should be looking for those treatments that are going to be considered very transformational and very important, where there could be a high budget impact, then work should start much earlier on planning for the introduction of those medicines. In that way, you would avoid the kind of shock to the system that occurred when the hepatitis C medicines were launched and everybody realised there was going to be a huge budget impact but it was too late.

"The process of understanding what medicines are coming down the line is called 'horizon scanning'. New medicines are in the research and development phase for maybe 10 years, and in the two or three years before they are to be launched, there is a lot of information available to support planning for their introduction by the NHS."

Dr Catchpole adds: "Industry recognises the very significant challenges the NHS has, so if there's another transformational medicine that will have a huge budget impact then the company and the NHS should absolutely be talking

together about how best to introduce that medicine. It might well mean some kind of innovative arrangement is needed that would allow patients to benefit from new medicines under a different payment profile.

"But that's a very different sort of discussion than about asking for prices to be further reduced below the level at which – following stringent evaluation – they have already been established as cost-effective and value for money by NICE."

Assessing the impact on new treatments

The NHS is legally bound, under the funding directive, to make drugs and other treatments available when they are recommended by NICE technology appraisal programmes. This has normally meant that the NHS has 90 days to make the treatment available, regardless of all other commitments.

The current system, however, appraises the most clinically- and cost-effective treatments on the basis of their impact on an individual – via Quality-Adjusted Life Years (QALYs) gained³ – without taking into account the numbers who might use the treatments, and therefore the overall cost of their provision.

This means that even relatively inexpensive new drugs can dent the NHS budget when there are hundreds of thousands of people with commonly occurring illnesses who will benefit from their use. Meanwhile, some very expensive new treatments, no less necessary but of benefit to fewer people with rarer conditions, add more to the bill and the total cost of NICE recommendations can run into hundreds of millions of pounds a year. This, of course, leaves less to spend on other equally vital NHS services, such as general practice, elective surgery and routine health checks.

Some charities are unconvinced by reassurances from NICE about the impact of the £20 million threshold and say that the phased introduction will limit availability of treatments for those who are eligible, with possibly catastrophic results for them.

The Alzheimer's Society says that with several promising dementia treatments in the pipeline, and 676,000 people with

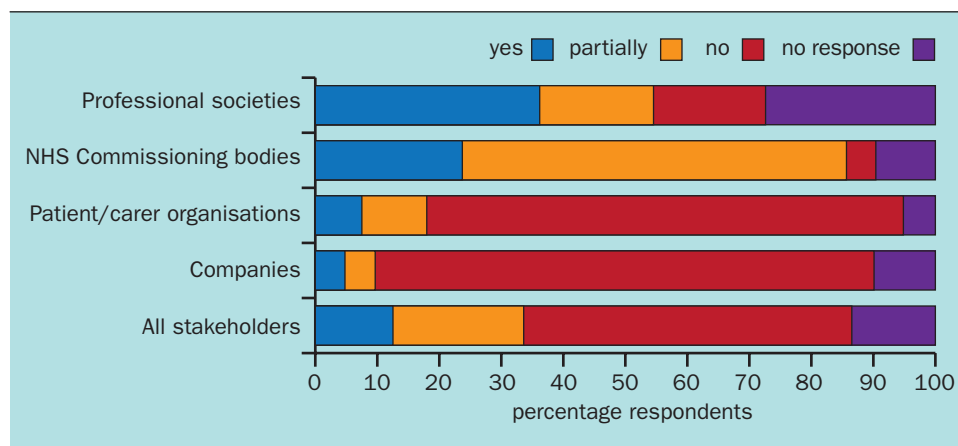


Figure 2. Stakeholders responses to the question "Do you agree that £20 million is an appropriate level?" From: NICE and NHS England. Consultation on changes to technology appraisals and highly specialised technologies. Analysis of responses to the consultation, February 2017²

dementia in England, there are concerns over the potentially damaging impact the added delay could have on the lives of people with the condition. The charity believes that for people with progressive long-term conditions, such as dementia, any additional delays to accessing treatments could cause their health to continue to deteriorate irreversibly.

Jeremy Hughes, chief executive at Alzheimer's Society, says: "Drug companies and the NHS must work together to ensure this new test would not delay long-awaited new drugs for dementia, England's biggest killer. It must not stop relatively inexpensive treatments being made available to patients: a treatment that could help every person with dementia in England would have to cost less than £29.60 per year to avoid facing this new test. The commitments in the NHS Constitution must be upheld."

Diabetes UK policy manager Nikki Joule comments: "We're extremely disappointed these changes have been introduced as it is likely to have an enormous impact on people who have conditions such as diabetes. Given the millions of people with diabetes who could benefit from a treatment, the easier it will be to breach the £20 million threshold.

"If Lucentis [ranibizumab] – the treatment which helps prevent blindness for people with diabetic retinopathy – was introduced today under the new threshold, it would be put on hold. NHS England and NICE must reconsider these changes and work with patients and others to agree an alternative that puts people living with conditions like diabetes first."

Emlyn Samuel, Cancer Research UK's senior policy manager, remarks: "We've made our concerns clear that the introduction of this budget impact test could have a significant effect on cancer patients. Any system that potentially delays access to new treatments, once they have been approved by NICE, is unacceptable. It's important that manufacturers price drugs responsibly, but we don't think this test is necessary. There are other options available to the government, which we strongly encourage them to explore."

Labour's Shadow Health Secretary, Jonathan Ashworth MP commenting on the NICE Board decision, argues: "It's

Treatment	Condition	Patient population	Annual cost by 2020/21	Source
Sacubitril valsartan (Entresto)	Chronic heart failure	64,500	£69,000,000	http://bit.ly/2n2SAQH
TNF alpha inhibitors	Rheumatic diseases	20,200	£59,185,000	http://bit.ly/1Q6p5jA
Aflibercept (Eylea)	Diabetic eye disease	6205	£26,000,000	http://bit.ly/2niadla
Abiraterone (Zytiga)	Prostate cancer	2000	£43,000,000	http://bit.ly/2mmMBTH
Ezetimibe (Ezetrol)	Heart disease	159,000	£52,000,000	http://bit.ly/2mmMBTH
Ranibizumab (Lucentis)	Diabetic eye disease	1200	£22,000,000	http://bit.ly/2mmMBTH
Pembrolizumab (Keytruda)	Skin cancer	1000	£48,000,000	http://bit.ly/2mmMBTH

Table 1. Seven drugs that would have breached the £20 million budget impact threshold, according to an analysis by the Labour Party

simply not acceptable that hundreds of thousands of patients could be left without the essential care and treatment they need. These plans are a clear breach of the NHS Constitution, which promises to introduce any treatment deemed cost effective by NICE within 90 days of approval. Theresa May's decision to squeeze health funding for so long is now restricting access to new and innovative drugs and the prime minister needs to explain what action she is going to take to make sure patients can access the drugs and medicines they need."

Mr Ashworth notes that an analysis by the Labour Party has shown that in 2015–16, seven drugs would have been blocked, affecting at least 254,105 patients, as shown in Table 1.

A NICE spokesperson responds: "The numbers quoted do not reflect NICE's assessment of uptake of the drugs – for some, the number quoted relates to the entire eligible population, and for others it refers to uptake after five or even seven years."

NICE also disputes the Labour Party's claims that the plans breach the NHS Constitution on patients' rights to access NICE-approved treatments, saying that

patients will still gain access to drugs, although where the net budget impact is high they might have to be phased in over a longer period. NICE adds that it "has always been able to consider requests from the NHS to extend the funding requirement; for example, where the necessary facilities or staff are not immediately available."

NICE's viewpoint

NICE chief executive Sir Andrew Dillon says: "Our analysis suggests that, based on the treatments we have approved, only one in five will trigger the budget impact test. The test is simply the start of a process for managing the introduction of new treatments where there will be a significant impact on the NHS budgets.

"We hope, and we think it is perfectly possible, that for some treatments that exceed the £20 million budget impact in their first three years, there will be commercial agreements between companies and NHS England that will at least minimise and in some cases avoid completely the need for any delay to access for patients.

"Even where there is a delay beyond the standard 90 days, NHS England

has committed to ensuring that there is some funding available to provide access throughout the phased implementation period.” He adds that NICE will review the changes in three years to assess their impact on allowing access to new drugs.

Other changes to the NICE evaluation process include the introduction of a new fast-track option for appraising treatments that offer exceptional value for money. These are ones with a likely cost of less than £10,000 per quality-adjusted life year (QALY) gained, where the upper end of NICE’s standard threshold range is £20,000–£30,000 per QALY gained.

The aim is to make these drugs available to patients within a month of publication of the NICE guidance, thus giving patients access to the most cost-effective treatments up to five months’ faster than before the approval of the changes, says NICE. It has also signalled that it intends to consider broadening the fast-track approach to a wider group of treatments, including medical devices and diagnostics, over the next two years.

Under its highly specialised technologies programme, treatments for very rare conditions deemed to provide significant QALY benefits could benefit from being assessed against a maximum threshold of £300,000 per QALY gained, rather than the £100,000 per QALY limit in the original proposal. This upper limit is 10

times higher than the standard NICE upper threshold and is being considered in order to reflect the transformational health benefits they can offer to patients, explains NICE. Drugs for very rare conditions will be evaluated against a sliding scale, so the more the medicine costs, the greater the health benefit it has to provide to be approved by NICE for routine NHS use.

NHS England’s acting director for specialised commissioning, John Stewart, says: “As well as significantly speeding up access for patients to the most cost-effective new technologies, NICE’s new approach also shows that the NHS is prepared to pay far more for highly specialised treatments that can transform patients’ quality of life.

“We listened carefully to the feedback received during the consultation and have adjusted our proposals by trebling, for the most transformative treatments, the threshold for determining routine funding – raising the upper limit to 10 times that used for standard treatments.

“These are not easy decisions, but we are committed to working closely with companies that are willing to price their products responsibly and this new flexibility will help us develop innovative win-win-win agreements – good for patients, good for taxpayers and good for those companies that are willing to price responsibly.”

References

1. NICE and NHS England. Proposals for changes to the arrangements for evaluating and funding drugs and other technologies appraised through NICE’s technology appraisal and highly specialised technologies programmes. October 2016. Available from: <https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/consultation-on-changes-to-technology-appraisals-and-highly-specialised-technologies>
2. NICE and NHS England. Consultation on changes to technology appraisals and highly specialised technologies. Analysis of responses to the consultation. February 2017. Available from: <https://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-technology-appraisals/TA-HST-consultation-report.pdf>
3. Quality-adjusted life year (QALYs). A measure of the state of health of a person or group in which the benefits, in terms of length of life, are adjusted to reflect the quality of life. One QALY is equal to 1 year of life in perfect health. QALYs are calculated by estimating the years of life remaining for a patient following a particular treatment or intervention and weighting each year with a quality-of-life score (on a 0 to 1 scale). It is often measured in terms of the person’s ability to carry out the activities of daily life, and freedom from pain and mental disturbance. (From: NICE Glossary, <https://www.nice.org.uk/Glossary>)

Declaration of interests

None to declare.

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