

Proposals for changes to the arrangements for evaluating and funding drugs and other health technologies appraised through NICE's Technology Appraisal and Highly Specialised Technologies programmes.

Summary of points:

- It is vital that patients get access to new medicines, particularly when they have been involved in their development, either by funding research through donations or taxation, or have participated in the clinical trials. **The introduction of a budget impact threshold of £20M per annum in any of the first 3 years is rationing by delay.**
- We have real concern that these proposals will act as a deterrent to industries and other sectors including charities, that wish to carry out research in the UK, and thus affect the nascent industrial strategy where life sciences are such an important part (as described in the accelerated access review). The proposal is a disincentive for industry and others to invest in the NHS. It sends a signal that the NHS is **not open** to innovation, due to the strict and arbitrary parameters of the proposal. **Disincentivising investment in the NHS is to the detriment of patient outcomes and care. The lack of information about how drugs that pass the budget impact threshold will be paid for by NHS England and adopted across the NHS creates another barrier to allowing patients to access new innovations.**
- The budget impact threshold of **£20M per annum in any of the first 3 years does not take into account interventions that might affect large patient populations, creating a disincentive for researchers to bring new treatments to people affected by conditions such as hypertension (8 million), asthma (3 million), diabetes (3 million), chronic kidney disease (2 million) or COPD (1 million)¹.**
- For therapies targeting rare conditions, the £100,000 QALY threshold and £20M budget impact threshold create a complex scheme, where in practice, most new treatments will pass directly into the annual specialised commissioning prioritization process. AMRC is very concerned that the NHS England prioritization process is an arbitrary and inequitable decision-making process that pits **groups of patients against each other to access treatments.**
- **The proposed new arrangements make no mention of WHEN treatments that are above the budget threshold, might be made available.** We understand informally, that this could be within 'short' time scales and 'possibility less than 18 months', but this has not been clearly outlined. There is also no information about how patients and their advocates might be involved in these discussion, or how their views would be tensioned against the desire of NHS England to curtail costs. **This obfuscation runs counter to NICE and NHS England stated desire to develop a transparent system.**

¹ Prevalence in England in 2015 from QOF data

https://data.gov.uk/dataset/quality_and_outcomes_framework_achievement_prevalence_and_exceptions_data

- We are concerned that the introduction of thresholds, emphasis on QALYs, and rerouting of ultimate decision making into NHSE will have negative impact **on patients with common and rare diseases**, and will **significantly undermine the role and value of NICE**.

About AMRC

AMRC represents 138 of the leading medical research charities funding research in the UK². We are responding to this consultation on behalf of our members, some of whom will also made their own submission.

Our members fund research focussed on the needs of patients for better treatments, therapies and interventions designed to improve the quality of life and ultimately prevent or cure their condition. As such, a focus on the patient perspective and patient voice is central to all of our work.

Medical research charities are a vital part of the mix of partners that work together to create the UK's vibrant life sciences sector. Funding from different actors in the ecosystem – including public and private sources - is interdependent and additive.

Every £1 of UK public funding for research generates between £1.13 and £1.60 of private investment³. The funding our member charities contribute to medical research is vital.

In 2015, AMRC member charities⁴:

- invested over £1.4 billion of research funding in the UK - more than either the Medical Research Council or National Institute for Health Research;
- made capital investments of £129 million in the UK;
- contributed to the knowledge economy by funding the salaries of over 15,000 researchers in the UK; and
- enabled 190,000 people to be recruited into charity-funded trials

Consultation questions

NHS England budget impact threshold

1 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?

No. Rather than creating another barrier, NHS England, NICE, companies and patient groups should be looking at ways to ensure the sustainable introduction of cost effective new technologies, so it becomes an integrated component of medicines evaluation across the board. Methods such as horizon scanning, understanding value and unmet need and innovative funding models are more appropriate for a health system that seeks to innovate.

² For a list of our members see <http://www.amrc.org.uk/our-members/member-directory>

³ Economic Insight (2015) What is the relationship between public and private investment in science, research and innovation? Commissioned by: BIS

https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/438763/bis-15-340-relationship-between-public-and-private-investment-in-R-D.pdf

⁴ <http://www.amrc.org.uk/publications/2015infographic>

2 Do you agree that £20 million is an appropriate level? If not, what level do you think the threshold should be set at and why?

No. The £20M level appears to be an arbitrary number. There is no evidence produced within the consultation that explains why this should be the cut off. For diseases with large population - (hypertension (8 million), asthma (3 million), diabetes (3 million), chronic kidney disease (2 million) or COPD (1 million)⁵, it is hard to see how any intervention can be developed that would be below the £20M threshold.

For example, the annual drugs bill for many diabetes treatments already exceed the £20M per annum threshold (Exenatide £21m, Liraglutide £41m, Sitagliptin £77m and Human analogue insulins £70m). If any of these medicines had been brought through under the new system, they would have been held up by this inappropriate barrier, preventing patients from getting appropriate treatments.

In effect, the threshold becomes an unexplained barrier to innovation and improved patient outcomes.

3 Do you agree that NHS England should enter into a dialogue with companies to develop commercial agreements to help manage the budget impact of new technologies recommended by NICE?

If the proposal went ahead, we would expect to see clear transparency around decision-making – particularly where the company makes other concessions. We would also expect that NHS England engaged with a wide group of other stakeholders including patients and their advocates, to ensure that a full understanding of the value and patient benefit of the treatment is part of the decision making.

Varying the timescale for the funding requirement

4 Do you agree that NICE should consider varying the funding requirement for technologies it recommends, for a defined period, in circumstances where NHS England makes a case for doing so, on the grounds that the budget impact of the adoption of a new technology would compromise the allocation of funds across its other statutory responsibilities?

No. The processes as described in this consultation make it very unclear what leeway NHS England will have in deciding how long there would like to 'vary' the period. In conversations with representatives of NHS England and NICE, a period of "months, definitely not 2 years" was mentioned. However, we are unconvinced that any 'real' timelimits will be set, and fear that NHS England will use this 'variation of the timescale for funding' to prevent patients accessing cost-effective treatments. At its most extreme, it would seem to be acceptable in the outlined process for NHS England to request a variation of 10 years, meaning that the intervention would be out of patent, and the cost drastically reduced. This rationing by delay is not acceptable.

Linking NICE and NHS England processes for evaluating highly specialised technologies

9 Do you agree that NICE and NHS England should use a cost per QALY below which the funding requirement is applied for highly specialised technologies?

No. NICE has previously acknowledged that the cost per QALY methodology is inappropriate for highly specialised technologies (HST) and to date has therefore conducted an evaluation of "value for money" (based on incremental benefit of the HST, requirement for other resources and budget impact). There are significant problems in relation to QALYs and rare disease medicines. The evidence base is limited due to disease epidemiology and very small patient numbers, and thus the resultant estimation of cost-effectiveness is subject to great variability. This means that both clinical and cost assumptions simply cannot be relied upon to support decision making.

⁵ Prevalence in England in 2015 from QOF data

https://data.gov.uk/dataset/quality_and_outcomes_framework_achievement_prevalence_and_exceptions_data

NICE/NHSE consultation

The implication of this proposal is access to rare disease medicines above a threshold cost per QALY would be blocked, because there would be no other route for funding.

10 Do you agree that £100,000 per QALY is the right maximum up to which the funding requirement would be applied? If not, what cost per QALY do you suggest, and why?

No. The basis on which this maximum cost per QALY has been calculated is unclear and therefore the figure appears somewhat arbitrary. Tools used to calculate QALYs are poor in terms of capturing patient experience for those with rare diseases.

11 Do you agree that if the cost per QALY level is exceeded, the technology should be considered through NHS England's specialised commissioning prioritisation process?

No. In practice, most new treatments will pass directly into the annual specialised commissioning prioritisation process.. We are concerned that whilst the NHS England specialised commissioning prioritisation process has been in existence for 2 years, funding of treatments have passed through this route very slowly and with little transparency. The prioritization process has been no quicker than for the previous process. **In addition, AMRC is concerned that NHS England appears to have taken a strategic decision to pit patients against each other, by discussing the relative merits of patient treatment and appearing to ask the public to choose which patient should receive help.** This disadvantages smaller patient populations.

12 Do you agree the proposed new arrangements mean that NICE would not need to take budget impact into account in its highly specialised technologies evaluations?

Other

13 Do you consider that any proposals in this consultation would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation?

Yes.

The NHS constitution sets out the right of patients to access drugs and treatments no longer than 3 months after NICE recommendation for routine use, except for 'limited circumstances'. These proposals will lead this pledge to be broken.

The proposals fail to address the issue of fairness and equity in access to medicines. NHSE has a history of poor performance in this area, and there are significant differences between the evaluation mechanisms of processes of the two organisations.

We are concerned that the introduction of thresholds, emphasis on QALYs, and rerouting of ultimate decision making into NHSE will have negative impact **on patients with common and rare diseases**, and will **significantly undermine the role and value of NICE**.

The NICE's charter states that NICE makes recommendations about "the use of new medicines, medical technologies and diagnostics identify the most clinically- and cost-effective treatments available." The charter does not set out a role for NICE to collaborate with NHS England to set budget thresholds for medicines and technologies. Therefore, this proposal appears to extend NICE's role beyond its current charter.