

# Consultation on changes to technology appraisals and highly specialised technologies

## Analysis of responses to the consultation

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## 1. Introduction

This report covers the responses received to the consultation on changes to technology appraisals and highly specialised technologies which ran from 13 October 2016 to 13 January 2017.

The use of quotes throughout the document is to illustrate some of the main issues raised. They do not necessarily reflect a balance of opinions.

## 2. The consultation in numbers

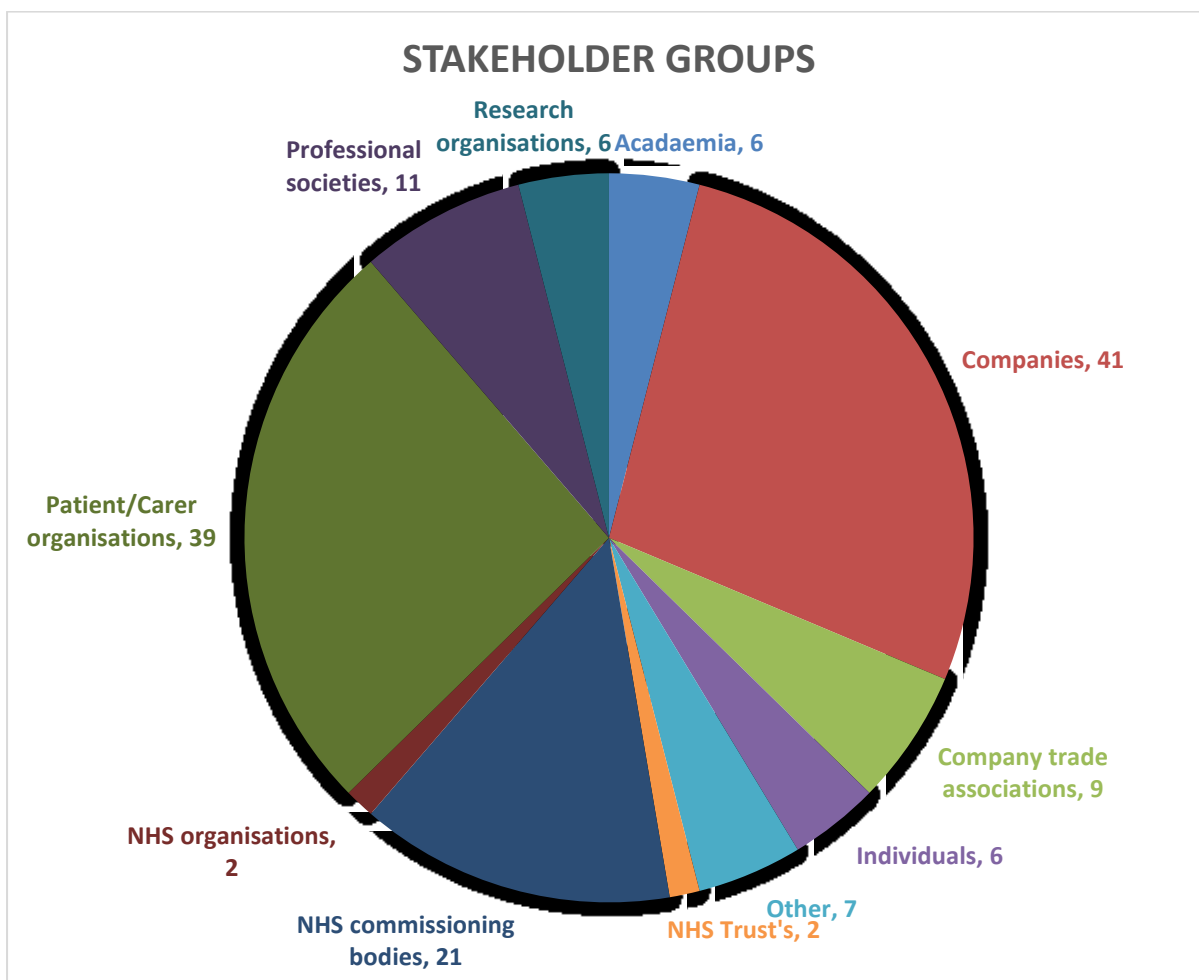
The consultation received responses from 151 stakeholders. We are aware that some organisations have collaborated in developing responses which have been submitted individually, therefore there is some duplication within the responses.

Summary of responses by question:

		Yes	Maybe	No	No response
<b>1</b>	Should there be a budget impact threshold (BIA)?	32%	12%	48%	8%
<b>2</b>	Should the BIA be set at £20m?	13%	22%	53%	13%
<b>3</b>	Should NHSE and companies negotiate where the BIA is exceeded?	47%	29%	17%	7%
<b>4</b>	Should NICE vary the Funding Directive where the BI threshold is exceeded?	23%	16%	50%	11%
<b>5</b>	Do you agree with the criteria for the NICE fast track process?	24%	36%	27%	13%
<b>6</b>	Should NICE fast track technologies anticipated to be less than £10,000 per QALY?	31%	28%	28%	13%
<b>7</b>	Should NHSE fund recommended fast track technologies within 30 days?	49%	13%	23%	15%
<b>8</b>	Should NICE merge its 'abbreviated' process into the fast track process?	40%	13%	22%	25%
<b>9</b>	Should there be an ICER cut off for automatic funding in the NICE HST programme?	23%	9%	48%	21%
<b>10</b>	Should the ICER cut off for HSTs be set at £100,000 per QALY?	11%	11%	53%	25%

11	Should HST topics above £100,000 per QALY go into the NHSE CPAG process?	18%	8%	50%	24%
12	Do you agree that the proposals for the HST programme mean that NICE would not need to take budget impact into account?	11%	10%	50%	30%
13	Do you think any of the proposals put NICE or NHSE at risk of failing to meet their statutory obligations under equalities legislation?	45%	7%	23%	25%

### 3. Who responded to the consultation?



Responses were received from:

- 41 companies
- 39 patient carer organisations
- 21 NHS commissioning organisations
- 11 professional societies
- 10 Research organisations
- 9 Trade associations
- 24 other organisations and individuals

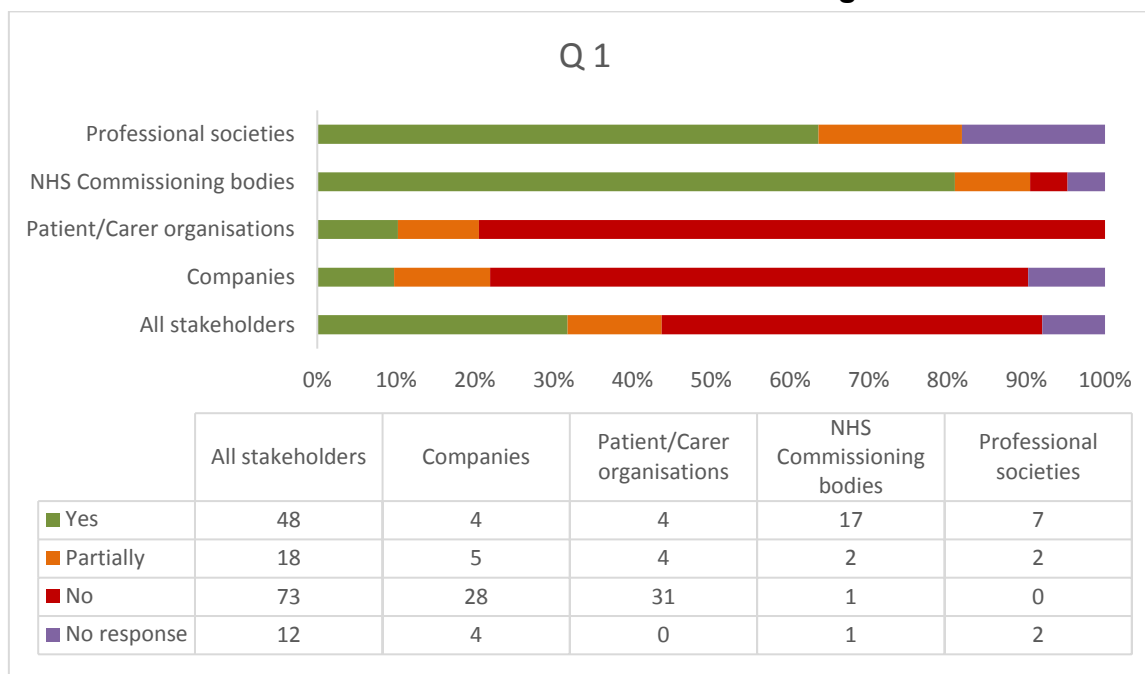
#### **4. Analysis of responses to the questionnaire by question**

Stakeholders were asked whether they agreed, disagreed or partially agreed with 13 questions based on key areas of the consultation document. Where stakeholders did not explicitly state 'Yes/No/Partially' in their response NICE staff selected the most relevant option based on their response. Because of time constraints, NICE staff did not follow up with these respondents to confirm their interpretations were correct. Where a stakeholder did not state 'Yes/No/Partially' and their response does not appear to answer the question, a 'no-response' has been allocated.

The following section shows the breakdown of stakeholder responses by question. The responses of the larger stakeholders groups (Companies, Patient/Carer organisations, NHS Commissioning organisations and Professional societies) have also been shown separately. Highlighted comments have been presented to give a general overview of the comments received for each question.

## Section1 – Budget impact

**Question 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?**



32% (48) of respondents agreed with the proposal, 12% (18) partially agreed and 48% (73) did not agree.

A general theme amongst responses was a recognition of the financial pressures that the NHS is currently facing.

### Company responses

A clear majority of companies (68%) did not agree with the proposal. Less than a quarter (23%) agreed or partially agreed with the proposal.

These are some examples of the recurrent themes in responses that did not agree with the proposal:

- 'PPRS is the primary mechanism for managing affordability in the NHS'  
[Shire Pharmaceuticals]

- ‘NHS England, NICE and the pharmaceutical industry must work constructively to ensure patient access to innovative medicines is not held up due to non-clinical considerations’ [Bayer]
- ‘Any threshold would hinder 1<sup>st</sup> to market products’ [Amicus Therapeutics Ltd]

Companies that agreed with the proposals highlighted the following:

- ‘it seems sensible to identify those technologies which will have the greatest impact on the health care budget’ [Cook Medical Ltd]
- ‘Recognising the economic challenges facing the NHS, Amgen agrees (in exceptional circumstances), with the principle of NHS England discussing with companies how best to manage the introduction of medicines that have an exceptionally high budget impact. However, the proposed threshold of £20m is inappropriate and we believe that this threshold should be £100m.’ [Amgen Ltd]

### **Patient/Carer Organisation responses**

Again, a clear majority (79%) of patient/carers organisations did not agree with the proposals.

Themes in the responses that did not agree included:

- ‘The introduction of a budget impact threshold could disadvantage innovative therapies especially where no other existing treatment exists.’ [PHN Support]
- ‘the solution to the affordable and sustainable introduction of new technologies should lie in better long-term planning and horizon scanning, as proposed in the Accelerated Access Review (AAR)’ [Kidney Research UK]
- ‘The addition of a budget impact threshold would add another layer of assessment and slow down the uptake of innovative medicines by NHS England’ [Kidney Cancer Support Network]

Comments in agreement with the proposal included:

- ‘we are all aware of the budgetary constraints and some mechanism is necessary at least to alert decision takers to the potential for new treatments to create budgetary dilemmas’ [Leber’s Hereditary Optic Neuropathy Society]

### **NHS commissioning bodies’ responses**

The vast majority (81%) of NHS commissioning bodies agreed with the proposal. Whilst supporting the proposals they also wanted clarity that the budget impact threshold would also apply to CCG’s and Local authorities.

An example of a supportive comment from a commissioning body was:

- ‘A budget impact based approach is also much more closely aligned to how the NHS operates in terms of financial planning.’ [South East London Area Prescribing Committee]

### Other responses

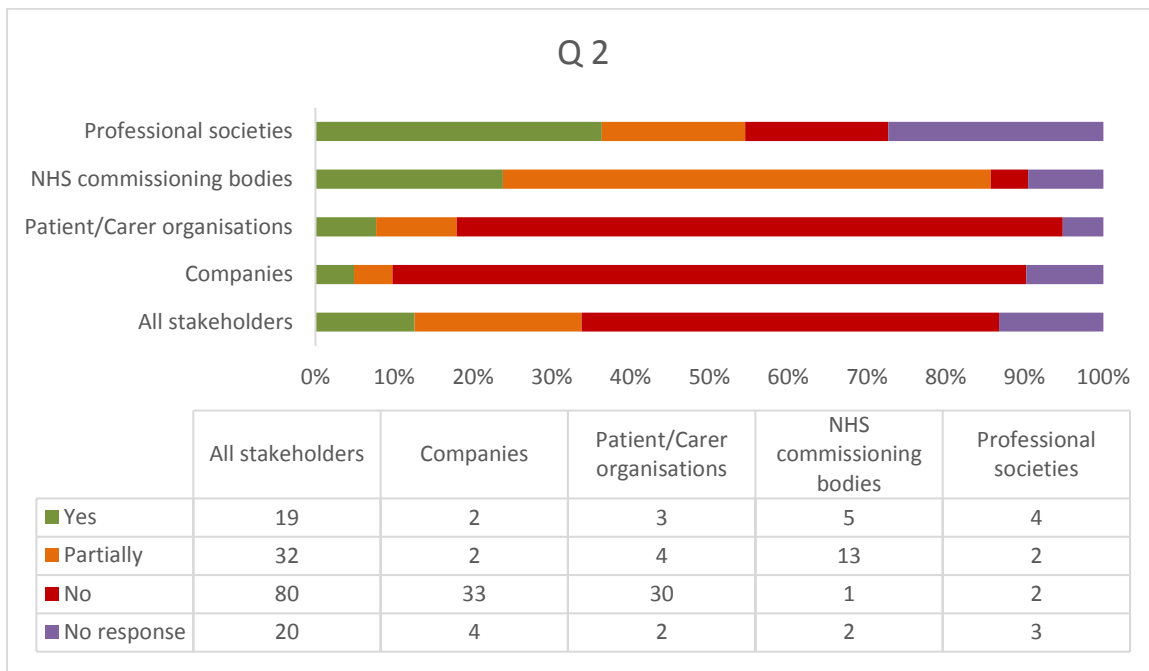
Comments that agreed with the proposal:

- ‘In the context of an increasingly financially constrained health budget, this proposal provides a clear framework for industry, healthcare purchasers, clinicians and patients’ [The Royal College of Ophthalmologists]

Comments that did not agree with the proposal:

- ‘The introduction of the budget impact (BI) threshold would likely lead to unjust inequalities arising between and within patient groups’ [King’s College London]
- ‘this should be dealt with by adjusting the cost-effectiveness threshold conditional on budgetary impact’ [University of Sheffield]

### Question 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?



13% (19) of respondents agreed with the proposal, 22% (33) partially agreed and 53% (79) did not agree. Stakeholders felt that the £20 million figure had been arbitrarily selected, with a lack of rationale provided.

### **Company responses**

80% of companies did not agree with this proposal whilst only 10% agreed or partially agreed. Comments suggested that if a threshold must be set it should be significantly higher, £100 million was frequently suggested.

Highlighted comments:

#### **Disagreed**

- 'It is concerning that 1 in 5 of NICE's technology appraisals over the last 12 months would have been affected, causing delayed or blocked access for large numbers of patients . If a threshold is to be set, this should be indicative and focused on exceptionally costly technologies, while taking account of other potential benefits arising (e.g. in moving care from hospitals to people's homes). A threshold which captures 1 in 5 recent technologies does not meet this description' [Shire Pharmaceuticals]
- 'The threshold proposed in this consultation has been put forward without substantive rationale, methodological detail, or consideration for impact on patient outcomes' [MSD UK Ltd]

#### **Agreed**

- 'This level appears appropriate in balancing the introduction of new innovation into the NHS with the ability of the NHS to afford these new innovations without compromising the availability of other treatments for patients' [Boston Scientific]

### **Patient/Carer Organisation responses**

77% of patient/carers organisations did not agree with the proposals.

Highlighted comments:

#### **Disagreed**

'Any threshold is arbitrary and unhelpful because it will add a meaningless criterion to confuse more robust criteria. The introduction of a specific threshold would be completely arbitrary and furthermore it would undermine the right of patients (as set out the NHS Constitution) to access NICE-approved technologies.' [Tuberous Sclerosis Association]



- ‘the proposal appears confused, conflicting with other proposed policy, coming as the Government introduces legislation to cap the overall cost of medicines, through the Health Service Medical Supplies (Costs) Bill. If the overall cost is capped to ensure spending is kept within defined budgets, why have an affordability test as well?’ [Alzheimer’s Society]

### **NHS commissioning body’s responses**

86% of NHS commissioning agreed or partially agreed with this proposal.

Highlighted comments:

#### **Agreed**

- ‘Yes, although there is a slight concern companies may try and fast-track everything.’ [Leeds North CCG, Leeds South & East CCG and Leeds West CCG, Leeds Teaching Hospital NHST, Leeds and York partnership FT]

#### **Disagreed**

- ‘We feel this should be set lower as all parts of the system are struggling and any additional expenditure would need to find finance’ [York and Scarborough medicines Commissioning Committee]

#### **Other responses**

Comments that agreed with the proposal:

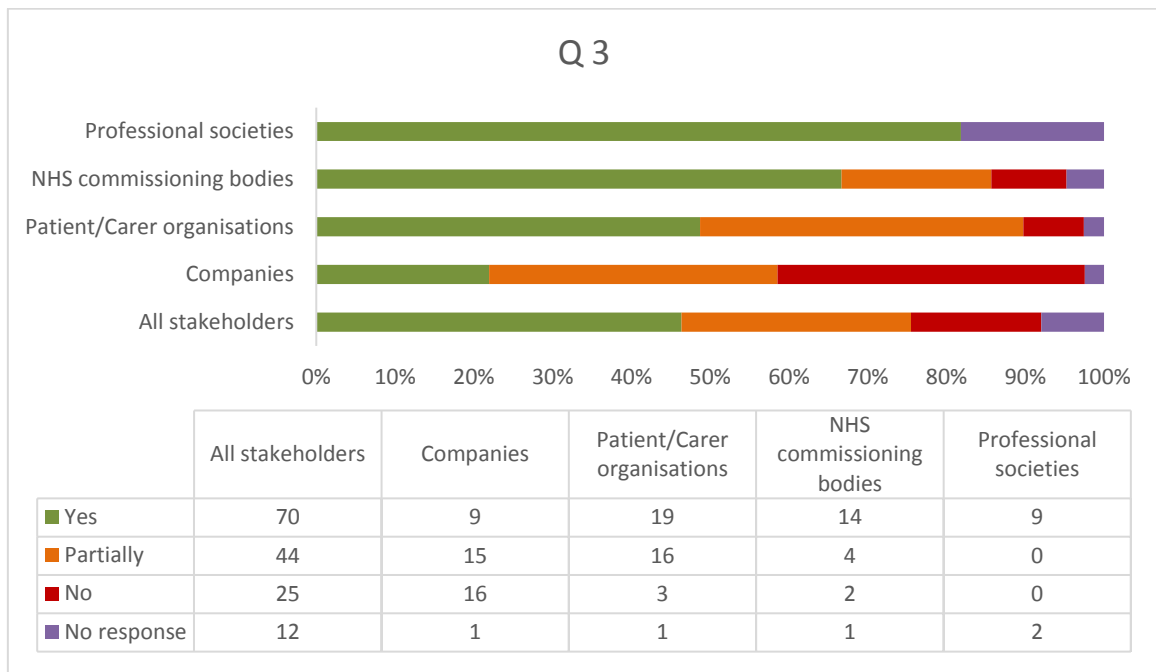
- ‘Based on current technologies undergoing HTA, a budget impact of £20 million should capture most technologies providing an important innovative advance in health care but which would also have a significant impact on financial planning’ [All Wales Medicines Strategy Group]
- ‘We are of the opinion that the value of the budgetary limit should be related to the proportional GDP spend on health rather than being set at a fixed value. This will maintain the relative priority attributed to new, innovative treatments.’ [Faculty of Public Health]

Comments that did not agree with the proposal:

- ‘If a new drug or intervention has a QALY of <£10,000 but affects commoner conditions such as heart attack, stroke, breast cancer, diabetes then clearly £20 million for the >55 million English population seems just too low to be workable. A link to patient volume seems a sensible approach’ [UK Neurointerventional Group]

- ‘a threshold of £20m is inappropriate and that a higher threshold of £100m in any of the first two years post-launch should be set as the trigger for dialogue. We propose that those medicines with a high net budget impact above £100m should be identified at 2-3 years prior to launch. Estimates would be based on best planning assumptions available at the time, and would be a “trigger” to signal formal dialogue to support the sustainable introduction of these medicines into the NHS.’ [ABPI]

**Question 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?**



47% (70) of respondents agreed with the proposal, 29% (44) partially agreed and 17% (25) did not agree. There was full or partial support for earlier engagement between companies and NHS agreement across all stakeholders groups.

**Companies**

39% of companies did not agree with the proposal, 22% agreed and 37% partially agreed.

Highlighted comments:

### **Agreed/Partially agreed**

- ‘a clear framework and timeline for this process should be put in place. This would enable industry to gather relevant information earlier so that conversations could be held in parallel to the NICE TA process; this could avoid delays and enable rapid access close to market authorisation.’ [MSD UK Ltd]
- ‘early negotiation between all parties should take place that sets the commercial conditions for the entry of the new technology in the UK market. This negotiation should address both value for money (eg; cost-effectiveness, via NICE appraisal) and any ‘exceptional’ affordability issues (eg; managed entry arrangements to help NHSE with financial planning), and not introduce any further delays to approval, implementation and patient access’ [Janssen and J&J Medical]

### **Disagreed**

- ‘we object in the strongest terms to any ‘commercial agreements’ that result in additional price cuts in order to make available a technology that has already successfully navigated a very stringent assessment by NICE’ [Roche Products Ltd]
- ‘we are of course supportive of the principle of commercial arrangements with NHSE, however a conversation with NHSE triggered by an arbitrary affordability threshold does not provide the flexibility we require and comes too late in the day to be of any value.’ [Merck]

### **Patient/Carer organisations**

49% of patient/carers organisations agreed with this proposal, whilst only 8% disagreed.

All comments in agreement highlighted the benefit of earlier engagement between NHS England and companies:

- ‘dialogue between NHS England and pharmaceutical companies and manufacturers producing innovative treatments should be a usual part of preparation to deliver a medicine within the NHS’ [Genetic Alliance UK]

### **NHS commissioning bodies**

67% of commissioning bodies agreed with this proposal, 10% disagreed. Alongside this broad agreement was a desire for greater transparency over pricing:

- 'It is essential commissioners are informed of the detail of the agreements to ensure that costs charged to the NHS reflect the agreements made and the benefit to NHS is to be realised in practice' [Surrey Downs CCG]

### **Other responses**

Comments that agreed with the proposal:

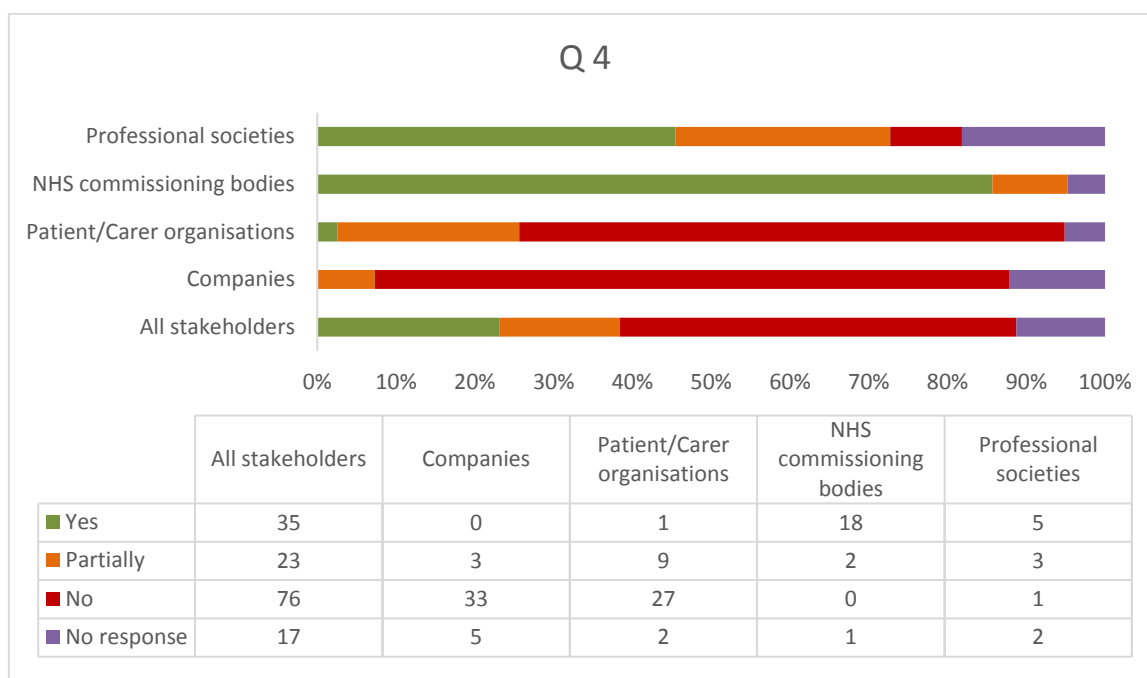
- 'We consider it reasonable for NHS England to negotiate further discounts with companies for technologies which are considered to be cost-effective by NICE but which have a substantial budget impact. We are however unsure what leverage could be applied by NHS England if the company knows that the NICE recommendation is to be positive and mandatory funding will eventually follow. A delay in funding may be insufficient to convince companies to provide additional discounts' [University of Sheffield]

Comments that disagreed with the proposal:

- 'It is also unclear why NICE needs to be involved in assessing budget impact if it is NHS England that will be using this information in negotiation with industry. Presumably the mandate and expertise to perform such an assessment also exists in NHS England, where it could be performed without any concerns about loss of independence or scientific integrity' [King's College London]

## Section 2 – Varying timescales for the funding requirement

**Question 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?**



23% (35) of respondents agreed with the proposal, 16% (24) partially agreed and 50% (76) did not agree.

### Companies

80% of companies disagreed with this proposal, only 8% partially agreed and none agreed.

Highlighted comments:

### Disagreed

- 'Budget impact is only one of many considerations, so to use this as the single metric for varying recommendations would be inappropriate' [Medtronic]
- 'Where NICE has found a technology to be cost-effective, breaking the link to the funding requirement undermines the NICE process and is contrary to the current PPRS agreement. In particular, the lack of clearly defined timelines suggests NHSE could pursue delaying tactics via this mechanism to put

additional pressure on companies to drop prices further regardless of value or cost effectiveness' [Kyowa Kirin]

- 'Varying the funding requirement for a new technology by extending beyond 90 days denies patients access negating their rights enshrined under the NHS Constitution to treatments which have been deemed as cost effective by NICE. This goes against the recommendations of the AAR and the stated desire for patients to be able to benefit from new technologies faster' [Roche Products]

### **Partially agreed**

- 'If this is based on the assumption that the initial years of implementation for a technology are likely to be more costly, then yes, particularly if this avoids directing funds away from other existing technologies' [Cook Medical UK]

### **Patient /Carer organisations**

Similarly to company responses, a majority of patient/carer organisations, 69%, did not agree with this proposal. One organisation agreed with the proposal and 23% were in partial agreement. Concerns with the proposal centred on the potential delayed access to new treatments.

Highlighted comments:

### **Disagreed**

- 'This measure will create yet another barrier to the adoption of new technologies. It will make the process less transparent and has the potential to cause regular and significant delays in the availability of new treatments.' [Asthma UK]
- 'The purpose of NICE's appraisals in delivering assured patient access would be subverted were delay to become the norm. The emphasis should be on better horizon scanning and budgetary planning, with the onus on companies to provide timely guidance and on NHS England to make the most of its considerable purchasing power' [Specialised Healthcare Alliance]

### **Partially agreed**

- 'If there is to be a variation it needs to involve the relevant patient organisations and clinicians, with a transparent process and adhere to a well-defined timescale, with a trigger for an appeal.' [Gauchers Association]

## **NHS commissioning bodies**

In contrast to companies and patient/carer organisations there was broad support for this proposal amongst NHS commissioning bodies. 86% agreed with the proposal with none disagreeing. Comments received were hopeful that the provisions should apply to CCG's as well.

- 'This should include any technologies which are the responsibility of CCGs' [NHS Dorset Clinical Commissioning Group]

## **Other responses**

Example of comment in agreement:

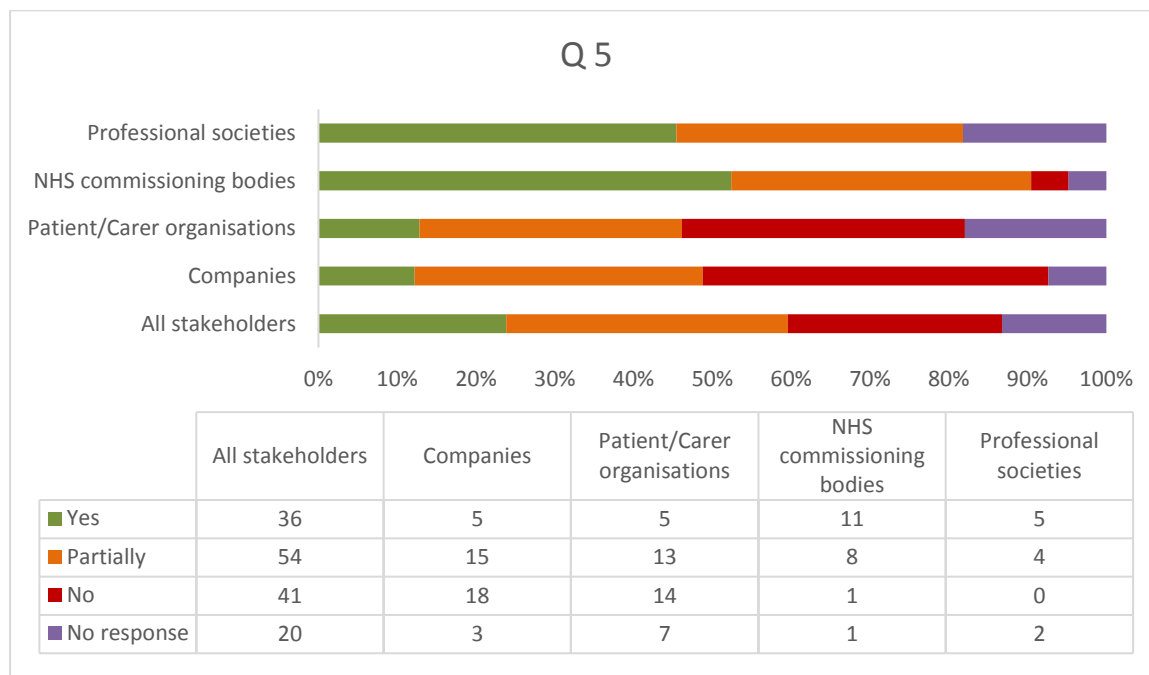
- 'NICE approving new technologies for use in the NHS based on cost-effectiveness explicitly does not consider budget impact. As NHS England is the budget holder, it makes sense for the budget holder to be in a position to influence when mandatory funding should begin' [BMJ]

Examples of comments that disagree:

- 'there seems to be a contradiction of principles between the pursuit of obtaining faster NICE recommendations for new drugs and relaxing how long it takes before NHS Trusts must find the resources to fund these technologies. It would be somewhat perverse to introduce new processes that demand the rapid appraisal of new technologies but then to increase the lag between their positive recommendation and their availability on the NHS' [University of Sheffield]
- 'By seeking to avoid the legal funding requirements for NICE technology recommendations for some medicines, NHS England's proposals will irrevocably weaken guarantees within the NHS Constitution and will likely further limit patient access to new cost effective medicines.' [ABPI]

## Section 3 – NICE Fast Track process

**Question 5: Do you consider that the criteria for the fast track process are appropriate? If not, what other criteria do you suggest?**



24% (36) of respondents agreed with the proposal, 36% (54) partially agreed and 27% (41) did not agree. There was a frequent misunderstanding that fast track topics would be prioritised for appraisal at the expense of carrying out standard appraisal topics.

### Companies

There was a fairly even split between companies that agreed or partially agreed, 12% and 37% respectively, and those that disagreed, 44%. Companies welcomed the option of a streamlined appraisal route, whilst some felt the criteria were too narrow.

Highlighted comments:

#### Agreed

- 'We believe attempts to accelerate appraisal timelines would be in the interests of the NHS' [Alnylam Pharmaceuticals]
- 'if NICE and NHS England truly want to speed up access to the medicines that will have the greatest impact on patient outcomes then they need to apply



this process to all innovative new medicines that are a step change in care or offer significant efficiencies and improvements to the patient pathway and patient experience' [Bayer]

### **Disagreed**

- 'As a principle, it is flawed. The industry supports NICE Guidance because a positive recommendation with the associated mandatory implementation and funding should be the fast track route to patient access, compared to those technologies not assessed by NICE. A fast track process implies an inconsistency with the NICE assessment process, when specific guidance (and the health of specific patients) is assigned greater importance over others simply due to upfront cost' [Akcea Therapeutics]
- 'we have concerns that the criteria outlined in the proposal are most likely to prioritise and accelerate access to those medicines where the unmet need is lower and where there are already established treatment options, thus creating perverse incentives for companies to disinvest in the most innovative therapies. This is in stark contrast to other government initiatives, such as EAMS and the Accelerated Access Review, which aim to ensure that acceleration is focused on areas of greatest unmet need' [Novartis]

### **Patient/Carer organisations**

Patient/Carer organisations were also split evenly between agreeing/partially agreeing, 13% and 33% respectively, and disagreeing, 36%, with the suggested criteria for a Fast Track process.

Highlighted comments:

### **Agreed**

- 'We are supportive of a faster and simpler process for very cost-effective medicines, whilst recognising that not many new medicines will fall into this category, so this new route will be of limited benefit to most patients.' [Breast Cancer Now]
- 'Any steps that aim to speed up patient access to medicines are to be welcomed' [Bloodwise]

### **Disagreed**

- 'We take the view that the fast track appraisal process is inequitable and potentially discriminatory across the board for new technologies' [Society for Mucopolysaccharide Diseases]

- ‘It is essential that NICE continues to focus on the development and implementation of robust processes for all new technologies, irrespective of price and taking into account the challenges of appraising technologies for ultra-rare diseases where patient numbers are very small.’ [Niemann-Pick UK]

## **NHS commissioning bodies**

There was a high level of support for the proposed Fast Track appraisal amongst NHS commissioning bodies, 90% either agreed or partially agreed with the proposal. Only one organisation did not agree with the proposed criteria.

Highlighted comments:

### **Agreed**

- ‘Commissioners would want to be assured that the evidence provided to meet the criteria was robust’ [Surrey Downs CCG]
- ‘The criteria are appropriate but again, this process should be available for all eligible technologies not just those commissioned by NHS England’ [Eastbourne Hailsham & Seaford CCG, Hastings and Rother CCG]

### **Other responses**

Comments agreeing with proposed criteria:

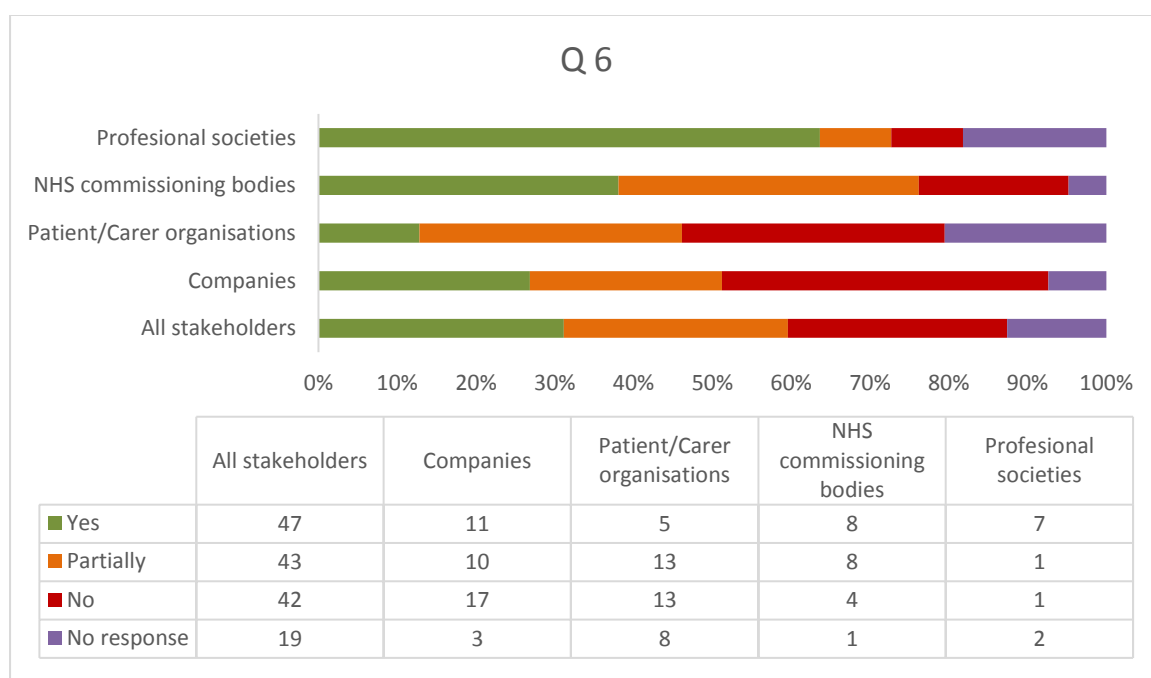
- ‘This is long overdue, and represents a valuable addition to the process where a quick & straightforward decision can be made.’ [Individual]
- ‘Timeliness is critically important and a fast track process for those technologies with a QALY less than £10,000 will result in earlier clarity on a larger number of technologies and provide patient benefit in areas of unmet clinical need at an earlier stage.’ [All Wales Medicines Strategy Group]

Comments that did not agree with the proposed criteria:

- ‘The criteria are fundamentally wrong – it’s not a threshold issue, it’s a matter of decision uncertainty. I also consider the biggest loss in health benefits from the NICE process is the de facto use of a £30K threshold for all appraisals and low levels of implementation. The potential benefits of getting a couple of drugs into the NHS three months quicker are negligible in comparison to these other issues.’ [University of Sheffield]
- Requirements for strong evidence and low uncertainty could delay patient access as companies would have to conduct studies in larger populations and

for longer duration, discouraging early access to medicines which could bring value to patients [MAP BioPharma Limited]

**Question 6: Do you agree that NICE should ‘fast track’ new health technologies with a maximum incremental cost effectiveness ratio of £10,000 per QALY and whose costs are estimated to fall below the budget impact threshold?**



31% (47) of respondents agreed with the proposal, 28% (42) partially agreed and 28% (42) did not agree. There was widespread disagreement to include a budget impact above £20m as a criterion for the fast track appraisal process.

**Companies**

51% of company responses either agreed (27%) or partially agreed (24%) with the proposal. 41% of companies did not agree with the proposal, again showing a close split in opinions.

Highlighted comments:

**Agreed**

- ‘the lower QALY removes a lot of the current gaming in the system to achieve current WTP thresholds’ [Medtronic]

- ‘Boehringer Ingelheim believes that a threshold of £10,000 per QALY gained for ‘fast track’ health technologies is reasonable for signalling cost-effectiveness; however, clarification is needed as to the definition of “a low degree of decision uncertainty” [Boehringer Ingelheim]

### **Disagreed**

- ‘the challenge will be on whose methods are used. Every technology appraisal has had disagreement between the ERG and the manufacturer on the base case assumptions for the cost effectiveness models. Therefore if the fast track appraisal is going to work there has to be more standardisation of model frameworks for different diseases.’ [Amicus Therapeutics Ltd]
- ‘AstraZeneca does not support the Fast Track proposal being reserved for health technologies that meet the proposed budget impact threshold. Fast Track of a medicine should be based on ICER vs. primary comparator alone.’ [AstraZeneca]

### **Patient/Carer organisations**

33% of patient/carers organisations disagreed with this proposal, mainly representing rare or ultra-rare diseases. Another 33% partially agreed with the proposals, whilst 13% fully agreed with the proposals.

Highlighted comments:

### **Agreed**

- ‘We are supportive of a faster and simpler process for very cost-effective medicines, whilst recognising that not many new medicines will fall into this category, so this new route will be of limited benefit to most patients’ [Prostate Cancer UK]
- ‘On the basis that these interventions are of sufficient interest anyway this seems like a good pragmatic approach’ [The Cure Parkinson’s Trust]

### **Disagreed**

- ‘It is a concern that companies will see this as an opportunity to concentrate R&D funding for those technologies under the £10,000 threshold’ [Batten Disease Family Association]
- ‘It is also illogical to send the signal to pharmaceutical companies that drugs below the £10,000 threshold will be automatically approved, which may mean that the drug is extremely valuable to the NHS, but then to stop implementation if they exceed the budget impact threshold. More analysis is

required into how many of drugs eligible for the fast-track process would breach the budget impact threshold.’ [Myeloma UK]

### **NHS commissioning bodies**

38% of NHS commissioning bodies agreed with the proposal, another 38% partially agreed with 19% disagreeing.

Highlighted comments:

#### **Agreed**

- ‘This will encourage companies to market costs below £10K which is good’ [Guildford & Waverley Clinical Commissioning Group]

#### **Disagreed**

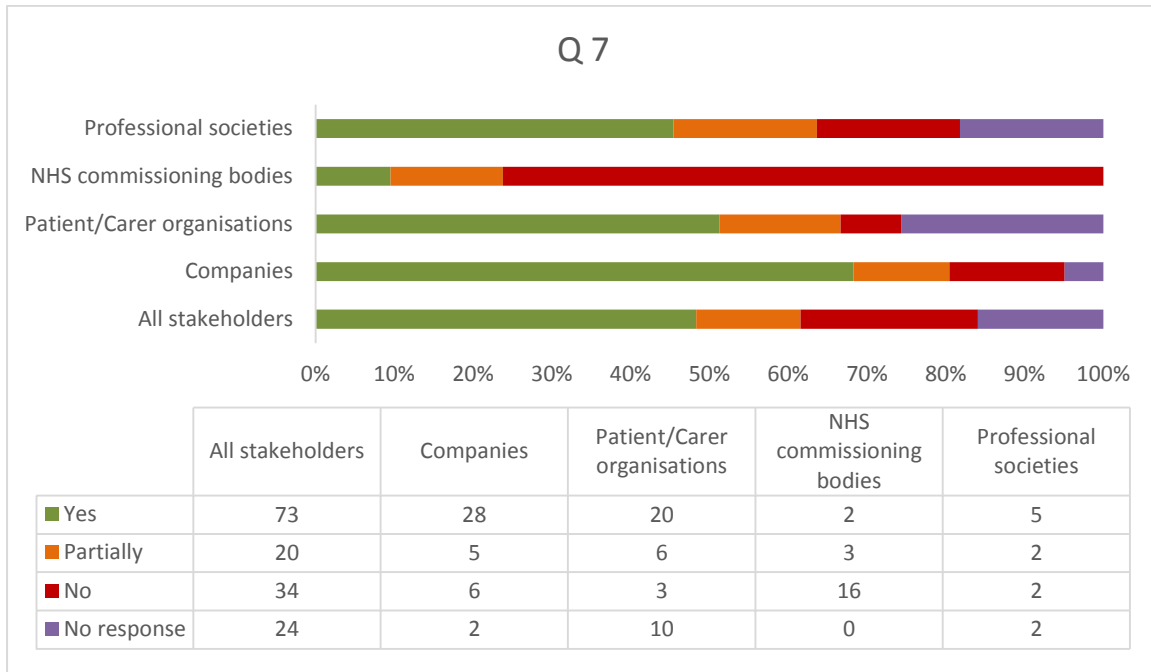
- ‘No, the threshold should be lower. £10,000/QALY is still relatively high as the cost-effectiveness of the NHS is estimated to be £13,000/QALY (K Claxton et al.).’ [North Central London Joint Formulary Committee]
- ‘we do not feel that even this is affordable given the current financial situation’ [York and Scarborough medicines Commissioning Committee]

#### **Other responses**

Comments in agreement:

- ‘Yes, provided NICE can establish a process which mitigates the risks of reaching the wrong recommendation and minimises the proportion of cases where the appraisal is re-routed to the usual STA process’ [University of Sheffield]
- ‘this would also encourage more competitive pricing of new products’ [British Society for Allergy and Clinical Immunology]

**Question 7: Do you agree that NHS England should commit to accelerating funding for technologies approved under the fast track process from 90 days to 30 days?**



49% (73) of respondents agreed with the proposal, 13% (20) partially agreed and 23% (34) did not agree.

**Companies**

There was wide support for this proposal amongst companies; 68% agreed with the proposal, a further 12% partially agree and 15% disagreed.

**Highlighted comments**

**Agree**

- ‘We support this proposal and would encourage NHS England to ensure robust processes are in place and well communicated to ensure these timelines can be met, both for technologies which fall under specialised commissioning as well as those commissioned by CCGs’ [Boston Scientific]
- ‘This proposal however raises the question of why it is not possible, or even greatly preferable, for NHS England to commit to accelerating the funding for all technologies approved to 30 days. These therapies have been demonstrated to be cost-effective compared to the existing standard of care and so represent a more efficient use of NHS funding.’ [Bristol-Myers Squibb]

**Disagree**

- ‘As this proposal stands, it is counterintuitive that less innovative therapies with an ICER of £10K per QALY should benefit from faster implementation than more innovative medicines with an ICER of £10K-£20K per QALY, when both are considered by NICE to be cost-effective’ [Novartis]
- ‘Whilst NHS England may be able to promise funding within 30 days of TAG for Fast Track-approved technologies which sit within specialised services, many technologies assessed by NICE will ultimately be funded by CCGs, and it is unlikely that NHS England can commit to such rapid funding on behalf of CCGs, although we would welcome this if it were made possible’ [Novartis]

### **Patient/Carer organisations**

Patient/Carer organisations also welcomed this proposal; 51% agreed, 15% partially agreed and only 8% disagreed.

Highlighted comments:

#### **Agreed**

- ‘From a patient’s point of view the faster we get access to treatment, the better. However perhaps it would be a more realistic aim to ensure that there is consistency in achieving the current 90 day period.’ [PNH Support]
- ‘If drugs are approved it is essential that patients have access to them as soon as possible so we agree that NHS England should commit to accelerating funding for approved technologies to 30 days’ [Parkinson’s UK]

### **NHS commissioning bodies**

NHS commissioning bodies did not support this proposal. 76% disagreed, with only 2 organisations agreeing and 3 partially agreeing. Concerns were raised over the levels of administration required to meet the 90 day implementation target, let alone 30 days.

Highlighted comments:

#### **Agreed**

- ‘As this consultation also applies to CCGs, there would be a willingness from CCGs to fast track technology appraisals but the suggested 30 days in practice would be hard to achieve in CCGs’ [Pharmacy Eastern Network]

#### **Disagreed**

- ‘If CCGs are to be included in this arrangement then it will be important to keep the 3 month implementation rule. Clinical engagement and adjusting

local treatment pathways to accommodate the guidance followed by sign off by local medicines policy development committees are crucial steps to successful implementation. There is no indication that fast tracking a technology in this way will impact on any of the above steps and make the guidance quicker to implement.' [Eastbourne Hailsham & Seaford CCG, Hastings and Rother CCG]

- 'Current governance processes within CCGs are unlikely to allow for approval within 30 days' [East Surrey CCG]

## **Other responses**

Highlighted comments in agreement:

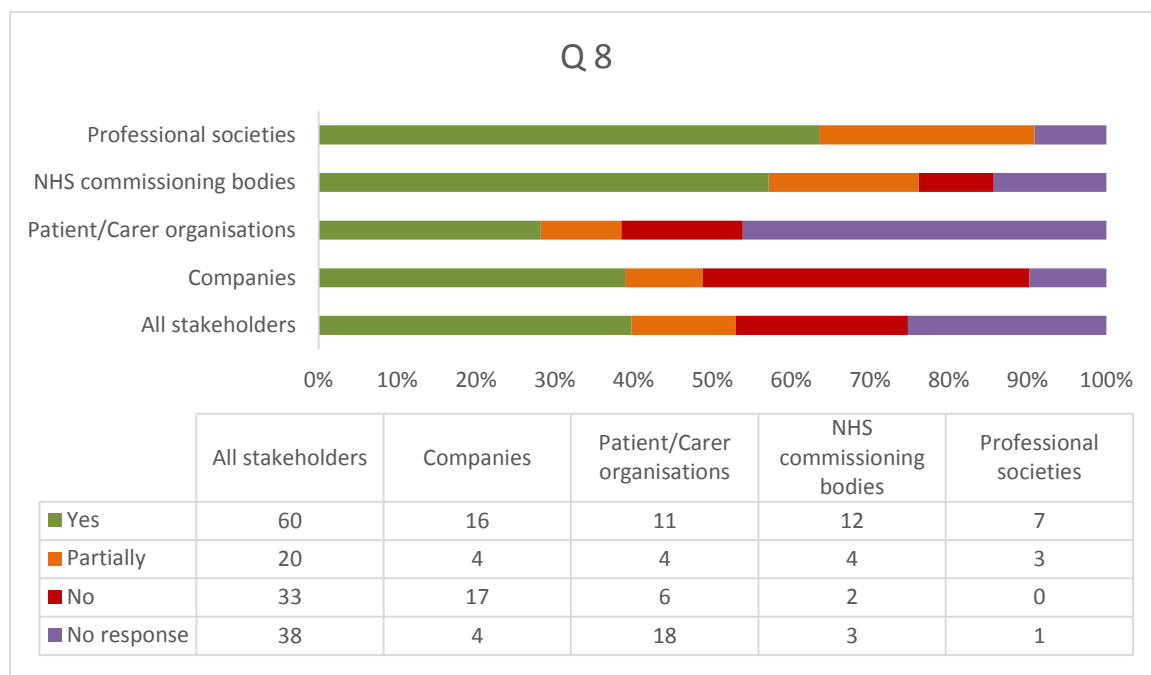
- 'This would benefit patients and could incentivise companies to keep costs low and below the £10,000 threshold.' [Faculty of Public Health]
- 'It is essential that a formal 30 day implementation window does not lead to delays and uncertainty for those products that fall outside the fast-track process' [MAP BioPharma Limited]

Highlighted comments that disagree:

- 'The current 90 days is in place in order that appropriate health resources, including staff are in place. There is no evidence to suggest that, just because a new technology meets the criteria for fast tracking, that such resources can be put in place any more quickly.' [Ethical Medicines Industry Group (EMIG)]
- 'Not unless it can be established that it does not impose an additional administrative burden' [University of York]



**Question 8: Do you agree that NICE should absorb its proposed ‘abbreviated’ technology appraisal process into the proposed fast track process?**



40% (60) of respondents agreed with the proposal, 13% (20) partially agreed and 22% (33) did not agree. The wording of this question caused confusion amongst stakeholders, with some stakeholders agreeing or disagreeing for the same reason – that the ATA/FTA processes should both be available as options.

**Companies**

Highlighted responses:

**Agreed**

- ‘Yes, where the stages are in alignment. Although the 2 distinct routes (abbreviated and fast-track) should remain, given their slightly different objectives.’ [Cook Medical UK]
- ‘Bayer supports the development of a suite of appraisal processes or approaches to ensure the route used is proportionate to the intervention in question’ [Bayer]

**Disagreed**

- ‘These two processes should remain separate routes of appraisal, with differing criteria and outputs’ [Chiesi]

- 'It is difficult to understand how either process is designed to work and the new proposals further confuse the already complex appraisal environment within the UK' [AbbVie]

### **Patient/Carer organisations**

Highlighted comments:

#### **Agreed**

- 'it would be simpler to have one shorter process for very cost effective medicines. We understand from the consultation events that "integrate" is a more accurate description of the intention than "absorb" and it makes sense to align two schemes with similar objectives and scope.' [Prostate Cancer UK]
- 'We agree with this proposal and welcome any move to simplify and consolidate the process for assessing the most cost effective medicines' [The Brain Tumour Charity]

#### **Disagreed**

- 'No. The abbreviated technology appraisal should be used where there is a 2nd or successive generation drug where the budget impact creates a saving or is cost neutral and should apply across all new technologies including NICE HST.' [Association For Glycogen Storage Disease (UK)]

### **NHS commissioning bodies**

The only comments received in response to this question are that this proposal would simplify the process.

#### **Other responses**

Comments in agreement:

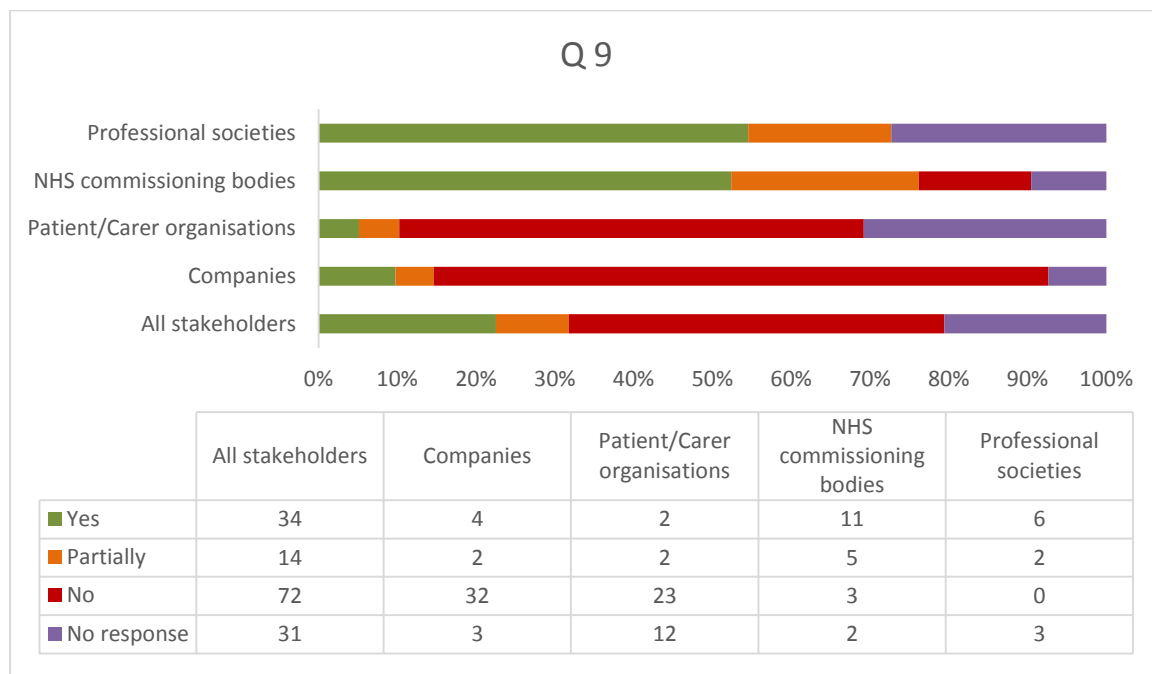
- 'It is not clear what 'absorb its proposed 'abbreviated' technology appraisal process into the proposed fast track process' means. That these two processes should as far as possible be the same seems sensible' [University of York]
- 'As long as the consolidated process is equally or more efficient than its existing predecessors. The new absorbed appraisal process must also be appropriately explained to all levels of stakeholders so they can engage and monitor its effectiveness' [Brain Tumour Research]

Comments that did not agree:

- ‘The criteria for each of the processes are different and they should remain separate’ [European Medicines Group (EMG)]

## Section 4 – Linking NICE and NHS England processes for evaluating highly specialised technologies

**Question 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?**



23% (34) of respondents agreed with the proposal, 9% (14) partially agreed and 48% (72) did not agree.

### Companies

There was strong opposition to this proposal from companies, 78% of whom did not agree with the introduction of cost per QALY for assessing highly specialised treatments. 10% agreed with the proposal and 5% partially agreed.

Highlighted comments:

### **Agreed**

- 'We support this proposal to use a similar methodology to evaluate the applicability of funding requirements for Highly Specialised Technologies as for other technologies.' [Boston Scientific]

### **Disagreed**

- 'No evidence has been presented as to why such a change of approach is needed. HST was established on the understanding from NICE that for ultra-orphan conditions a cost per QALY is an inappropriate metric to fully assess the benefit of such medicines' [Amicus Therapeutics Ltd]
- 'We are strongly opposed to the introduction of a specific cost-effectiveness threshold into the HST process but we support further research to develop an appropriate structured decision making process for ultra-orphan medicines, as well as orphan medicines' [Shire Pharmaceuticals]
- 'the funding requirement should be based on unmet clinical need. A process that allows a holistic consideration of the clinical outcomes, unmet need and budget impact would appear to provide a much better indication of the value that a particular treatment might bring to patients and to the NHS. It can already be seen from the technologies that have been reviewed via the HST process that a cost per QALY threshold is inappropriate to assess and fully capture the value of these technologies' [PTC Therapeutics]

### **Patient/Carer organisations**

Patient/carers organisations were also strongly opposed to this proposal. The majority of patient/carers organisations involved in the consultation represented rare diseases. 59% disagreed with the proposal. Only 10% either agreed or partially agreed, split evenly between the options.

Highlighted comments:

### **Agreed**

- 'If this proposal helps more medicines to receive a positive recommendation then we would support this proposal' [Breast Cancer Now]

### **Disagreed**

- 'We do not believe that cost per QALY should apply to Highly Specialised Technologies' [Batten Disease Family Association]

- ‘There are significant problems in relation to QALYs and rare disease medicines. The implication of this proposal is that access to rare disease medicines above a threshold cost per QALY would be blocked’ [Tuberous Sclerosis Association]
- ‘Whilst QALY is a rational standard, there needs to be a movement to consider a more holistic approach and other health economic formulas and the involvement of healthcare professionals, economic specialists and most importantly patients and carers.’ [Action Duchenne]

### **NHS commissioning bodies**

Once again, the NHS commissioning bodies contrasted the views of companies and patient/carer organisations by supporting this proposal. 52% agreed with the proposal, with a further 28% partially agreeing. 14% NHS commissioning bodies disagreed with the proposal.

Highlighted comments:

#### **Agreed**

- ‘This would seem appropriate from an equity perspective as all other treatments considered by NICE use a cost per QALY threshold’ [NHS East and North Hertfordshire CCG]

#### **Disagreed**

- ‘The cost/QALY needs to be equitable for all. I think if there are different cost/QALY for different things then the process could be up for challenge as to why?’ [Chiltern and Aylesbury Vale Clinical Commissioning Groups]

#### **Other responses**

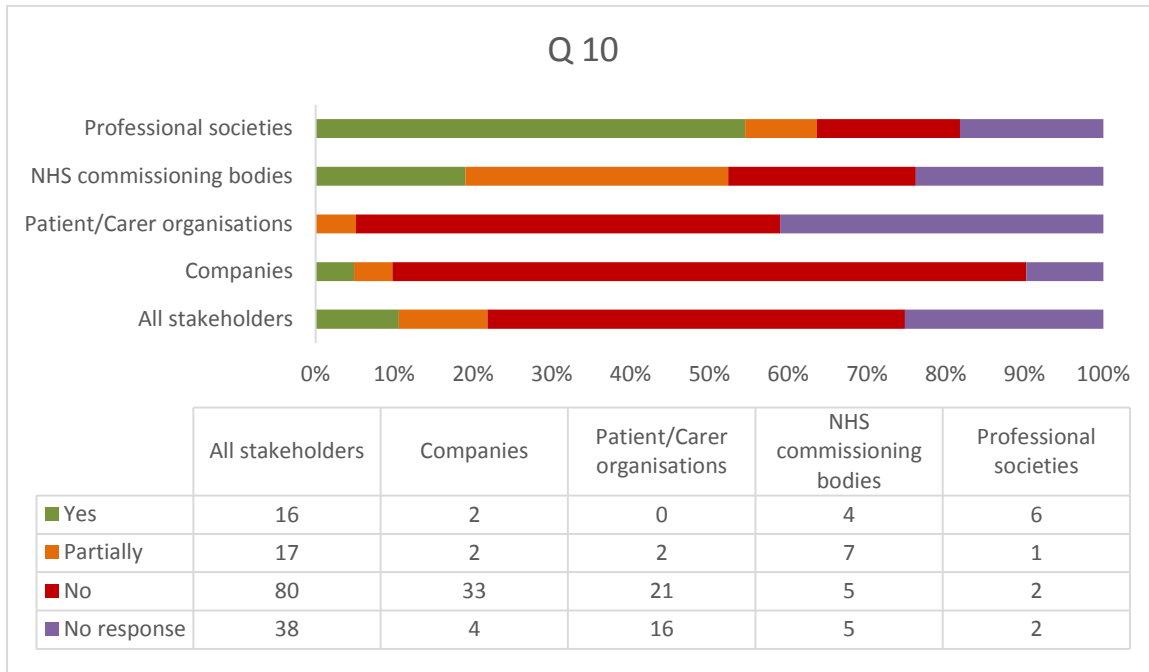
Highlighted comments in agreement:

- ‘The healthcare system operates on a finite budget, money spent in one area is not spent in another. It should therefore all be treated with extreme care.’ [Individual]

Highlighted comments that disagree:

- ‘This could disadvantage patients with extremely rare diseases, and could deter manufacturers from developing innovative treatments for extremely rare conditions’ [All Wales Medicines Strategy Group]

**Question 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?**



11% (16) of respondents agreed with the proposal, 11% (17) partially agreed and 53% (80) did not agree.

**Companies**

Again, companies were opposed with this proposal. 80% disagreed with only a combined 10% either agreeing or partially agreeing.

Highlighted comments:

**Agreed**

- 'Boehringer Ingelheim believes that the proposed threshold of £100,000 per QALY gained for Highly Specialised Technologies is reasonable' [Boehringer Ingelheim]

**Disagreed**

- 'None of the 3 medicines that have gone through HST to date were close to being £100,000 per QALY and it is fair to say that it is unlikely for any ultra-orphan medicine to achieve and ICER below based on the incremental costs (especially if versus no treatment/palliative care) and incremental QALY gain seen for rare diseases' [Amicus Therapeutics Ltd]

- ‘£100,000/QALY is an arbitrary figure, not underpinned by validated methodology.’ [Amgen Ltd]
- ‘There is a lack of transparency reported within the consultation document as to how the cost effectiveness threshold of £100,000 has been derived. With no validated methodology underpinning the threshold presented it is not possible to comment as to whether it is the right maximum. Given the nature of the HST process, it seems unlikely that many medicines for rare diseases would fall under the proposed threshold of £100,000 per QALY. Therefore, the mandatory requirement for funding by NHS bodies would be lost, disadvantaging patients with limited/ if any treatment options’ [MSD UK Ltd]

### **Patient/Carer organisations**

Patient/Carer organisations were also opposed to this proposal. 53% disagreed, 2 organisations partially agreed but none supported the proposal.

Highlighted comments:

#### **Agreed**

- ‘Broadly yes, but there should be a degree of flexibility built into the threshold for special circumstances, eg for older patients where the cost of life-changing treatments are likely to be amortisable over fewer years than for younger patient’ [Leber’s Hereditary Optic Neuropathy Society]

#### **Disagreed**

- ‘Going forward the £100,000 cost per QALY will without any doubt condemn children and adults with an ultra-rare disease to an early death by an arbitrary Government health policy’ [Association For Glycogen Storage Disease (UK) Limited, Gauchers Association and the Society for Mucopolysaccharide Diseases]
- ‘We do not understand how and why the £100,000 cost per QALY was chosen. NICE and NHSE must set out how they reached this estimate’ [Cancer 52 and CML Support]
- ‘The HST QALY ceiling of £100,000 per QALY doesn’t appear to be rooted in a rigorous methodology and it is unclear how this figure has been calculated. Inclusion of an arbitrary figure not rooted in evidence violates NICE’s Charter’ [Cystic Fibrosis Trust]

## **NHS commissioning bodies**

33% of NHS commissioning bodies partially agreed with this proposal, with a further 19% agreeing. 24% did not agree.

Highlighted comments:

Stakeholders that agreed with the £100,000 threshold did not provide any additional comments.

### **Disagreed**

- 'should be less; the NHS cannot afford this' [South Worcestershire CCG, Redditch and Bromsgrove CCG and Wyre Forest CCG]
- 'if a more lenient threshold is given then funding will be needed from the government to implement this. It will be highly expensive' [Chiltern and Aylesbury Vale Clinical Commissioning Groups]

### **Other responses**

Highlighted comments that agree:

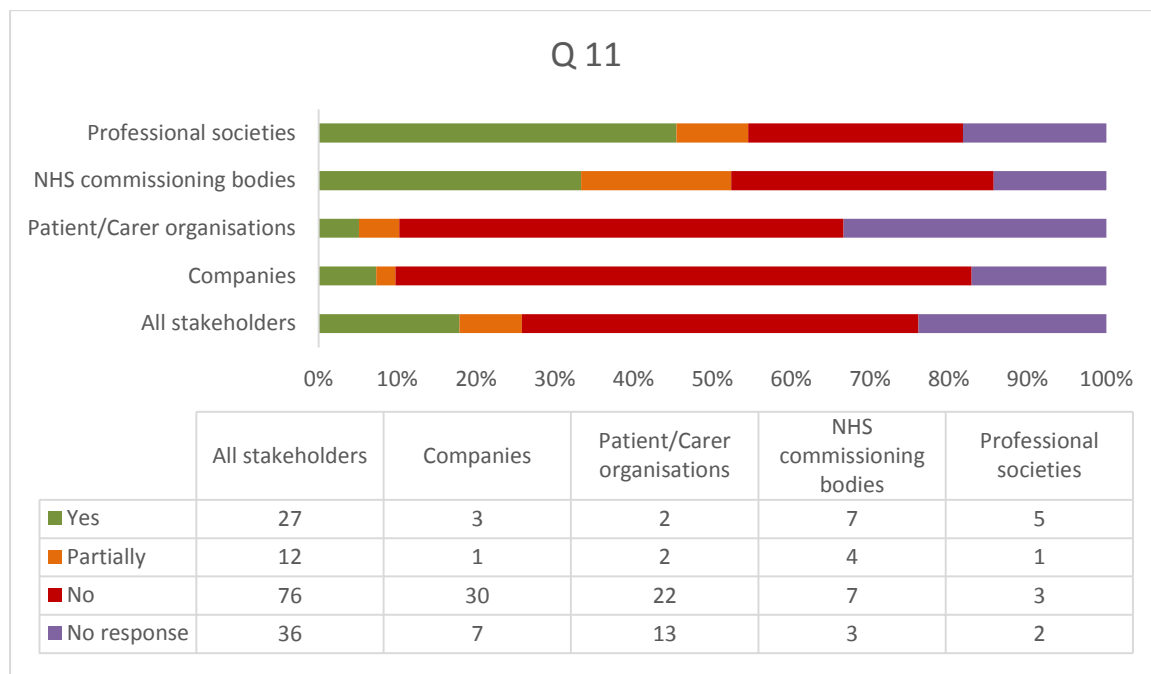
- 'On balance, this is reasonable, given the potential development costs and small population to treat.' [The Royal College of Ophthalmologists]
- 'Agree that a much higher threshold is needed for these drugs' [Royal College of Paediatrics and Child Health]

Highlighted comments that disagree:

- 'There are very valid reasons why society may be willing to accept a higher cost effectiveness threshold for innovative technologies targeting rare and very rare diseases. However, NICE's appraisal committees have historically dealt with such considerations through deliberation and discretionary judgement rather than through the operation of a hard threshold' [King's College London]
- 'In the absence of a commissioning framework that establishes the special status of rare and ultra rare diseases, we consider that the threshold of £100,000 is too high as it will displace much more cost effective technologies for other conditions' [Faculty of Public Health]
- 'this appears to be an arbitrary sum, dependent on factors that have not been fully described' [Brain Tumour Research]



**Question 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?**



18% (27) of respondents agreed with the proposal, 8% (12) partially agreed and 50% (76) did not agree.

**Companies**

Reinforcing their opposition to the proposals on HST 73% of companies did not agree with this proposal. 10% either agreed or partially agreed with the proposal.

Highlighted comments:

**Agreed**

- ‘Any opportunity for dialogue in these situations is welcome. However, any alternative “prioritisation process” for technologies which exceed the cost per QALY level for HST should be well-defined and subject to rigorous consultation and transparency, as is the case for all NICE appraisal methods’ [Biogen]

**Disagreed**

- ‘If technologies have to first go through NICE assessment to determine if they exceed what is clearly an arbitrary threshold, then this would cause lengthy

delays to access, particularly in cases where the timing of NICE assessment does not align with scheduled NHSE prioritisation rounds' [Amgen Ltd]

- 'The NHS England prioritisation process is not the optimal route for highly specialised technologies due to existing delays and a lack of transparency in the process by which decisions are made.' [MAP BioPharma Limited]
- 'Greater clarity, transparency of process and speed of process would be needed before this route for commissioning could be supported' [NAPP Pharmaceuticals]

### **Patient/Carer organisations**

Again, patient/carers organisations did not support this proposal. 56% did not agree with the proposal, 10% either agreed or partially agreed with the proposal.

Highlighted comments:

#### **Agreed**

- 'if it seems that if this is a very special case it seems sensible to consider it under a different category and probably under different budget constraints' [The Cure Parkinson's Trust]

#### **Disagreed**

- 'It is surely mistaken for NICE and NHS England to propose directing Highly Specialised Technologies to an assessment and prioritisation route which has been acknowledged as deficient in that... The ultimate impact would be a discriminatory, slow moving system which failed to facilitate innovation for smaller patient groups, potentially creating a serious breach of trust' [Specialised Healthcare Alliance]
- 'CPAG's "one size fits all" prioritisation mechanism disfavors interventions for smaller patient populations. Furthermore, the sequential review of medicines would inevitably impede timely uptake of innovative medicines.' [Tuberous Sclerosis Association]
- 'The specialised commissioning prioritisation process is not currently fit for purpose with respect to its current remit. The process should be functional before its scope is expanded.' [Genetic Alliance UK]

## **NHS commissioning bodies**

NHS commissioning bodies were divided in response to this proposal. 33% agreed with the proposal whilst another 33% disagreed. 19% partially agreed with the proposal.

Highlighted comments:

No additional comments were provided by organisations that agreed with this proposal.

### **Disagreed**

- 'The NHS should utilise one source of information that considers value for money and that should be NICE – if NICE do not accept that the technology meets that threshold then the NHS should not commission it' [South East London Area Prescribing Committee]
- 'As the process splits there is a danger that inconsistent appraisal methodologies are used and potential for lower access criteria for higher cost therapy' [East Surrey CCG]

### **Other responses**

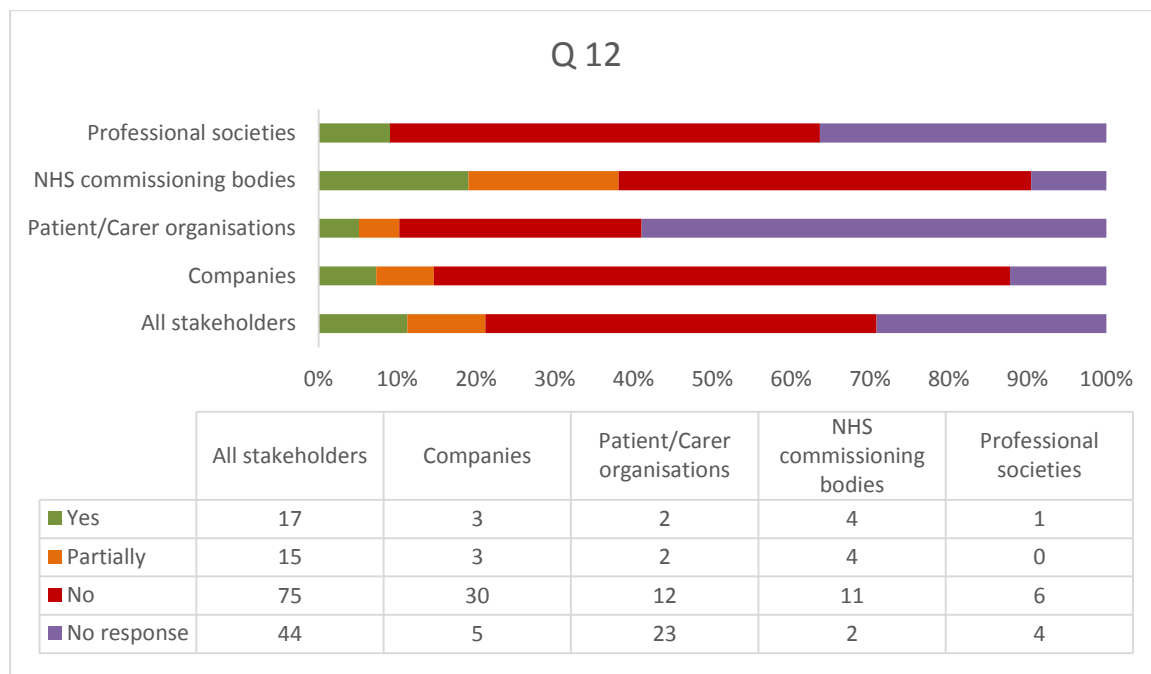
Highlighted comments that agree with the proposal:

- 'We would be supportive of this approach, as long as there is a robust, fair and transparent process for prioritising medicines that exceed £100,000 per QALY alongside all other technologies that enter the annual prioritisation process' [Welsh Health Specialised Services Committee]

Highlighted comments that disagree with the proposal:

- 'The cost-effectiveness threshold should be set at an appropriate level where this would not be required for new highly specialised technologies.' [BMJ]
- 'We do not consider this arrangement to be fair because it places a small (and poorly defined) subset of technologies at a significant advantage compared with others' [King's College London]
- 'The NHS England prioritisation process is not the optimal route for highly specialised technologies due to existing delays and a lack of transparency in the process by which decisions are made' [European Confederation of Pharmaceutical Entrepreneurs]

**Question 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?**



11% (17) of respondents agreed with the proposal, 10% (15) partially agreed and 50% (75) did not agree.

**Companies**

73% of companies did not agree that budget impact should not be considered for HST evaluations. 15% either agreed or partially agreed with the proposal.

Highlighted comments:

**Agreed**

No substantive comments were received from companies that agreed with this proposal.

**Disagreed**

- ‘The previous HST positive recommendations have, invariably, incorporated a managed access agreement, as such it would be potentially ill advised to suggest that budget impact will no longer be a key consideration with highly specialised treatments.’ [Akcea Therapeutics]

- ‘The “purity” of the NICE process should be maintained and its guidance should reflect the cost-effectiveness and benefit that the technology brings to the NHS. There should not be change to the HST process.’ [Napp Pharmaceuticals]

### **Patient/Carer organisations**

58% of patient/carers organisations did not respond to this question. 30% did not agree with the proposals and once again 10% either agreed or partially agreed with the proposal.

Highlighted comments:

#### **Agreed**

- ‘Budget impact threshold is unlikely to be exceeded due to the rarity of the diseases considered in the HST process’ [Kidney Cancer Support Network]

#### **Disagreed**

- ‘Budget impact should be considered, as long as it is done so in a fair, equitable and transparent way that does not discriminate against ultra-rare patient communities’ [Niemann-Pick UK]

### **NHS commissioning bodies**

In contrast to responses to the previous HST questions, NHS commissioning bodies did not agree that budget impact should not be considered. 52% did not agree with the proposal, 21% agreed and another 21% partially agreed.

Highlighted comments:

#### **Agreed**

No substantive comments were received from NHS commissioning bodies that agreed with this proposal.

#### **Disagreed**

- ‘This is assuming that budgetary impact is all placed on NHSE as opposed to CCGs. It may be determined that CCGs are the commissioners of the technology and if this is the case, the budget impact would be applicable as there is no singular prioritisation process for CCGs as there is for NHSE’ [Bedfordshire Clinical Commissioning Group]

- ‘any consideration should be a balance of value for money and affordability and therefore the proposed £20m threshold is equally applicable’ [South East London Area Prescribing Committee]

### **Other responses**

Highlighted comments that agree with the proposal:

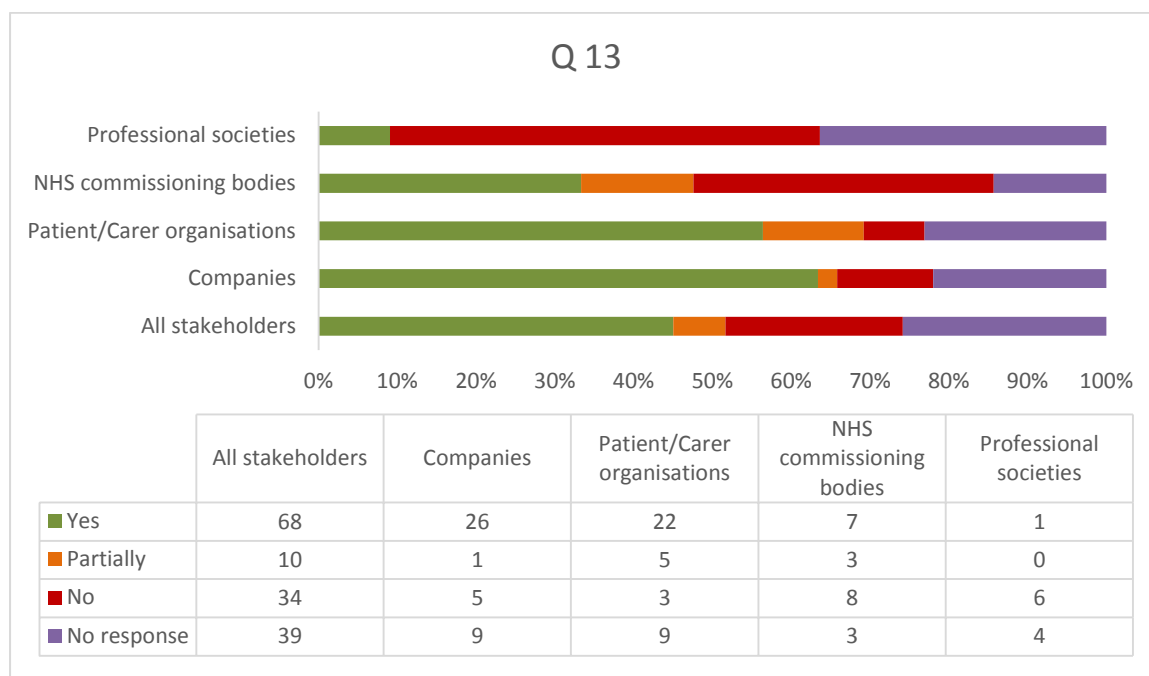
- ‘I expect so, as the population sizes are so small that the threshold is unlikely to be affected the size of spend’ [University of Sheffield]

Highlighted comments that disagree with the proposal:

- ‘Budget impact will always need to be taken into account’ [Institute for Clinical and Economic Review]
- ‘while budget impact is seldom significant for very rare conditions, some assessment would nevertheless continue to make sense as part of a financially aware approach to commissioning.’ [European Confederation of Pharmaceutical Entrepreneurs]

## Section 5 – General comments

**Question 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?**



45% (68) of respondents agreed that the proposals would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation, 7% (10) partially agreed and 23% (34) did not agree.

### Companies

63% of companies agreed that the proposals would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation, 12% did not agree and one organisation partially agreed.

Highlighted comments:

#### Yes

- 'we have some concern that the implementation of the budget impact threshold is likely to disproportionately affect technologies related to cancer treatment. This, in combination the decision to assess all cancer treatments and other measures now in place to assess promising cancer treatments earlier, suggests an inequitable concentration of resources around oncology' [Roche Diagnostics]

- ‘There is a danger that the proposals for HSTs would in most cases prevent patients with very rare conditions from accessing clinically effective treatments, leaving them behind (and untreated) in a way which was not intended by the NHS Constitution’ [Sobi Ltd]

## No

No substantive comments were received from companies that did not think the proposed changes would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation.

### Patient/Carer organisations

56% of patient/carers organisations agreed that the proposals would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation, 13% partially agreed and 8% did not agree.

Highlighted comments

## Yes

- ‘there are substantial risks attendant to the proposals for Highly Specialised Technologies, which would systematically disadvantage people with rare conditions’ [Specialised Healthcare Alliance]
- ‘As civil servants involved in the health service, the priority should be patients and wider society. The whole premise of this consultation seems to be about cost and not approving the most innovative and promising new medicines that would benefit those of greater need.’ [Action Duchenne]
- ‘We are very concerned about how the budget impact threshold and the associated potential delays would impact on patients with terminal and end of life conditions. These patients cannot afford to wait longer for medicines to be introduced and are often relying on the next breakthrough treatment to become available so they can have another option of treatment. The higher accepted cost per QALY of £50,000 for end of life medicines, would in fact make these medicines more likely to be halted by the budget impact threshold proposals’ [Prostate Cancer UK]

## No

No substantive comments were received from patient/carers organisations that did not think the proposed changes would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation.



## **NHS commissioning bodies**

33% of NHS commissioning bodies agreed that the proposals would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation, 14% partially agreed and 38% did not agree

Highlighted comments:

### **Yes**

- 'We have concerns that disease rarity is a very poorly defined basis for offering differing ICER thresholds. With the rapid advances in genotyping and personalised medicine it seems possible that even relatively common diseases such as breast cancer could be split into a series of rare diseases.' [South East London Area Prescribing Committee]

### **No**

No substantive comments were received from NHS commissioning bodies that did not think the proposed changes would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation.

### **Other responses**

Highlighted comments that feel that the proposals would result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation:

- 'The use of arbitrary thresholds suggests that the sole purpose of NHS England and NICE is to control the financial impact of new medicines. There is not enough consideration given to the clinical and wider value of these treatments and the varied needs of the people who might benefit from them.' [European Confederation of Pharmaceutical Entrepreneurs]
- 'It is understood that no impact assessments have been conducted regarding how either the £20 million budget impact threshold or the £100,000 cost per QALY for HST will impact patient access and outcomes. This is of significant concern. With two out of three of the highly specialised technologies NICE has published final guidance on meeting the £20 million budget impact threshold, it is highly likely that patients with rare diseases would be adversely impacted by these changes' [British Society of Gastroenterology]

Highlighted comments that feel that the proposals would not result in NICE or NHS England failing to comply with their responsibilities under the relevant equalities legislation:

- ‘The Faculty strongly supports this consultation as an important step in improving the equitable provision of effective healthcare’ [Faculty of Public Health]
- ‘No, however this may result in CCGs or providers failing to comply.’ [Bridgewater Community Healthcare NHS Foundation Trust]

### General comments

183 general comments were received in addition to responses to questions included in the consultation. Of these comments, 11 were further comments on budget impact, 2 on varying timescales, and 19 on the FTA process and 17 on HST.

Highlighted general comments:

- ‘Going forward NICE and NHS England need to come up with a policy setting out their expectations on data they require in order to appraise new therapies for ultra-orphan diseases.’ [Society for Mucopolysaccharide Diseases]
- ‘The complexity of the language used in the consultation questions is a barrier to patient groups/representatives engaging with this consultation. The questions could have been put much more simply and perhaps accompanied by an example/diagram/process map where relevant.’ [PNH Support]
- ‘The circumstances for all the situations when technologies do or don’t meet the cost/QALY and/or the budget threshold is confusing and inconsistent. It would help if it could be demonstrated as part of a pathway.’ [South Worcestershire CCG, Redditch and Bromsgrove CCG and Wyre Forest CCG]
- ‘Timescales for implementation proposed as April 2017 – this could have serious implications for CCGs financial and implementation planning who are planning for at least the next 2 years. A clear timetable of which drugs are involved in any of these processes should be published as soon as possible.’ [Thames Valley and Wessex Commissioning Pharmacists Group]
- ‘whilst the initiative is welcomed, BioMarin is concerned that the consultation does not specifically consider the clinical need of the patients. The overarching driver for prioritization of treatments in our view should be based on clinical need where clinical need should consider severity of disease, availability of alternative effective treatment options, potential for substantive improvement in health and quality of life. If a significant need exists for a patient and a treatment is potentially available, then the review of this treatment and any resulting mechanism that enables faster access should be accelerated.’ [BioMarin Europe Limited]

- ‘the proposals at issue here seem to be at odds with the AAR move to accelerate transformative technologies to create patient benefit sooner. In fact, with a budget threshold set to effectively delay implementation of a technology, for example where it could benefit a larger population, the proposals appear to run counter to the AAR altogether’ [Kidney Research UK]
- ‘Please can you clarify how this will impact on EAMs scheme’ [Leeds North CCG, Leeds South & East CCG and Leeds West CCG, Leeds Teaching Hospital NHST, Leeds and York partnership FT]
- ‘The proposals in the consultation document potentially represent that pragmatic way forward up to 2020. The two important caveats are that the costs of new drugs, both those that are fast-tracked and those that are above the cost impact threshold, should be tracked transparently in aggregate and by provider where necessary. This will ensure that the policy is having the intended effect to reduce new cost pressures and that individual providers are properly reimbursed. In the long run, and in the context of the UK’s post-Brexit economy, it will be important that the NHS is properly funded to meet demand, that patients’ access to new medicines and technologies is not constrained and that the NHS is able to remain a globally attractive partner for biomedical researchers and the life sciences industry.’ [The Shelford Group]

## Appendix A

### List of stakeholders

#### Companies

- AbbVie
- Amicus Therapeutics Ltd
- Agendia NV
- AstraZeneca
- Akcea Therapeutics
- Bayer
- Alexion Pharmaceuticals UK
- Biogen
- Alnylam Pharmaceuticals
- BioMarin Europe Limited
- Amgen Ltd
- BlueBird Bio

- Boehringer Ingelheim
- Boston Scientific
- Bristol-Myers Squibb Company
- Celgene UK & Ireland
- Cell Medica, Ltd.
- Chiesi
- Cook Medical UK
- Eli Lilly and Company Ltd
- Genomic Health
- Gilead Sciences Ltd
- GSK
- Incyte Biosciences UK Ltd
- Janssen and J&J Medical
- Kyowa Kirin
- MAP BioPharma Limited
- Medtronic
- Merck
- MSD UK Ltd
- Napp Pharmaceuticals
- Novartis
- Novo Nordisk Ltd
- Pfizer
- PTC Therapeutics
- Roche Diagnostics
- Roche Products Ltd
- Sanofi UK
- Servier Laboratories Ltd
- Shire Pharmaceuticals
- Sobi Ltd

### **Patient/Carer organisations**

- Action Duchenne
- ALD Life
- Alzheimer's Research UK
- Alzheimer's Society
- Association For Glycogen Storage Disease (UK) Limited
- Asthma UK
- Batten disease family association
- Bloodwise
- Breast Cancer Care
- Breast Cancer Now
- Cancer 52
- Children's liver disease foundation

- CML Support Group
- Cystic Fibrosis Trust
- Diabetes UK
- Duchenne UK
- Gauchers Association
- Genetic Alliance UK
- Kidney Cancer Support Network
- Kidney Research UK
- Leber's Hereditary Optic Neuropathy Society
- Leukaemia CARE
- Lymphoma Association
- MS Society
- Muscular Dystrophy UK
- Myeloma UK
- National Aids Trust
- Niemann-Pick UK
- Pancreatic cancer UK
- Parkinson's UK
- PNH Support
- Prostate Cancer UK
- Society for Mucopolysaccharide Diseases
- Specialised Healthcare Alliance
- Target Ovarian Cancer
- The Brain Tumour Charity
- The Cure Parkinson's Trust
- The Haemophilia Society
- Tuberous Sclerosis Association

### **Professional Societies**

- Association of Breast Surgery
- British Association of Dermatology
- British Society for Allergy and Clinical Immunology
- British Society of Gastroenterology
- British Society of Neuroradiologists
- Faculty of Public Health
- Royal College of Paediatrics and Child Health
- Royal College of Physicians
- The Royal College of Anaesthetists
- The Royal College of Ophthalmologists

- UK Neurointerventional Group

## **NHS Organisations**

- Bedfordshire Clinical Commissioning Group
- Bridgewater Community Healthcare NHS Foundation Trust
- Chiltern and Aylesbury Vale Clinical Commissioning Groups
- Derbyshire Joint area prescribing committee
- East of England Priorities Advisory Committee
- East Surrey CCG
- Eastbourne Hailsham & Seaford CCG, Hastings and Rother CCG
- Guildford & Waverley Clinical Commissioning Group
- Leeds North CCG, Leeds South & East CCG and Leeds West CCG, Leeds Teaching Hospital NHST, Leeds and York partnership FT
- NHS Dorset Clinical Commissioning Group
- NHS East and North Hertfordshire CCG
- NHS England Specialised Commissioning (Midlands & East Region)
- Norfolk & Waveney Therapeutics Advisory Group
- North Central London Joint Formulary Committee
- Nottingham City CCG
- Oxfordshire CCG
- Paediatric Neurosciences Clinical Reference Group
- Pharmacy Eastern Network
- Salford Royal NHS Foundation Trust
- South East London area prescribing committee
- South Worcestershire CCG, Redditch and Bromsgrove CCG and Wyre Forest CCG
- Surrey Downs CCG
- Thames Valley and Wessex Commissioning Pharmacists group
- UK Genetics Testing Network

- York and Scarborough Medicines Commissioning Committee

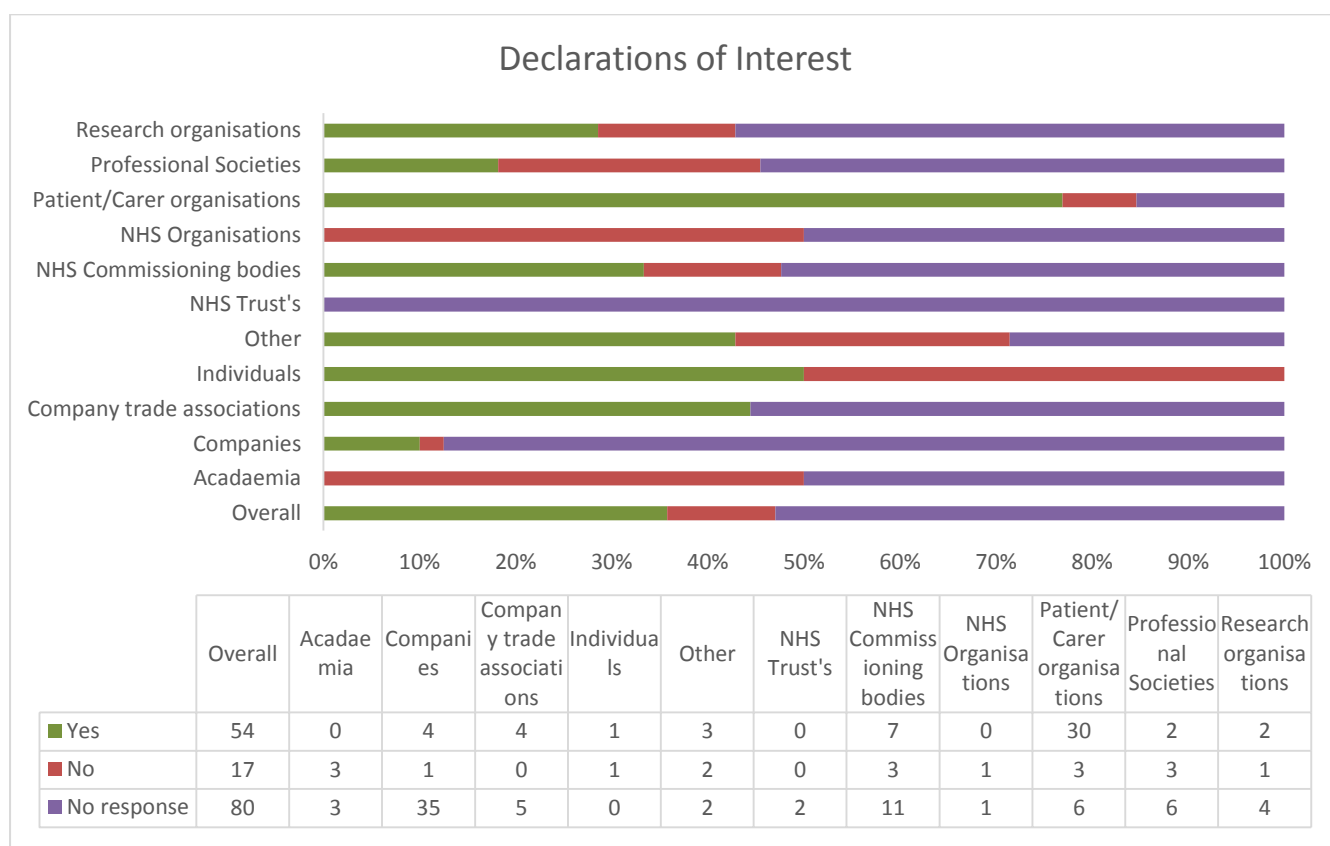
### Other organisations

- ABHI
- ABPI
- All Wales Medicines Strategy Group
- American Pharmaceutical Group
- Association of Medical Research Charities
- BioIndustry Association
- BIVDA
- Brain Tumour Research
- Cancer Research UK
- Device Access UK Ltd
- Ethical Medicines Industry Group (EMIG)
- European Confederation of Pharmaceutical Entrepreneurs
- European Medicines Group (EMG)
- Health Foundation
- Institute for Clinical and Economic Review
- King's College London
- Manchester Metropolitan University
- MAP BioPharma Limited
- Mapi Group
- Milliman - Health Actuarial Services
- The Institute of Cancer Research
- The Medical Technology Group
- The Shelford Group
- UK Medicines Information
- Universities Allied for Essential Medicines UK
- University of Edinburgh
- University of Leeds
- University of Sheffield
- Welsh Health Specialised Services Committee

## Appendix B

### Declaration of interest disclosures

Stakeholders were asked to declare whether they had received any payments, grants or other funding from the pharmaceutical industry in the last three years. Unfortunately, 53% (82) of stakeholders did not provide a response to this question.



Overall, 36% (54) of stakeholders declared they had received payments from the pharmaceutical industry in the past 3 years. Of the non-company stakeholders 46% (46) declared a payment within the last 3 years.

The stakeholder group with the highest percentage of respondents affirming that they had received such payments were patient/carer organisations, 77% (30) of whom said they had received payments from industry.