Questions to answer in the consultation –FIRST DRAFT BCA SUBMISSION

Note to members - this consultation is submitted in the form of an online survey. This is a copy of the questions.

Where a choice must be made between pre-written answers, the highlighted text indicates the response that will be chosen.

Theme 1: Alignment of the current guidance development process

General comments on theme

- **Would you like to provide general comments in relation to the proposals in this theme?**

Yes, I would like to provide general comments on this

No, I do not have general comments on this

- **If yes: Please share your general comments here:**

The Blood Cancer Alliance supports the move to simplify NICE's appraisal processes and the terminology used within them. In our 2020 report on access to medicines in blood cancer, the BCA identified that complexity within NICE's processes or within the terminology used during Technology Appraisals and Highly Specialised Technology appraisals were a factor in why patients and patient organisations sometimes found it difficult to effectively engage.

Comments on specific proposals

Would you like to add comments relating to specific proposals? If so, please select all that apply from the list below:

- I do not have any comments relating to specific proposals
- Develop a simplified singular process for all Centre for Health Technology Evaluation (CHTE) programmes (para 45)
- Align terminology used across all CHTE programmes (para 46)
- Scoping consultation length will be flexible from 5-20 days dependent on the needs of the topic (para 49)
- Scoping workshops will take place virtually (para 50)
- Scopes for simple topics will not be consulted on (para 51)
- Companies will provide a 'Summary of Information for Patients' with their evidence submission (para 59)
- Patient and carer organisations can provide written submissions to all guidance programmes (para 60)
- NICE will provide dedicated stakeholder relationship managers for patient and carer organisations (para 63)
- Committees will make recommendations on different types of guidance (TA, MTG, HST, DG) (para 67-68)

- Committee meetings will be held virtually (para 69)
- Medical technologies and diagnostics guidance can be developed without consultation (para 71)
- A shorter (less than 20 working days) consultation length can be used for some topics (para 72)
- Terminating, discontinuing and suspending guidance (para 74-76)
- The option of a multiple technology assessment for highly specialised technologies (para 78)
- The option of a multiple technology assessment for medical technologies guidance (para 79)
- Routing topics to clinical guidelines (para 81)
- Retain separate types of technology guidance for Diagnostics, Highly Specialised technologies, Medical Technologies and Technology Appraisals

How strongly do you agree or disagree that you support the proposals related to: (Options: Strongly agree, agree, neither agree nor disagree, disagree, strongly disagree, don't know/ NA.)

Please use this space to share any comments on the proposals (one box per proposal):

- Develop a simplified singular process for all Centre for Health Technology Evaluation (CHTE) programmes (para 45) - STRONGLY AGREE – ***We welcome the proposal to develop a simplified singular process in order to better help patients and their representatives engage.***
- Align terminology used across all CHTE programmes (para 46) - STRONGLY AGREE – ***In our submission to NICE's recent topics selection consultation the BCA highlighted that there can be confusion amongst patients and patient representatives surrounding the terminology used. While we strongly welcome the proposal to align terminology across programmes, and we also urge NICE to then publish definitions and explanations of common terminology for patients and their representatives.***
- Scoping consultation length will be flexible from 5-20 days dependent on the needs of the topic (para 49) - STRONGLY DISAGREE – ***The BCA believes that shortening the scoping consultation time frame has the potential to discourage or prohibit individual patients and patient representative groups and organisations from participating in TA processes. Individual patients and patient representative organisations have significant pressures on their time, and have the added complexity of being lay people and having to potentially work harder to understand associated documents, for example. A very short consultation time frame would likely leave them unable to engage in any meaningful way.***
- Scoping workshops will take place virtually (para 50) - AGREE - ***We agree that this is a positive proposal, particularly for blood cancer patients, not just because of reduced travel times, but also because immune-compromised patients will be able to attend without risk. This will be particularly reassuring in the context of COVID-19. However, care should be taken to ensure patients and their representatives who do not have the means to attend virtually, either through not owning requisite technology or lack of technical literacy, can still engage.***
- Scopes for simple topics will not be consulted on (para 51) - STRONGLY DISAGREE – ***The BCA firmly believes that in order to improve patient input into TA and HST processes, patients and their representatives must be given the opportunity to be involved at every step. Patients and their***

representatives must also be given an opportunity to advise NICE as to whether they believe a scoping workshop is required. The needs of patients and patient representatives, and their ability to effectively engage need to be equally taken into account alongside the needs of NICE, clinical groups and the company when deciding whether to hold a scoping workshop or not.

- Companies will provide a 'Summary of Information for Patients' with their evidence submission (para 59) - AGREE – ***The BCA supports this proposal. Research conducted as part of the BCA's Access to Medicine Report 2020 identified that patients, as lay people, find the technical nature of documents and discussion difficult to engage with during appraisal processes. We therefore also ask for consideration to be given as to whether lay summaries can be issues at other stages of the appraisal process to help patients and their representatives to engage. Additionally, in order to facilitate full engagement, NICE should provide lay guidance for all aspects of the appraisal, and not just those it believed to be relevant to patients and their representatives. We would welcome further proposals in this area from NICE and a separate opportunity to comment on them.***
- Patient and carer organisations can provide written submissions to all guidance programmes (para 60) - AGREE – ***The BCA supports the opportunity for patients and their representatives to engage in every step of the appraisal process.***
- NICE will provide dedicated stakeholder relationship managers for patient and carer organisations (para 63) - STRONGLY AGREE - ***We strongly support this proposal, which we believe will facilitate better patient engagement in the process. Not only will having a point of contact simplify the process by which patients and patient representatives can engage, but such managers can also be a source of information and clarity. The BCA believes these managers should be mandated to proactively distribute information and feedback to patients and their representatives, as well as respond to requests for information and updates from patient representatives.***
- Committee meetings will be held virtually (para 69) - STRONGLY AGREE - ***We agree that this is a positive change, particularly for blood cancer patients, not just because of reduced travel times, but also because immune-compromised patients will be able to attend without risk. This will be particularly reassuring in the context of COVID-19. However, care should be taken to ensure patients and their representatives who do not have the means to attend virtually, either through not owning requisite technology or lack of technical literacy, can still engage. The ability to observe committee meetings virtually should also be mandatory to allow all those who wish to engage in the process the opportunity to be as informed as possible.***
- A shorter (less than 20 working days) consultation length can be used for some topics (para 72) - STRONGLY DISAGREE – ***As discussed in relation to paragraph 49, a shortening of timelines will make is more challenging for individual patients or patient representatives to engage due to external time pressures on individuals and small organisations, and due to the need to familiarise with challenging and technical materials.***
- Terminating, discontinuing and suspending guidance (para 74-76) - AGREE – ***We would also support a more defined time frame for informing patients and patient representatives as to when a decision is taken in this area.***
- Routing topics to clinical guidelines (para 81) - DISAGREE – ***Treatments completing successful technology appraisals have a legal requirement to be funded within 90 days. We are concerned***

that if an appraisal is merged into a clinical guideline programme this mandate for funding would no longer apply. We understand that the intention with this proposal is to speed up the process. However, we will not support blood cancer treatments being subject any alternative process in which a funding mandate is not applied.

Final comments on theme

Please share any final comments on the proposals in this theme below, including any areas that have not been covered or other proposals which you think should be considered.

The BCA is clear that NICE should ensure patients and patient representatives are given the opportunity to engage in every step of the engagement process, and that they are as empowered to do this as possible, through clear information, lay guidance, simplification of terminology and a point of contact. We are pleased that these proposals clearly address some of these issues.

However, absent any form of monitoring or reporting on impact of patient engagement, it will not be possible to assess whether changes intended to strengthen the quality and frequency of patient engagement are effective. We strongly urge NICE to develop a comprehensive evidence base on their approaches to involving patients and their representatives - with a focus on the difference it makes to decisions.

The BCA would have liked to see clear proposals as to how NICE intends to report on patient engagement and, critically, its impact, in both TAs and HSTs. The BCA would urge NICE to consider a formal requirement for each TA and HST to include such a report.

The BCA would have also like to see more formal proposals for how NICE can improve its feedback to patients and their representatives throughout the appraisal process. We understand that this may be the role of the assigned Stakeholder Engagement Manager, but ask that NICE provides more clarity on the expectation for that resource, and whether introducing this resource is an opportunity to improve proactive distribution of feedback and information to patients and their representatives.

Simplification is key to facilitating better patient engagement in appraisal processes. The BCA urges NICE to ensure that when proposed changes are made, lay guidance is published alongside the often lengthy and complex methods and process guide, making clear when and how patients and their representatives can engage.

We are also disappointed that this paper does not make proposals for better facilitating patient representation during committees. Blood cancer patient representatives have reported feeling as if their views are dismissed or not fully heard during committees. It would be helpful If guidance and training for all committee members outlined the importance of the patient view and of patient evidence.

Theme 2: Opportunities for new process improvements and ways of working

General comments on theme

- **Would you like to provide general comments in relation to the proposals in this theme?**

No, I do not have general comments on this

- **If yes: Please share your general comments here:**

Comments on specific proposals

Would you like to add comments relating to specific proposals? If so, please select all that apply from the list below:

- Developing guidance on Digital Health technologies (para 24) ● Use Experts from scoping in guidance development (para 85)
- Professional, patient & carer organisations to nominate for all guidance topics (para 86)
- Use Experts nominated for related topics and guidelines (para 87)
- Working in parallel with the regulatory process (para 94-95)
- Technical engagement shall become an option in Technology Appraisals and other guidance programmes (para 102)
- The low ICER fast-track appraisal option will be removed (para 109)
- Develop a cost-comparison fast-track appraisal (para 110)
- Not using a committee to make recommendations in a fast-track appraisal (para 110)
- A simpler approach to evaluations of technologies with multiple indications (para 114)
- Managing company submissions (para 116)
- Developing guidance on combination treatments (para 118)
- Develop a process to evaluate Biosimilars (para 123)
- NHS Treatment eligibility criteria (para 126)
- Recording when NICE Scientific Advice has been sought (para 131)

How strongly do you agree or disagree that you support the proposals related to: (Options: Strongly agree, agree, neither agree nor disagree, disagree, strongly disagree, don't know/ NA.)

Please use this space to share any comments on the proposals (one box per proposal):

- Use Experts from scoping in guidance development (para 85) - STRONGLY AGREE - ***We agree that this is a positive development in some cases, but have concerns about transparency as to which experts are selected in the absence of scoping workshops.***
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- Professional, patient & carer organisations to nominate for all guidance topics (para 86) - STRONGLY AGREE - ***NO COMMENT NEEDED***
- Use Experts nominated for related topics and guidelines (para 87) - AGREE ***– While we support this proposal, we would again like to raise the need for transparency as to which experts have been chosen.***
- Working in parallel with the regulatory process (para 94-95) - Strongly Agree – ***This is also an opportunity to consider how NICE's processes can map onto the new MHRA accelerated and innovative pathways.***
- Technical engagement shall become an option in Technology Appraisals and other guidance programmes (para 102) - disagree – ***If technical engagement meetings become optional, there should be supporting guidance or advice that for blood cancers and rarer cancers it is always appropriate to include this, along with technical engagement documentation, in the TA process.***

In blood cancers, the technical engagement step has proven to be valuable, not least in addressing issues early, rather than waiting until Committee stages.

- The low ICER fast-track appraisal option will be removed (para 109) - STRONGLY AGREE ***– NO COMMENT NEEDED***
- A simpler approach to evaluations of technologies with multiple indications (para 114) - agree- ***The BCA supports this principle but requires more detail as to how a simpler approach would work.***
- Managing company submissions (para 116) - disagree – ***We are concerned about the impact of this proposal in blood cancer, and particularly in rarer blood cancers. The patient populations within rarer blood cancers are very small, and medicines that give enormous benefit in terms of health outcomes and quality of life can be incredibly expensive to manufacture. In some cases, commercial agreements can take longer, although it is common that agreement on price is finally reached. We believe delaying the appraisal process until pricing agreement is reach has the potential to inhibit timely access to vital new and effective treatments for blood cancer patients. HST has proven not to be an alternative for these kinds of treatments. HST has to date not considered any blood cancer treatments. If this measure is applied, we are concerned that some innovative blood cancer treatments will fall between the two stools of HST and TA, due to cost issues alone, and blood cancer patients will be disadvantaged.***
- Developing guidance on combination treatments (para 118) - agree – ***We agree with the principle of developing better guidance on combination treatments but would require more detail to enable further comment.***
- Develop a process to evaluate Biosimilars (para 123) - Strongly agree - ***No comment needed***
- NHS Treatment eligibility criteria (para 126) - agree – *This is a welcome step to improve transparency, as it will increase the ability of patients and their representatives to understand how treatments will be made available and to which patient cohorts, and to make representations in this area.*

Additional questions

What changes can we make to our processes to help reduce health inequalities in the way we develop our guidance, stakeholders participate and how health inequalities are identified and considered in making recommendations?

Final comments on theme

Please share any final comments on the proposals in this theme below, including any areas that have not been covered or other proposals which you think should be considered.

Theme 3: Opportunities for new process improvements and ways of working

General comments on theme

- **Would you like to provide general comments in relation to the proposals in this theme?**

Yes, I would like to provide general comments on this

No, I do not have general comments on this

- **If yes: Please share your general comments here:**

The BCA believes stronger patient and patient representative voice is needed within the managed access proposals. Again, we urge NICE to formulate a comprehensive evidence base on its approach to involving patients and their representatives across all aspects of appraisal processes - with a focus on the difference it makes to decisions. We urge NICE to recognise that patients and patient representative groups have important expertise in non-RCT and real-world data.

Comments on specific proposals

Would you like to add comments relating to specific proposals? If so, please select all that apply from the list below:

- Commercial proposals and managed access proposals (para 140-145)
- The Budget Impact test (para 147)
- The status of a recommendation for managed access (para 150)
- Managed access entry (para 157-159)
- Data collection agreement development and oversight (para 164-166)
- Managed access exit (para 169)

How strongly do you agree or disagree that you support the proposals related to: (Options: Strongly agree, agree, neither agree nor disagree, disagree, strongly disagree, don't know/ NA.)

Please use this space to share any comments on the proposals (one box per proposal):

- Commercial proposals and managed access proposals (para 140-145) - neither agree or disagree - ***If companies are encouraged to consider managed access earlier, it must still remain an option later should issues arise during the process. Any information about uncertainties should be shared with all stakeholders for discussion.***
- The status of a recommendation for managed access (para 150) - agree – ***NO COMMENT NEEDED***
- Managed access entry (para 157-159) - AGREE - ***The CDF has proved to be vital for blood cancer patients to access new and innovative treatments, given the small patient numbers often involved. We support the proposal to route treatments into managed access schemes without the need for full HTE, and would welcome more information about entry requirements to the CDF.***
- Data collection agreement development and oversight (para 164-166) - Agree – ***The BCA's Access to Treatment report 2020 identified that often when treatments are made available via CDF, data to address the uncertainties that prevented a full TA is not being collected. We therefore welcome the indication of improvement in data collection for treatments in managed access schemes and strongly support better guidance and data collection oversight. We also believe tha*t *patients and patient representatives should have the opportunity to be involved in the development of managed access agreements, as well as being included in oversight groups***.

- Managed access exit (para 169) - – ***We support assessment of the aspects of treatments that were uncertain and caused them to go via a managed access route. We do not support reassessment of things previously agreed.***
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- Final comments on theme

Please share any final comments on the proposals in this theme below, including any areas that have not been covered or other proposals which you think should be considered.

Theme 4: Opportunities for new process improvements and ways of working

General comments on theme

- **Would you like to provide general comments in relation to the proposals in this theme?**

No, I do not have general comments on this

- **If yes: Please share your general comments here:**

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Comments on specific proposals

Would you like to add comments relating to specific proposals? If so, please select all that apply from the list below:

- The vision of the highly specialised technologies programme (para 173-176)
- The key principles for the highly specialised technologies programme (para 186-190)
- The criteria for excluding technologies from HST topic selection (para 186-190)

How strongly do you agree or disagree that you support the proposals related to: (Options: Strongly agree, agree, neither agree nor disagree, disagree, strongly disagree, don't know/ NA.)

Please use this space to share any comments on the proposals (one box per proposal):

- The vision of the highly specialised technologies programme (para 173-176) - DISAGREE – ***The issue within blood cancer is that there are many rare types, with incredibly small patient numbers. While this vision for HST would cover treatments for ultra-rare conditions, it is unlikely that any blood cancer innovation would be considered under HST. No blood cancer treatment has been considered under HST to date despite rarity. However, STA is, in many cases, incredibly challenging for blood cancer treatments as achieving patient numbers for full clinical trials is not possible, due to rarity. Many innovations for blood cancers are therefore stuck in a no man's land between HST and STA. If NICE is unwilling to consider rarity modifier, as we have called for in our response to the Methods Review consultation, a new appraisal process should be develop that sits between HST and STA, so that blood cancer patients are not disadvantaged by having a rare condition, but not so rare as to qualify for HST.***

- The key principles for the highly specialised technologies programme (para 186-190) - DISAGREE – ***IN the absence of a rarity modifier in STA or a new appraisal process for rarer conditions, we do not support key principles for HST that are as restrictive as currently proposed.***
- The criteria for excluding technologies from HST topic selection (para 186-190 - DISAGREE - ***The criteria are too restrictive to include blood cancers that have limited chance of fulfilling the STA process. We also support a stronger emphasis on quality of life, which is often incredibly challenging for patients with blood cancers.***

Additional questions

Are there any areas where the vision does not address the needs of ultra-rare diseases?

Final comments on theme

Please share any final comments on the proposals in this theme below, including any areas that have not been covered or other proposals which you think should be considered.

Both the Methods Review proposals and these proposals do not leave us confident that the challenges of rare, but not ultra-rare, conditions will be considered, let alone addressed. We strongly urge NICE to consider how it will meet this challenge for the future, and ensure blood cancer treatments, and indeed blood cancer patients, have a fair chance in appraisal processes.

Final comments on consultation

Please share any final comments on the consultation here:

For some time now, the BCA and the blood cancer patients we represent have felt that the patient voice is lost in the process for securing access to innovative new medicines and treatments that can improve outcomes and quality of life dramatically for them.

NICE's process should reflect the importance of patient representation, and address the challenges that patients often encounter when trying to engage with appraisals. While many proposals contained within this review are welcome, there is still scope to go further. This includes addressing the specific challenges of rarer blood cancers within the appraisal process; increasing transparency and accessibility for patients so that they understand when and how to engage; recognising patient groups as valuable and important data sources; and formalised measuring and reporting of patient impact within appraisal processes.

Profile

To enable us to confirm safe receipt of your comments, and follow up for clarification if necessary please confirm your:

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