Response to the NICE Review Consultation – FINAL

Closing Date: 13th October 2021

Q1: Please select the consultation(s) that you would like to comment on below:

X Methods (including questions on valuing the benefits of health technologies and understanding and improving the evidence base)

X Processes (including questions on alignment, new ways of working and Commercial and Managed Access)

X Topic selection (including questions on highly specialised technologies routing criteria and the eligibility criteria for devices, diagnostics and digital)

Methods

This section of the form focuses on the Methods consultation document. In particular, we would like to understand more about your perspectives on core themes relating to valuing the benefits of health technologies and understanding and improving the evidence base.

Please read and review the consultation document (Proposals a to o and appendix paras 1.1 to 1.52) then answer the questions below and add any comments you have on the themes on the next page.

Q1: How strongly do you agree or disagree with the proposals related to:

	Strongly agree	Agree	Neither	Disagree	Strongly disagree	N/a or don't know
A modifier for severity of disease					X	
Consideration of uncertainty within decision-making			x			
Health inequalities			x			
Aligning modifiers across programmes			x			
Discounting					X	

Q1a: A modifier for severity of disease

Two alternative options for a modifier for severity of disease are presented in proposal g and h.

Of the 2 alternative options presented in proposal g and h (and appendix 1 paragraphs 1.18 and 1.19), which do you prefer?

Option 1
Option 2

Please use this space to share any comments on the options:

- ***While Option 2 is the preferable of the two if this must be introduced as currently proposed, the BCA has very significant concerns about the impact of cost-neutrality on these proposals. As it stands, it appears that this will see those with the most severe diseases see their access to the end-of-life modifier limited.***

Q2: Comments on each proposal:

A modifier for severity of disease:

- ***While we welcome the principle of the introduction of a severity modifier, and appreciate it may mean more patients benefit, we have significant concerns about the implications of cost-neutrality on access of treatment for those patients who have previously benefitted from the end-of-life modifier.***
- ***A significant number of blood cancer patients have benefited from the end-of-life modifier, with those in need of life-extending new treatments able to access the modifier increasing the ICER threshold.***
- ***Our concern is that the requirement for cost-neutrality in the new severity modifier will mean this route to accessing new life-extending treatments will no longer be available. We do not understand the basis for the cost-neutrality requirement.***
- ***Urgent clarification is needed on this proposal that ensures patients who would previously have benefitted from the end-of-life modifier are not affected by the introduction of the severity modifier.***
- ***In our previous consultation submissions, the BCA called for a rarity modifier. We are incredibly disappointed this has not been included, or indeed the issues surrounding the requirement for this fully addressed. We do not believe the severity modifier will do anything to address the issue of treatments that are not eligible for HST, but with patient numbers so small that there is insufficient data to address uncertainty in an STA process.***

Consideration of uncertainty within decision-making:

- ***Uncertainty is unavoidable in complex treatment pathways, such as those found with many blood cancer treatments. We only expect the issues of uncertainty to become more prevalent as more treatments come down the line in years to come. We do not feel confident that the measures proposed by NICE go far enough to meet potential present and future need for consideration of uncertainty.***
- ***The BCA feels there in insufficient detail as to how consideration of uncertainty will be approached to negate the need for a rarity modifier. Recommendations in this area feel like a consolidation of the status quo, rather than a means of securing necessary change to improve access to treatment for patients with rarer diseases including blood cancers.***
- ***While we welcome the inclusion of rarity and small patient populations, as well as innovative and complex technologies, in the list of reasons for greater flexibility – and agree it could ensure more equitable access for patients with***

rarer blood cancers - in the absence of a framework or any detail as to how this will work in practice, it is hard to analyse whether it will be effective in addressing uncertainties.

- ***In particular, we would like to see information as to how NICE will ensure all committees apply this principle equitably. Indeed, the BCA would also like to see transparency of reporting in terms of how decisions are made on whether greater flexibility has been applied to take account of rarity and small patient populations. IN the absence of this framework for committees, and transparency of decision-making, it could be the case that different committees apply the principle in different ways.***

Health inequalities:

- ***From a patient perspective, it is disappointing that this has not been prioritised. Additionally, we are disappointed that there is no comment on how current efforts to combat health inequalities are, and will be, monitored for effectiveness***

Aligning modifiers across programmes:

Discounting

- ***It is disappointing to see that the discount change has not been recommended, despite widespread support from patient groups and the pharmaceutical sector. Further, best available evidence supports a change to the discount rate to 1.5%.***
- ***We would like to stress our concern that the current discounting may not appropriately value treatments that confer long term benefit.***
- ***This could disadvantage many cell and gene therapies, such as CAR-T cell therapy, making it harder for NICE to approve innovative new treatment options that are recommended, despite their potential to reduce disease burden, ongoing treatment for chronic conditions and provide treatment where currently there are no other options.***

Q3: Understanding the evidence base

We would like to understand more about your perspectives on some core themes relating to understanding and improving the evidence base.

Please read and review the consultation document (proposal P, appendix 1 paragraphs 2.1 to 2.16) then answer the questions below and add any comments you have on the themes on the next page.
How strongly do you agree or disagree that you support the proposals related to:

	Strongly agree	Agree	Neither	Disagree	Strongly disagree	N/a or don't know

Implementing the proposed cases for change for sourcing, synthesising and presenting evidence, and considering health-related quality of life				X		
Considering real-world evidence				X		
Calculating the costs of introducing health technologies			X			
Analysing uncertainty			X			

Q4: Comments on each proposal

Implementing the proposed cases for change for sourcing, synthesising and presenting evidence, and considering health-related quality of life:

- ***We are disappointed that the BCA's comments on issues relating to data collection from managed access agreements including the Cancer Drugs Fund have not been addressed.***
- ***We would like to re-iterate the need for stronger guidance as to how, under the CDF, NICE and NHSE should establish a link between the main clinical uncertainties identified at the time of the first appraisal by NICE and the clinical data that is generated during the time that a treatment is within the CDF. This will ensure that while available under CDF, the right data is being collected to ensure treatments can move through the STA process when appropriate.***

Considering real-world evidence:

- ***The BCA is please that there is further clarification about the role of real-world evidence in the appraisal process.***
- ***We would, however, urge NICE to consider including within the manual guidance as to how patient group submissions of qualitative real-world evidence should be considered. This must include qualitative real-world evidence submitted directly by patient representative organisations into the process. This would promote the recognition of patients and their representative organisations as an important, and to date, overlooked, source of qualitative evidence.***
- ***The decision-making framework must include qualitative evidence provided by a patient group, alongside any quantitative evidence.***
- ***Lastly, we call for transparent reporting as to how qualitative, real-world evidence provided by patient representative organisations has been used in decision-making, including the impact it had.***

Calculating the costs of introducing health technologies:

Analysing uncertainty:

Q5: Methods additional comments

We understand you may have comments relating to the methods consultation document that have not been covered in our questions about valuing the benefits of health technologies or understanding and improving the evidence base.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others.

5a: Please share any comments on whether the proposed methods will help to achieve this aim, or if the proposals raise any concerns with regard to equality:

5b: Please share any other comments:

- ***As indicated, it is our position that the changes proposed to STA process methods do not appear to fully address the challenges of appraising treatments for rarer blood cancers, that do not qualify for HST. No blood cancer treatment has qualified for HST in the past, yet the small patient populations have proved a barrier to the STA process for blood cancer treatments. We are not confident the changes proposed will address this problem.***
- ***It is not clear how the including rarity as a condition for greater flexibility will be applied fairly across all appraisals in practice. We would like to see greater clarity and stronger proposals in this area.***
- ***We welcome the proposals relating to treatments that are not cost-effective at zero price. Again, we would like to see greater clarity as to how this will work in practice, however, given the scale of the challenge for some blood cancer treatments.***
- ***We are disappointed to see that the discussed discount amendments have not been introduced. This would align the processes of NICE with the most up to date evidence and current Treasury Green Book recommendations.***
- ***Treatments with long-term benefits, such as cell and gene therapies, have the potential to be significant treatment options for patients in the future. The current system is not appropriately prepared innovations in this area and does not address many of the challenges that cell and gene therapies currently face. Further considerations must be made to ensure the futureproofing of the work of this consultation.***
- ***We continue to be concerned about the inclusion of the proposal for sub-groups. We believe this proposal may have serious implication for equality, and also risk impacting those with the severest, or who may live longer. We believe the proposal to be wholly at odds with the intention of this review, and indeed at odds with the very principle of universal access to healthcare.***
- ***In general, the BCA considers this review to be a missed opportunity, with little changing in practice, and little ambition for the future set out.***

- ***We would have liked to see a more ambitious set of proposals for change that would make significant progress in addressing issues within appraisal processes that impede timely access to new and effective treatments for blood cancer patients – but also proposals that 'future-proof' NICE's processes.***

5c: You can attach a document here:

Processes

This section of the form focuses on the Processes consultation document.

In particular we would like to understand more about your perspectives on alignment, new ways of working and Commercial and Managed Access.

Q1: Alignment
We would like to understand more about your perspectives on the alignment of processes within the consultation document.

Please read and review the consultation document then answer the questions below and add any comments you have.

1a: Have the processes been aligned appropriately?
Yes
No

Please use this space to share any comments:

1b: Are there any remaining unwarranted differences in the processes of guidance development for Diagnostic Assessment, Highly Specialised Technologies, Medical Technology Evaluation and Technology Appraisal?
Yes
No

Please use this space to share any comments:

Q2: New ways of working
We would like to understand more about your perspectives on some core themes relating to new ways of working.

Please read and review the consultation document then answer the questions below and add any comments you have on the themes on the next page.

	Strongly agree	Agree	Neither	Disagree	Strongly disagree	N/a or don't know
Technical Engagement					X	

Rapid review of guidance for biosimilars			X			
Treatment eligibility criteria			X			
Managing high company base case ICERs				X		
Alternative draft scope consultation timings				X		

Q3: Comments on each proposal
Please use this space to share any comments on the proposals on new ways of working:

Technical Engagement:

- ***The BCA is extremely disappointed by the absence of proposals to ensure patients and their representative organisation feel more able to engage in a meaningful way in the technical engagement process.***

- ***Layman's guidance and training, information in simplified language, and other resources should be made available.***

- ***As the BCA has previously suggested, we would also like to see the process include a meeting similar to PACE, in which patients and their representative organisations can discuss areas of concern and debate. We are disappointed this has not been considered.***

Rapid review of guidance for biosimilars:

Treatment eligibility criteria:

Managing high company base case ICERs

- ***It is a concern that this proposal has remained and could allow appraisals to be terminated without proper stakeholder consultation.***

Alternative draft scope consultation timings

- ***The BCA welcomes the decision not to change the timings in this area. It is imperative to recognise that patients and their representative organisations have limited resources and may require more time to ensure meaningful engagement in the process.***
- ***We do not support scoping timelines cuts that are proposed indications for which there has been an appraisal in the last 12 months. As discussed,***

sufficient time must be given for patients and their representative organisations to engage, in the context our limited resources.

- ***Full and proper consultation with patients and their representative organisations can be a time-consuming but necessary stage in appraisal. Any additional restrictions on timing risks jeopardising meaningful patient engagement and involvement.***

Q4: Commercial and Managed Access

We would like to understand more about your perspectives on the proposals relating to Commercial and Managed Access.

Q4a: Please read and review the consultation document then answer the questions below about how clear or unclear you find them and add any comments.

	Very clear	Clear	Neither	Unclear	Very unclear	N/a or don't know
Commercial activity			X			
Managed access activity		X				

Q4b: Please use this space to share any comments on the proposals:

Commercial activity: no comments

Managed access activity:

- ***The BCA still believes stronger patient and patient representative voice is needed within the managed access proposals – this will not be achieved in the proposals as they stand.***

Q5: Additional comments

We understand you may have comments relating to the processes consultation document that have not been covered in our questions about alignment, new ways of working or Commercial and Managed Access.

Please use the space below to share any equality considerations, or wider thoughts or comments.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others.

Q5a: Please share any comments on whether the proposed processes will help to achieve this aim, or if the proposals raise any concerns with regard to equality:

Q5b: Please share any other comments:

- ***The BCA is very disappointed that NICE has not chosen to put improving patient engagement in appraisal processes at the heart of this review. When BCA members consult with patients, it is the primary issue they raise when discussing the need for change in NICE processes.***
- ***The BCA has been 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. It is very disheartening not to see stronger proposals within the consultation documents as to how this can be better achieved. Patients' report feeling as if they have a minor role within NICE appraisals and this review was a clear opportunity to address that problem - an opportunity which has been missed.***
- ***We would also again like to raise that absent any form of monitoring or reporting on impact of patient engagement, it will not be possible to assess whether any 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.***

Q5c: You can attach a document here:

Presentation of the guidance manual

The draft guidance manual brings together the proposals from the methods and processes consultation documents as an illustrative example of the proposals put into practice.

Q1: The manuals

Please read and review "Developing NICE technology guidance: The draft manual" and share your thoughts below.

1a: What are your initial impressions of how the guidance manual is presented?

- ***We appreciate the need for the manual to be a formal, lengthy and technical document to ensure NICE's appraisal processes are properly administered. However, the BCA would also strongly urge NICE to create lay guidance for patients and their representative organisations, to allow them to understand the process fully.***

- ***We also believe NICE should offer a programme of training for patients and their representative organisations on appraisal processes, how to engage and what kinds of evidence and information it is valuable for patients to share.***

- ***We are disappointed that the manual will not include guidance on how to engage patients for committees, to ensure they treat patients and their representatives as equals, with valuable insight, during the process. As we have previously raised, patients report that they do not feel their voices are heard in committee stages and we do not believe this will change after this review.***

Q1b: If you have any comments on the chapters in the guidance manual please provide these here: (otherwise please click next)

Involvement & participation

The scope

- ***The manual does not make clear what kinds of evidence patient groups might submit in the category of real-world evidence. Clarification is needed in this area in the form of guidance.***

Developing the guidance

- ***As discussed, the BCA has called for better reporting of patient engagement in appraisal processes, both in terms of how patients and their representatives were engaged, but also the impact that engagement has on decision-making. Absent this, it is hard to see how patients will feel adequately represented in the process.***

Guidance surveillance

Topic selection

This section of the form focuses on the Topic Selection consultation document.

In particular, we would like to understand more about your perspectives on how clear certain elements are.

Q1: Highly Specialised Technologies

We would like to understand more about your perspectives on the HST evaluation programme.

Please read and review the consultation document and draft topic selection manual, page 10 - section 6, then answer the questions below and add any comments you have.

Q1a: How clear or unclear is the aim of the HST evaluation programme?

Very clear
Clear
Neutral
Not very clear
Not clear at all
Don't know / NA

Q1b: Please use this space to share any comments on the proposals on the aim of the HST evaluation programme:

- ***The BCA called for greater clarity on HST criteria in previous consultation submissions and welcome that this has been made available.***

Q2: Highly Specialised Technologies (HST) routing criteria

We would like to understand more about your perspectives on the routing criteria.

Please read and review the consultation document and draft topic selection manual, page 10 – section 6, then answer the questions below and add any comments you have.

Q2a: How clear or unclear is the refined routing criteria for HST?

Very clear
Clear
Neutral
Not very clear
Not clear at all
Don't know / NA

Q2b: Please use this space to share any comments on the proposals on the routing criteria for HST:

- ***We are concerned about the impact of the routing criteria on the eligibility for treatments to utilise the HST process. While we support the clearer definition of the rarity threshold for HST, these represent more barriers to access to the HST process – with little by way of justification of that decision. We believe this risks disincentivising rare illness drug development in the UK.***
- ***An example we would point to in blood cancer is Midostaurin for Advanced Systemic Mastocytosis (ASM). The ASM patient population is small enough for this treatment to qualify for HST. However, other criteria mean it cannot be considered under the HST process – including the requirement for the whole patient population using a drug be very small. Midostaurin was first approved for use in Acute Myeloid Leukamia. Therefore, the whole patient population is too large for it to go via the HST route for ASM patients, despite the fact that the size of the ASM patient population points to HST being the most appropriate route.***
- ***We would like to understand the justification for choosing the cut off points identified, and request further information.***

Q3: Topic selection: eligibility criteria
We would like to understand more about your perspectives on the eligibility criteria.

Please read and review the consultation document and draft topic selection manual, page 3 – section 4 then answer the questions below and add any comments you have.

Q3a: How clear or unclear is the eligibility criteria (section 4) for devices, diagnostics and digital technologies?

Very clear
Clear
Neutral
Not very clear
Not clear at all

Don't know / NA

Q3b: Please use this space to share any comments on the proposals on the eligibility criteria:

Q4: Additional comments

We understand you may have comments relating to the topic selection consultation document (and manual) that have not been covered in our questions about highly specialised technologies or the eligibility criteria for devices, diagnostics and digital.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others.

Q4a: Please share any comments on whether the proposals for Topic Selection will help to achieve this aim, or if the proposals raise any concerns with regard to equality:

- ***The believes NICE decisions in this area should be subject to a formal appeal process.***
- ***Patient and their representative organisations should be notified of decisions proactively and at an early stage to give a chance to challenge, and a formal process provided to do so.***

Q4b: Please share any other comments:

Q4c: You can upload a document here: