Cancer Research UK (CRUK) is pleased to have the opportunity to respond to this consultation. CRUK is the world’s largest independent cancer charity dedicated to saving lives through research. We support research into all aspects of cancer and this is achieved through the work of over 4,000 scientists, doctors and nurses. We want to accelerate progress so that three in four people survive their cancer for 10 years or more by 2034.

We believe that all patients should have access to the best, evidence-based treatments for their condition. NICE, through its technology appraisals (TA) programme, has a critical role in supporting patient access to cancer drugs, which can improve patient survival, allow people to return to work, look after their families, and lead more active and fulfilling lives.
In our submission to the first phase of this consultation on proposed changes to the technology appraisals programme,¹ we stated our support for NICE’s ambitions to increase the programme’s capacity, and to streamline its operation. We are pleased that NICE has been able to take forward many of the first phase proposals.

We also welcome the changes to the proposed amendments to patient and clinical expert attendance at TA Committee meetings since the first phase. The “opt-out” model now proposed is more appropriate since it retains the opportunity for patient experts to outline their perspective and insights to the committee members in person. We noted the value of this face-to-face interaction in our first phase submission.

CRUK urges NICE to continue considering how to increase its technology appraisal capacity, and its procedures for assessing technologies effective in multiple indications. This reflects the call in the Accelerated Access Review (AAR) for NICE to “review its methods and processes to ensure they are fit for the future”. ii

Globally, it is estimated the oncology pipeline contains over 600 potential therapies in late stage development. iii We therefore welcome NICE’s ambition to increase the capacity of its TA programme by 20-25 appraisals per year. This offers NICE the chance to produce guidance on more of these technologies earlier, accelerating patient access.

However, NICE must consider further how it will respond to the personalised medicine agenda, and treatments effective in multiple indications. More than 50% of major cancer medicines marketed in 2014 were for multiple indications; by 2020 this is expected to reach 75%. iv As noted in the first phase consultation document, v in many cases companies are seeking marketing authorisations for their new therapies in ten or more different indications.

Such treatments could rapidly consume the extra appraisal capacity NICE believes present reforms will release, coming to dominate the TA programme and ‘crowding out’ appraisals of other technologies. This would mean further delays in patient access to certain new and innovative medicines. NICE, in partnership with additional stakeholders including NHS England, should develop a new approach to assessing individual medicines which are licensed for multiple indications.

NICE should clarify how their intention to issue FADs rather than draft guidance where possible will impact on interim funding arrangements for cancer drugs.

Under the July 2016 reforms to cancer drugs access in England, interim funding can be made available (post-marketing authorisation) when NICE issues positive draft guidance for a drug’s use in routine commissioning or the Cancer Drugs
Fund (CDF). This can accelerate patient access to the drug. The updated TA process however envisages that a FAD will be produced as the default outcome of the first committee meeting on a topic, omitting the production of draft guidance.

While a positive FAD can also unlock interim funding, this change has the potential to result in delays to patient access to cancer drugs. This will logically be the case if a FAD is produced at a later stage of the (updated) TA process than would have been the case for draft guidance under the present process, delaying provision of interim funding.

NICE should clarify whether they believe this is a possibility. If so, NICE must ensure that, where a FAD is issued on a topic without prior draft guidance under the updated TA process, the FAD is issued no later in the TA pathway than would have been the case for draft guidance under the present process. This should include the release of FADs prior to a technology receiving its marketing authorisation where, under the present process, draft guidance would have been released at the same stage.

Alternatively, if NICE do not feel this ambition is realistic, they should explore options for a mechanism which does not absorb appraisal capacity, but which is still able to trigger interim funding (and so unlock earlier patient access to the drug) if the technology is likely to receive a positive recommendation in final guidance.

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<td>NICE must ensure their focus on improved engagement with companies to drive a more streamlined TA process does not lead to a loss of transparency.</td>
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<td>The reforms set out in the first phase of this consultation (including issuing earlier invitations to participate, and the introduction of a technical engagement step) are a welcome move towards the ambition outlined in the AAR, for NICE to develop a “flexible health technology appraisal pathway that can be tailored to a product’s value proposition”. vi We welcome NICE’s acknowledgement of its vital role in the accelerated access and innovation agenda.</td>
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<td>However, the shift away from discussions in the public setting of the committee meeting, towards earlier and more substantial engagement between NICE and companies, must not make the appraisal process less transparent. We raised this concern in our response to the consultation’s first phase.</td>
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<td>We therefore welcome the proposal to send the technical report produced before the appraisal committee meeting to consultees and commentators. The content of this report must be as fully representative of the discussion between the company and the technical team up to the point of the report’s release as possible. Where there is further engagement between the company and NICE once the report has been released, details of this engagement should also be released to consultees and commentators ahead of the committee meeting.</td>
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CRUK encourages NICE to work with relevant stakeholders, including NHS England, patient organisations, and companies, to ensure its methods and processes are adaptable to a range of commercial and access arrangements.

We were supportive of the 2016 changes to the CDF, giving NICE the option of recommending a technology for inclusion in the CDF on a managed access basis. We welcome emerging evidence that these changes are helping accelerate patient access to innovative medicines.\textsuperscript{vii}

This managed access option will continue to be valuable in the future given emerging trends which may result in increasing use of less mature evidence in technology appraisals. These developments include earlier licensing decisions by regulators, and the dominance of targeted treatments in the oncology pipeline.

Increased use of flexible commercial arrangements, such as outcomes-based pricing, represents a potentially valuable way to manage the uncertainty produced by these trends at a systemic level. They can help the NHS to manage the financial impact of new technologies while continuing to secure patient access to innovative medicines.

We hope NHS England’s use of outcomes-based pricing arrangements for drugs to treat diseases including Hepatitis C and Multiple Sclerosis signals their interest in using such arrangements for future cancer drugs.\textsuperscript{viii} CRUK (in partnership with Greater Manchester Health and Social Care Partnership) is currently undertaking a research project exploring the feasibility of an outcomes-based payments scheme in the NHS, in order to generate new evidence on the potential benefits and operational principles of such a scheme.\textsuperscript{ix}

NICE must ensure it is prepared for this possible shift. Its processes and methods must allow it to effectively assess the clinical and cost implications of new arrangements of this kind as well as more traditional pricing and access agreements. This will also have to be combined with broader changes elsewhere in the healthcare system on issues such as data collection and oversight, which are equally important in facilitating such novel arrangements.

This ambition reflects the vision in the Life Sciences Industrial Strategy for the appraisal of medicines to be able to accommodate “a range of flexible funding and reimbursement vehicles”.\textsuperscript{x} It is also in line with the call in the AAR for NICE to implement “changes that support NHS England in agreeing commercial arrangements for strategically important products”. It is important that this support extends to facilitating the adoption of innovative and flexible arrangements of this kind through the TA programme.
| 2.4.26 | 2.4.27 | CRUK wishes to see more appraisals being concluded as close to the point of licensing as possible, to minimise delays for patients. In particular, closer alignment between the regulatory and TA processes should be harnessed to improve join-up between the Early Access to Medicines Scheme (EAMS) and NICE’s TA programme.

Drugs in EAMS have been independently identified as having high potential to effectively address unmet clinical need, and many EAMS medicines have been for oncology indications. It is therefore important to CRUK that medicines can cross over from EAMS to commissioning in line with NICE guidance as quickly and as seamlessly as possible. This will accelerate the transition of successful EAMS drugs to a wider patient cohort, as well as minimising potential breaks in patient access to these drugs between EAMS expiry and NICE issuing positive guidance.

We acknowledge that NICE approval can already happen more quickly for medicines formerly available under EAMS. But delays remain too common. For example, venetoclax for treating CLL was available under EAMS from August 2016 until December 2016 (when it received marketing authorisation), but NICE did not issue draft guidance on its use until February 2017, and it was not granted a positive recommendation (for use in the CDF) until October 2017.

The more than two-month wait between licensing and draft guidance in this case is close to NICE’s standard 90-day target for guidance on cancer products after marketing authorisation. This suggests that NICE could do more to prioritise the accelerated appraisal of EAMS medicines.

We note the independent review of EAMS carried out in 2016 identified that earlier HTA of EAMS-approved products “will help to shorten the patient access gap which currently exists between MA and adoption in the NHS”. We echo its recommendation that NICE work with pharmaceutical companies to “continue to consider how they can accelerate the review of EAMS-approved products.”

In particular, NICE should consider how to ensure the option of an EAMS meeting between companies, NICE’s Office for Market Access, and NHS England, is taken up by all companies with an EAMS-approved product. This opportunity for discussion of issues such as possible data collection arrangements, and potential barriers to market access post-EAMS, represents a valuable forum. This meeting currently incurs a fee-for-service charge. NICE should explore options with NHS England and MHRA to remove the fee associated with this meeting, which could act as a potential disincentive to companies.

| 3.5.13 | 3.4.12 | CRUK believes NICE should offer more clarity on how patient input is captured and used in the TA process, and its impact on decision-making. This will allow NICE to ensure it is providing patients with a meaningful role in decisions that affect them, and that the issues most important and urgent to patients are fully considered at every stage of the process. |
We welcome NICE’s aspiration for earlier and deeper engagement with patients, achieved through means such as the inclusion of patient expert evidence as part of the technical report. The amendments to the proposed changes to patient and clinical expert attendance at TA Committee meetings, since the consultation’s first phase, are also welcome. These steps will help to ensure the unique patient perspective, including the value of patient-centred outcomes which might otherwise be omitted from NICE’s analysis, is properly reflected in the TA process.

We welcome the creation of the “lay lead” role to focus on patient and carer evidence on the new NICE technical teams. This is a sensible and valuable way to ensure people affected by cancer have a voice in the process. Giving a platform to the patient perspective from the point of the topic’s introduction in this way is fundamental to the kind of meaningful patient involvement referenced above.

The creation of this role should also be seen by NICE as an opportunity to more precisely follow and capture how patient input is used in the TA process. This will allow NICE to make further and informed changes to realise its ambition for earlier and deeper patient engagement, as well as helping to promote patient and public confidence in NICE and its decision-making procedures.

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<td>PLEASE NOTE: The Institute reserves the right to summarise and edit comments received during consultations, or not to publish them at all, where in the reasonable opinion of the Institute, the comments are voluminous, publication would be unlawful or publication would be otherwise inappropriate.</td>
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vi Bell J. et al, *op. cit.*


