Cancer Research UK response to the Cancer Drugs Fund reform consultation

February 2016

Cancer Research UK (CR-UK) welcomes the opportunity to respond to this consultation. All cancer patients should be able to access the best, evidence-based treatments available for their condition. The Cancer Drugs Fund has given thousands of patient's treatment that they would not otherwise have had, but it's been clear for some time that it is not sustainable. The Cancer Strategy[1] published last year, and the National Audit Office report[2], set out the need for reform of the system. It is absolutely crucial that data collection is a requirement in the new system to inform decision making. The system of cancer drugs approvals must take the unique context of cancer treatment into account. This means that the continuation of a reformed national fund for cancer, working alongside NICE, is appropriate in the medium term.

The proposals are an important step forward, and may create a more effective system than the current CDF, but to further develop the proposals, we would like to see:

- NICE to take a flexible approach to assessment going forward, particularly on the approach to further data collection following conditional decisions.
- NICE should engage with colleagues in the devolved nations more closely in this process and clarify how the changes will impact patients in Wales and Northern Ireland, where there are no specific funds to manage conditional access.
- NHS England should ensure greater alignment of these proposals with the national cancer research agenda.
- NICE and NHS England should provide further detail on what the proposals will mean for drugs going through the Early Access to Medicines Scheme (EAMS) and drugs receiving the EMA's planned PRIME designation.
- Government should provide reassurance that there will be sufficient resource given to NICE to meet increased demands.
- NICE should be transparent in stating the rationale for decisions to provide conditional access or not, and considerations made around data requirements.
- NHS England should clarify funding requirements after patient numbers for data collection are met, to ensure that no patient would miss out on appropriate treatment.
- NICE should be more flexible on the evidence requirements for indications in rarer cancers to make the process fair for them.
- NICE should be more flexible on timelines needed to assess a drug further to a "Recommended for use within the CDF" decision, and data requirements for each drug should be context-dependent.
- The Patient Access Scheme Liaison Unit (PASLU) should be more open to innovative pricing schemes in future to increase the scope for making new drugs cost-effective for the NHS.

[1] http://www.cancerresearchuk.org/sites/default/files/achieving_world-class_cancer_outcomes_-_a_strategy_for_england_2015-2020.pdf

[2] https://www.nao.org.uk/wp-content/uploads/2015/09/Investigation-into-the-Cancer-Drugs-Fund1.pdf

We view the current proposals as a welcome element of a longer term solution. A managed access approach is likely to be a key part of the future system of drug approvals. However, we would also encourage the Government to work with NICE to consider its cost-effectiveness thresholds as part of a wider assessment for a longer term solution for access to cancer drugs.

This response represents CR-UK's position; however we consulted with nearly 40 cancer patients through a focus group and online survey to help develop this response. We also consulted with cancer clinicians through the National Cancer Research Institute Clinical Studies Groups,[3] and the Experimental Cancer Medicines Centres.[4]

1. **Do you agree with the proposal that the CDF should become a 'managed access' fund for new cancer drugs, with clear entry and exit criteria?**

Agree

We agree in principle with the proposal that the CDF should become a 'managed access' fund. The rationale for the CDF was to improve patient access to newer cancer drugs, which it has done, but the CDF in its current form is not sustainable given the pressure presented by the growing pipeline of new drugs. Not taking action to reform the CDF could ultimately lead to fewer transformative drugs being made available to patients. NHS England, NICE and other partners must continue to do as much as possible to support cancer patients to have a wider range of treatment options and access to newer, evidence-based cancer drugs where appropriate.

We have heard from patients that they want a process that places an emphasis on the added benefit of new drugs, collects good data, and acts as quickly as possible. They would also like to see any changes implemented quickly to ensure minimal disruption for patients. Furthermore, patients need certainty that drugs will continue to be commissioned unless clinical evidence suggests otherwise, rather than cost. Clinicians have expressed similar wishes, and want to see a process which prioritises helping them access the most innovative drugs, and give their most difficult to treat patients a wider range of options.

The managed access fund model is a sensible approach to allowing patients to have the most promising drugs earlier, while increasing the financial sustainability of the CDF. By providing a funding direction to some drugs at the point of license, it will provide early access with more certainty than the current CDF. In the CDF drugs may have funding removed based on unpredictable budget fluctuations, whereas conditionally-funded NICE drugs under the proposals would only have funded removed if their value compared to existing treatments is unproven. It is encouraging that more data in general will be collected on the benefits of new drugs – patients and clinicians have expressed major disappointment to us that this was a missed opportunity within the current CDF.

The managed access approach must be transparent in terms of the criteria used to recommend drugs on a conditional basis, and flexible around the data requirements for each drug. Where cost prohibited a conditional recommendation, this should be stated clearly. Though NICE will have to consider the individual context for each drug and develop a data collection plan based on this, the process for this and how the data is considered should be transparent.

[3] http://csg.ncri.org.uk/
[4] http://www.ecmcnetwork.org.uk/about-us

NHS England and NICE should ensure that no patient would miss out on appropriate treatment based on the 'first come first served' approach to managed access, where only the number of patients needed for data collection will have their treatment funded by the CDF. This could be a particular problem for common cancers where the number of patients needing treatment may create high demand quickly. It is not clear that industry will be required to fund further patients after sufficient patient numbers are achieved, but a NICE rapid review is still pending. NHS England and NICE should ensure the time lag between data collection achieved and decision should be as short as possible.

Another important issue is the predictability of Committee for Medicinal Products for Human Use (CHMP) opinions and their timing. NICE already asks companies for their best possible estimates of the timing of marketing authorisation of their products, but these can significantly miss the mark. It would be helpful to know what engagement has taken place with the CHMP and companies to understand how NICE can better anticipate licensing decisions.

NICE must be given sufficient resource to carry out more rapid decisions based on an increased workload for the Technology Appraisal Programme.

The CDF in its current form has created a disparity in access to some cancer drugs across England, Wales and Northern Ireland. These proposals will not address this issue, so NICE should be engaging with the devolved administrations about the impact of the proposals and how each country can avoid inequalities in access as far as possible.

Finally, it would be helpful to have confirmation of what budget the CDF will be allocated going forward, as well as the resource provided for the CDF Investment Group, as neither is specified in the document.

NICE should be transparent in stating the rationale for decisions to provide conditional access or not, and considerations made around data requirements.

NHS England should clarify funding requirements after patient numbers for data collection are met.

NICE should engage with colleagues in the devolved nations more closely in this process and clarify how the changes will impact patients in Wales and Northern Ireland, where there are no specific funds to manage conditional access.

2. **Do you agree with the proposal that all new cancer drugs and significant new licensed cancer indications will be referred to NICE for appraisal?**

Agree

This is encouraging in principle, as lack of NICE guidance for some cancer drugs can create disparities in patient access. Having all cancer drugs looked at could improve certainty for patients. However the problems associated with patient access to drugs for rarer cancers– which we discuss in our answer to question 3 below – will only be addressed if more cancer drugs ultimately receive a Final

Appraisal Decision from NICE. Simply putting more drugs through the appraisal process will be unhelpful if they are likely to struggle based on available data.

NHS England and NICE should be clear about what approach will be taken for those drugs that may not otherwise be referred to NICE because of issues such as low patient numbers and a paucity of data. Such drugs will clearly struggle based on the evidence requirements expected of those for more common diseases, so NICE should therefore be more flexible about its evidence requirements in these cases.

NICE should be more flexible on the evidence requirements for indications in rarer cancers to make the process fair for them.

3. **Do you agree with the proposal that the NICE Technology Appraisal Process, appropriately modified, will be used to evaluate all new licensed cancer drugs and significant license extensions for existing drugs?**

Agree

NICE should provide further details on what approach it will take to assessing indications for rarer cancers – if NICE is to evaluate all cancer drugs in future, it is crucial that it can do so in a fair manner. NICE technology appraisal guidance does not guarantee uptake of new drugs, but where it is absent commissioners are unlikely to fund the drugs in question given cost constraints. As such, cancer drugs that do not get past the NICE scoping process – for example in rare cancers – are at a disadvantage. That NICE will look at all cancer drugs is therefore helpful, as noted in our response to question 2 above.

Feedback from clinicians is that the CDF to date has helped improve access to drugs for rarer cancers. There are likely to be a small number of these drugs each year, though a considerable proportion of new cancer drugs – for example, in 2014 the EMA granted marketing authorisations (either new applications or license extensions) to 15 new cancer drugs, and 6 of these had orphan designation.[5]

Ensuring that all new cancer drugs and significant license extensions are appraised by NICE should help remove what is in practice an inequity between rarer and more common cancers in terms of recommendations NICE makes around access to treatments. It is crucial that NICE allocates the necessary resource to carry out an increased number of technology appraisals, which will happen more quickly, and is flexible about evidence requirements for these. NICE should provide further details on what approach it will take to assessing indications for rarer cancers – if NICE is to evaluate all cancer drugs in future, it is crucial that it can do so in a fair manner.

4. **Do you agree with the proposal that a new category of NICE recommendations for cancer drugs is introduced, meaning that the outcome of the NICE Technology Appraisal Committee's evaluation would be a set of recommendations falling into one of the following three categories:**
   - **i. Recommended for routine use**
   - **ii. Recommended for use within the Cancer Drugs Fund**

[5] Report provided by EMA to CRUK via email

iii. Not recommended

Agree

In line with our response to question 1 around the managed access model, we agree with this in principle. In practice, this should allow NICE to make conditional rather than negative recommendations for more drugs, and continue to make positive recommendations as appropriate. Drugs recommended through the CDF route will by definition not have sufficient evidence to warrant a definitive decision. In keeping with NICE's transparent approach to publishing decisions, NICE should publish summaries of how it considered the evidence and its views on any uncertainties for all cancer drug appraisals.

NICE should be more flexible on timelines should more than two years be needed to assess a drug further to a "Recommended for use within the CDF" decision. Some drugs, for example those in rarer cancers, may need relatively more time and support for data collection so NICE must be sensitive to context. In some cases, additional clinical data may not add more to the decision making process so NICE should be pragmatic about the requirements it makes around evidence generation.

NICE should be more flexible on timelines needed to assess a drug further to a "Recommended for use within the CDF" decision, and data requirements for each drug should be context-dependent.

5. **Do you agree with the proposal that "*patient population of 7000 or less within the accumulated population of patients described in the marketing authorisation*" be removed from the criteria for the high cost effectiveness threshold to apply?**

Agree

The 7000 patient threshold does not clearly align with the purpose of the End of Life Criteria, which supports NICE to recommend drugs with higher incremental cost-effectiveness where it would be used for patients in an end of life setting. It is not clear how much difference this change will make in practice, but the removal of an arbitrary limit is welcome.

6. **Do you agree with the proposal for a draft NICE cancer drug guidance to be published before a drug receives its marketing authorisation?**

Agree

There is clearly a trade off to be made in investing the necessary resource in a NICE appraisal and accepting the risk that a drug will not gain a marketing authorisation (MA). We believe this proposal makes a worthwhile trade off if it ultimately allows more patients to access some drugs very close to or at the point of marketing authorisation. The current CDF does this but provides unstable funding for drugs. The presumption of patient access before NICE's final decision, using funding that is linked to NICE decisions, would therefore be a radical and welcome change.

If in future there are a significant number of appraisals that do not go forward because the drug did not receive an MA, this proposal would need to be reviewed.

7. Do you agree with the process changes that NICE will need to put in place in order for guidance to be issued within 90 days of marketing authorisation for cancer drugs going through the normal European Medicines Agency process?

Agree

No additional comments.

8. **Do you agree with the proposal that all drugs that receive a draft NICE recommendation for routine use, or for conditional use within the CDF, receive interim funding from the point of marketing authorisation until the final appraisal decision, normally within 90 days of marketing authorisation?**

Agree

No additional comments.

9. **What are your views on the alternative scenario set out at paragraph 38, to provide interim funding for drugs from the point of marketing authorisation if a NICE draft recommendation has not yet been produced, given that this would imply lower funding for other drugs in the CDF that have actually been assessed by NICE as worthwhile for CDF funding?**

Appraisals where submissions have been made in a timely manner to allow NICE to make a faster decision should be prioritised for funding. This approach should incentivise all parties to work towards a faster decision.

10. **Do you have any comments about when and how it might be appropriate for the CDF in due course to take account of off-label drugs, and how might this be addressed?**

A separate piece of policy development should be carried out to understand the different scenarios where off-label/unlicensed indications might emerge and be eligible for routine funding. Off-label use is important for treating many cancer patients and the NHS should take advantage of opportunities to learn more about this to increase patient benefit. There are some immediate steps that could support patient access to off-label drugs in appropriate contexts:

- Anecdotal evidence from NHS England and clinicians suggests that the CDF is well used to fund off-label indications. This would be through the Individual Cancer Drugs Fund Request (ICDFR) route. Since the ICDFR route will presumably disappear further to the proposed reforms, as the CDF will effectively become an early stage of the NICE process, the generic Individual Funding Request (IFR) process will need to be made more flexible to support off-label prescribing in cancer. Clinicians tell us that current IFR processes are not supporting them sufficiently to prescribe some treatments for their most difficult to treat patients.

- Better data collection is needed on off-label use to support commissioning and patient access. Clinicians have stressed the importance of this to us, as we may risk missing novel approaches to offer to patients, for example where there is a change in dosage or schedule.

- Better use could be made of other NICE guidance (outside the Technology Appraisal Programme) to support off-label use where deemed appropriate.

11. **Do you agree with the proposal to fix the CDF annual budget allocation and apply investment control mechanisms within the fixed budget as set out in this consultation document?**

Agree

We broadly agree with the stated approach, however, there is a risk that only funding treatment for the number of patients needed for robust data collection could create inequitable access amongst patients. We would be extremely concerned if NHS England and NICE planned to limit access to those patients requesting a treatment after this 'threshold' of patients needed for data collection has been reached. NHS England and NICE should therefore clarify what specific requirements will be made of industry to cover costs for additional patients. An important part of NICE's current approach is the ability to negotiate confidential discounts with industry – this should be continued under the new system and further developed. For example, the Patient Access Scheme Liaison Unit (PASLU) should be open to more innovative schemes beyond simple discounts, where these will clearly bring patients benefit and manage budgets.

PASLU should be more open to innovative pricing schemes in future to increase the scope for making new drugs cost-effective for the NHS.

12. **Do you consider that the investment control arrangements suggested are appropriate for achieving transparency, equity of access, fair treatment for manufacturers and operational effectiveness, while also containing the budget? Are there any alternative mechanisms which you consider would be more effective in achieving those aims?**

No comment.

13. **Are there any other issues that you regard as important considerations in designing the future arrangements for the CDF?**

We believe that a managed access approach will likely be needed in any future system of cancer drugs funding. However, in the longer term we also believe that a wider review of the pathway for access to cancer drugs is still needed to address the extent that it works for innovative new drugs, including assessment of NICE processes and cost-effectiveness thresholds. This would not need to be carried out to the same timescales as the current consultation, but is a crucial aspect of access to medicines which should be considered. The recent Triennial Review of NICE and views we have heard expressed by a range of stakeholders suggest NICE is highly trusted and viewed as very effective, and therefore any further changes should build on this platform.

It is important that the new approach is complementary to, and does not detract from, research. Funding treatment costs for patients in nationally peer-reviewed non-commercial studies is often no longer assumed to be a priority, and obtaining funding for Excess Treatment Costs (ETCs) can be very challenging for researchers. Some trials could create cost savings for the NHS – for example, in demonstrating the value of a lower dose of treatment – but detailed financial negotiations on ETCs

can stop some trials from opening, particularly where the manufacturer has declined to provide free drugs as the conclusions may damage commercial interests. NHS England has recently added important clarifications to its ETC policy[6] with more emphasis on the onus of commissioners to cover ETCs. The reform of the CDF is a useful opportunity to raise awareness of this, and build links with the National Cancer Research Network (NCRN), National Cancer Research Institute (NCRI) and other networks. The planned evaluation of the new ETC policy should include impact on cancer research and alignment with changes to the CDF.

It bears repeating that clarity is needed on the potential impact on the UK devolved nations – particularly Wales and Northern Ireland. If a drug receives a "Recommended for use within the CDF" then this is effectively a recommendation from NICE, which both countries normally follow – but in this case the costs of the drug are presumably covered by the CDF, which is part of the English health budget and is governed by NHS England. CDF-funded drugs have been much less readily available to cancer patients in the UK outside of England to date, though unwarranted gaps in access have not been quantified. This will continue if only English patients can access drugs with this conditional recommendation.

It will be important that the new approach aligns with the Early Access to Medicines Scheme and the EMA's forthcoming PRIME designation, and that NICE and NHS England can guarantee a smooth transition from industry funding to NHS funding when the drug, in theory, is simultaneously licensed and receives a decision from NICE.

In general the reforms put an emphasis on NICE doing more, and making faster decisions. It is extremely important that NICE is sufficiently resourced for this greater workload.

We believe that the current rules around fulfilling Individual Cancer Drugs Fund Requests (ICDFRs) are far too restrictive, and a review of the Individual Funding Request process, including ICDFRs, is needed. Exceptional access to certain medicines is extremely important for some patients who do not fit the "typical" patient profile for a given indication, based on the relevant clinical trial(s) evidence that led to the medicine's approval by regulators and NICE. In cancer, there is consensus that patients vary widely in their response to treatments[7]. Developments in genetics and genomics may improve our understanding of why this variability exists, but this will take time. In the meantime, the arbitrary limit on the number of patients with certain circumstances to count as "exceptional" is unhelpful.

We note that NICE has separately circulated details on planned transition arrangements. These appear broadly sound if NICE is provided with sufficient resource to fulfil its increased workload. However, it is not clear how the group of drugs currently funded via the CDF that do not have a planned NICE appraisal will be funded after April 2016. We understand that companies would be expected to make submissions for these drugs to be funded under the new system, but would like clarity on whether access to these drugs will still be funded in the intervening period.

NHS England should ensure greater alignment of these proposals with the national cancer research agenda.

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[6] https://www.england.nhs.uk/commissioning/research/etc/

[7] http://www.nature.com/nrd/journal/v1/n1/full/nrd705.html

NICE and NHS England should provide further detail on what the proposals will mean for drugs going through the Early Access to Medicines Scheme (EAMS) and drugs receiving the EMA's planned PRIME designation.

14. **Do you agree that, on balance, the new CDF arrangements are preferable to existing arrangements, given the current pressures the CDF is facing?**

Agree