



**PROSTATE
CANCER UK**

tackle

Action for patients

Registered Charity No. 1163152

Consultation on Proposals for a New Cancer Drugs Fund (CDF) Operating Model from 1st April 2016

Prostate Cancer UK and Tackle Response

Introduction

This response has been developed collaboratively between Prostate Cancer UK and Tackle and includes the views of 52 men surveyed for their views on the proposals to reform the CDF.

It is clear that men with prostate cancer have benefitted from the CDF. In fact, thousands of men with this disease have had early access to life-saving new treatments, many of which have subsequently taken a long time to enter into routine commissioning. Without the CDF and with only NICE to rely on, many of these men would have been denied access to new treatments that have extended or improved their lives. We cannot accept any reforms that return men to the pre-CDF world of repeated prostate cancer treatment rejections. Consequently, our response aims to highlight where we believe the reforms make this pre-CDF return likely. We also suggest ways that we think the proposals can be improved so that men have a greater likelihood of getting access to new treatments.

Question Responses

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

Disagree

Unsure

Please provide comments to support your response:

We recognise that the proposals enable the management of a finite budget that has recently come under pressure from the high demand for new and innovative cancer treatments, as well as the high prices pharmaceutical companies were able to charge for these. We also welcome the continuation of a ring fenced fund for cancer treatments that enables them to receive conditional approval while further evidence is collected to demonstrate their efficacy, and while patients have the opportunity of early access to new and innovative cancer treatments.

However, we have concerns that the finite pot available – that a recent addendum to the PPRS (1) has demonstrated will be reduced in coming years – will be unable to achieve the patient access to new and innovative treatments made possible by the original Cancer Drug Fund. Our concerns can be attributed to the following:

- The ability of a reduced finite pot to fund the pipeline of new and innovative cancer treatments without requiring manufacturers to substantially drop their prices below the price agreed at draft appraisal. This could likely only be achieved if the appraisal price is guaranteed and is substantial enough to incentivise those manufacturers who receive a conditional approval to use this conditional period to demonstrate clinical benefit. Given the need to achieve cost-effectiveness in a NICE appraisal, we question whether the appraisal price could ever be enough to offset the potential price reductions needed to enter the CDF;
- The extent to which the pharmaceutical industry will make their treatments sufficiently affordable to access the Fund without incentives that can reduce the cost of expensive development costs. A potential incentive could be in the form of surrogate endpoints, which would shorten lengthy Phase III trials and provide the opportunity of longer patents;
- The potential for manufacturers to avoid a conditional approval because of:
 - a) the longer-term risk of providing treatments for free, should the final determination after the evaluation period end in a 'no' and;
 - b) the cost of funding the collection of a pre-determined data set over a period of up to 24 months, when the price being paid for the treatment may leave them in financial deficit

- The financial risks associated with these funding requirements may see manufacturers wait for their data to mature before pursuing a licence, which would delay patient access;
- The level of patient access made possible during conditional approval, when NICE determine the patient numbers required for further evaluation of a treatment with evidence uncertainties.

We believe that a more robust solution is needed - one that supports the pharmaceutical industry to make its treatments affordable to the NHS while also accounting for the wider NHS savings each treatment makes possible. Any approach that simply attempts to manage manufacturer cost cutting without their full support is likely only to see patients lose out.

44% of the men we surveyed were concerned that the new proposals would be a step backwards for men needing the relevant treatments to help them deal with advanced prostate cancer. They wanted to see a more patient-centric approach to the process. They also feared that costs would dictate drug availability and patient need would be lost in the equation.

"I have benefited from the Cancer Drugs Fund for the past 14 months and I'm still responding well to treatment. So grateful every day for the time it is giving me to enjoy life with my wife, daughters and elderly parents. Precious time to make practical arrangements, and laugh together".

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

Disagree

Unsure

Please provide comments to support your response:

The Standard Operating Procedure that was introduced to identify which treatments to remove from a Cancer Drugs Fund close to the brink of financial collapse, created appraisal duplication. Referring all new cancer drugs and significant new licensed cancer indications to NICE will remove this duplication. At the same time, the opportunity of a faster appraisal for new cancer treatments to some extent offers the potential for earlier patient access to these treatments that was a core benefit of the original Cancer Drugs Fund. The opportunity for conditional approval also potentially offsets a return to the numerous rejections of new cancer treatments that were the basis for the Cancer Drugs Fund to be set up.

However, we question whether a conditional approval, though a very welcome step-change to the appraisal process, is sufficient to resolve the constant rejection of new cancer treatments that preceded the Cancer Drugs Fund. These rejections resulted in half of the patients supported by the Fund between April 2013 to March 2015 receiving drugs that had previously been appraised but not recommended by NICE because they did not meet its clinical and/or cost-effectiveness thresholds (2).

Clearly a conditional approval will demonstrate that cost-effectiveness has been achieved. However, we are not convinced that the evaluation period as recommended by the proposals will necessarily be able to demonstrate the level of clinical benefit required by NICE, unless it is an opportunity for the manufacturer to continue a Phase III trial. This is because the evaluation approach proposed does not replicate the efficacy of a clinical trial and may not be capable of providing the evidence of clinical benefit needed for an approval within the two year timeframe set. The evaluation may also have its efficacy affected if the patient numbers set for it are dictated by the level of CDF money available and not what is needed to deliver an effective clinical trial.

In addition to this, the NICE appraisal that results in a conditional approval will not immediately result in patient access, if the manufacturer is required to negotiate an affordable CDF price via the CDF Investment Group. If this is the case - and the consultation document does not make this clear - then this negotiation process could delay patient access. Worse still, if a price cannot be agreed, patient access may not occur at all.

We believe that wider reforms are needed if all new cancer drugs and significant new licensed cancer indications are to be referred to NICE for appraisal, without a CDF in its current form.

These could include:

- The opportunity for clinical effectiveness to be determined through surrogate endpoints so that the efficacy of the treatment can be established and appraised quicker, with the potential to make the evaluation process provided by a conditional approval less necessary;
- An emphasis on progression free survival that is equal to overall survival, not only because this data can be reached quicker, but because clinical benefit should not just be about how long people will live, but that they live better lives. This is critical for prostate cancer treatments that can offer deliver strong progression free clinical benefit, but low overall survival benefit;
- The evaluation of new treatments in relation to where they sit on the treatment pathway. As more new treatments for a disease come through – and there is evidence of 90 clinical trials underway for prostate cancer – they cannot be appraised in isolation. Doing so will limit the identification of the patient sub-population most likely to benefit. It will also prevent a thorough understanding of when in their treatment pathway patients will gain most benefit from a new treatment and in what sequence. Without this evidence there is no means to narrow the eligible patient population, which is critical not only to patient outcomes, but also an ability to likely reduce the treatment’s cost to the NHS and therefore increase patients’ access to it;
- The means to identify the wider savings to the NHS that a new treatment provides and to account for this in the determination of its cost effectiveness.

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 licence extensions for existing drugs?

Agree

Disagree

Unsure

Please provide comments to support your response

We can see the benefit of having one process by which treatments are routinely introduced into the NHS and value the opportunity of a faster process and an additional route to approval via conditional approval.

However, we have some concerns about the proposed modifications.

- There is no process set out for treatments that demonstrate strong clinical benefit but are not cost-effective, other than a rejection. What is more, NICE still has no powers for price negotiation to achieve cost-effectiveness and there is insufficient time made available in the process and ahead of the draft appraisal for manufacturers to develop flexible pricing models or to pursue a patient access scheme;
- Treatments that are given a conditional approval appear to need to be provided at a lower price, if they are to ensure their entry to a finite CDF. It is not clear what incentives are in place to motivate manufacturers to cut their prices and evaluate their treatment further, rather than wait for their data to mature before seeking a license. If they did the latter, patient access would simply be delayed;
- Any treatment given conditional approval that after 2 years of further evaluation then receives a NICE rejection will leave the manufacturer liable to continue to provide the treatment, for free to the patients already receiving it. This has the potential to disincentivise manufacturers to pursue the conditional approval route. A CDF contingency pot should be made available instead;
- There is nothing to guarantee that an approval after conditional approval will enable the manufacturer to achieve the original draft appraisal price – assuming the price paid by the CDF is lower than the original draft appraisal. Without this guarantee, there is likely to be no incentive for the manufacturer to pursue a conditional approval;
- There is also a real risk that not all patients who need a drug will be able to access it during the data collection period. This is because the proposals suggest collecting data from an agreed number of patients. This could leave many others without treatment. We consider it completely unacceptable for there to be this variation in access, particularly if the treatment is offering an extension of life, as this will be leaving some patients to die, while others do not.

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;

iii. Not recommended.

Agree

Disagree

Unsure

Please provide comments to support your response:

We have already outlined some of the concerns we have for the conditional approval option – please see above. The following sets out some of our other issues with this approach:

- There are no mechanisms outlined in the proposals to enable horizon scanning so that the pipeline of new cancer treatments is known in advance. Equally important is advance insight into how many of these treatments will likely receive conditional approval. Without these insights it is not possible to determine how a finite pot can be allocated to enable all of the treatments with data uncertainties to receive CDF funding;
- There is therefore a risk that there will be more treatments requiring CDF funding than budget available. What is not clear is how entry to the CDF can be equitable and no new treatment left unfunded. We believe a process is needed so that the Fund is not simply accessed on a first come, first serve basis and that patient need is put at the centre of this process; perhaps with greater priority given to those treatments that deliver to an unmet need.

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 higher cost effectiveness threshold to apply?

Agree

Disagree

Unsure

Please provide comments to support your response:

We agree

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

Agree

Disagree

Unsure

Please provide comments to support your response:

We value the earlier access to treatments that this approach could make possible, but are also concerned whether manufacturers will have been able to complete their complex pricing processes or what opportunities will be made available to them to price flexibly or pursue a patient access scheme.

There is also potential for the data made available for appraisal will be immature, as a license is often pursued before data has fully matured. If this is the case, it will likely result in high demand for CDF funding via a conditional approval that outstrips the budget available, leaving patients without access to newly licensed treatments.

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 licensing process?

Agree

Disagree

Unsure

Please provide comments to support your response:

We value the opportunity of a faster appraisal process and think there is potential to use the 90 days for the following:

- For NICE to work with the manufacturer to provide opportunities for pricing to meet the QALY threshold, should the draft appraisal have resulted in a no on the basis of a failure to demonstrate cost-effectiveness, but the treatment has demonstrable clinical benefit. If effective, it is possible that the draft decision could be changed to a final recommendation for routine use or conditional approval, making patient access more likely;
- For the manufacturer to have time to present new data, should a draft conditional approval be based on data uncertainties brought about by the way data was presented or where there are only a few data anomalies. Again, this has the potential for the final determination to recommend for routine use, removing the need for CDF funding.

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

Disagree

Unsure

Please provide comments to support your response:

There will need to be a mechanism in place to ensure that patients receiving the treatment continue to access it, should the final appraisal decision result in a no. This mechanism cannot

wholly rely on the pharmaceutical industry to provide the treatments for free, as this may disincentivise them to go for marketing authorization until they have a clear demonstration of clinical benefit, which would delay patient access. There may therefore need to be a CDF contingency fund put in place.

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?

This scenario will give patients accelerated access to treatments they could potentially benefit from, but it could also see the CDF used to fund a treatment that has little or no clinical benefit. In addition, if the draft decision from NICE is no, but the treatment does have clinical benefit, then there has to be a contingency available that makes sure patients who are on the treatment continue to access it. A route is also needed for this treatment to be reappraised and for price negotiations to take place with the manufacturer if it is rejected because it has failed to demonstrate cost effectiveness.

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

There is a potential to expand the use of a NICE Rapid Evidence Review, rather than an appraisal, which could be conducted using pre-published trial data. This would need to involve the relevant NHS England Clinical Reference Group, who can use the time before publication to determine the treatment's cost-effectiveness for the NHS. The CDF could then be used while the in-year budget or full commissioning policy is under development so that patients who will benefit get access as soon as publication occurs.

Docetaxel, an off-patent repurposed drug, has demonstrated a survival benefit of over 15 months as part of a large-scale clinical trial called STAMPEDE. Prostate Cancer UK recommended that the relevant Accelerated Access Review strand use docetaxel's early adoption onto the NHS as an exemplar. Since then, we have worked collaboratively with NHS England to get this treatment commissioned sooner after the research's publication than we think has happened before. We believe there is potential for the process that NHS England used to be replicated across clinically beneficial, off-patent re-purposed treatments.

We recognise that Docetaxel does not face the re-licensing challenges of re-purposed treatments used for another indication, but believe that an update to General Medical Council Guidance on off-label prescribing has the potential to remove the bureaucratic and costly burden of re-licensing. We would of course only recommend this where there is sufficient published evidence of the treatment's clinical benefit.

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

Disagree

✓ **Unsure**

Please provide comments to support your response:

Good practice would dictate that it is better to stay within the planned budget. However it is questionable whether the budget available – and which is set to reduce over the coming years - reflects the demand for cancer patient treatments that the CDF overspend has demonstrated.

If control mechanisms are to be put in place, better horizon scanning is needed to determine the cancer drugs pipeline so that scenario planning can be put in place. This will enable each new treatment likely to require CDF funding to get an appropriate share of the cake in relation to the patient population. Manufacturers can then know how much funding is available for each treatment, while NHS England can know the number of treatments they need to fund. No treatment loses out and all patients that could benefit get access, assuming manufacturers are prepared to accept the price on offer. In addition to this, there needs to be flexibility for treatments that horizon scanning does not pick up.

Without this type of budgeting approach, there is a potential for the number of treatments requiring entry to the CDF to outstrip the budget available. The only alternative is to have clear criteria for the treatments that take priority for funding, but these will need to be developed in consultation with patient organisations, so that no patient loses out in favour of another.

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?

Transparency is only possible if the number of treatments the CDF can fund is made clear from the outset for each financial year and there is clarity about the price that will be paid for them. The number of fundable treatments – even if an estimate – needs to include all those on the cancer drugs pipeline that are likely to result in conditional approval. Without this there can be no equity of access. Operational effectiveness can only be determined if and when the CDF is operational and will require a robust, independent evaluation of its ability to provide access to new treatments.

There is also a danger that Industry may take steps to keep the number of drugs on the CDF low to ensure they do not find themselves penalized by having to cover the cost of a CDF overspend

Our survey showed that 38% of respondents wanted the NHS to develop a partnership approach with the pharmaceutical industry. This included greater acknowledgement of the costs the industry incur to bring a drug to market and to respond to this with a suitable reimbursement process. There was recognition that there were inevitable limitations of a public health service, and only a transparent process could ensure fair process where there may be varying opinions on availability of treatments.

“A large number of our members have benefited from the CDF. To say it had been a lifesaver would be a gross understatement. It has given them access to the drugs they needed when all other avenues to life saving treatment were closed at the very time they needed it.”

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

When evaluating a new medicine, NICE should engage with the relevant sub-population of patients to understand their perceptions of quality of life, which can change as a disease progresses. We therefore recommend that:

- The pharmaceutical industry provides better quality of life data that is specific to the patient population that would benefit, as well as to their type and stage of the disease;
- NICE brings together a group of patients relevant to the new medicine during their appraisal of a new treatment. These patients should be supported to score quality of life in relation to the overall survival benefit of the new medicine and their scores should be taken into account by the technical committee.

Our survey of men showed that 10% wanted frontline clinicians to be more involved in making decisions about the availability of drugs. They felt that clinicians played a key role in many NICE processes but that greater value should be given to their independent judgement, professional expertise and experience and their views were not adequately represented in the process. As such their increased inclusion in the proposed process would be beneficial.

“All treatments that are available should be offered to all men with prostate cancer whenever the doctor thinks it is appropriate.”

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

Disagree

Unsure

Please provide comments to support your response:

We believe that these proposals can only be effective if manufacturers are prepared to make their treatments affordable to the NHS. We recognise that a finite NHS budget requires policies that motivate them to do this, but think this creates a stand off between the industry and NHS England / NICE in which the only losers are patients.

There has to be a more robust solution that can incentivise and support manufacturers to make their treatments affordable to the NHS, while also being able to take account of the wider NHS cost savings their treatments can make possible. What we also need is a set of reform proposals that are less about restricting the pharmaceutical profits possible in a climate of finite health resources and more about what patients need and deserve.

Our survey showed that 15% of respondents wanted more patient involvement at all levels of the proposed structure that would be more meaningful than simply having patient advocates. They expressed the view that men with prostate cancer should be participating as equal partners on decision making bodies and in the on-going oversight of the new CDF process.

15% of respondents also perceived an inequality in terms of the drugs available across the range of cancers, with many wanting more transparency built into the new process. An example given was the availability of drugs to tackle bone health that were perceived to be freely available to women, but to which men appeared to have limited access.

“My husband was given access to drugs via Cancer Drugs Fund to extend life. Every additional moment spent with loved ones is a blessing....”

(1) Department of Health and the Association of the British Pharmaceutical Industry. Addendum to the 2014 Pharmaceutical Price Regulation Scheme [Internet]. 2015. Available from: https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/487579/CDF_revised_addendum_acc.pdf

(2) House of Commons, Committee of Public Accounts. Cancer Drugs Fund Twentieth Report of Session 2015–16 [Internet]. 2016. Available from: <http://www.publications.parliament.uk/pa/cm201516/cmselect/cmpubacc/583/583.pdf>