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BOARD PAPER - NHS ENGLAND

Title:

Cancer Drugs Fund

Lead Director:

Bruce Keogh, National Medical Director **/** Paul Baumann, Chief Financial Officer

Purpose of Paper:

A 12 week public consultation on proposals for reforming the Cancer Drugs Fund (CDF) closed on 11th February 2016. In light of consultation responses received, the Board is asked to consider and agree proposals for a phased and managed transition from the current CDF to a new operating model. In particular this includes:

- Agreeing the implementation of a new managed access fund, with clear entry and exit criteria;
- Agreeing that the new scheme should go live from 1 July 2016 to allow for further work on the operational detail;
- Agreeing that existing CDF drug indications should continue to receive transitional funding, subject to certain conditions, from 1st April 2016 until the point that NICE is able to complete their appraisal or reconsideration of these drugs;
- Agreeing the financial control mechanisms set out in this paper; and
- Agreeing the overall budget for the CDF of £340m.

For patients, the new CDF will help provide faster access to the most promising new cancer treatments.

For taxpayers, the new CDF will drive stronger value for money in drugs expenditure.

For drug companies willing to price their products responsibly, the new CDF offers a new fast-track route to NHS funding for promising drugs at the point of marketing authorisation, with a speeded up and more transparent NICE assessment process.

The Board is invited to:

The Board is asked to approve the proposal set out in this paper.

CANCER DRUGS FUND

PURPOSE

1. The Board concluded in late 2015 that the current arrangements for the Cancer Drugs Fund (CDF) were unsustainable and inappropriate, and that there was a need for fundamental and urgent change to the fund's operating model; this need was also underlined by the National Audit Office (NAO), the Public Accounts Committee (PAC) and the independent Cancer Taskforce in recent reports. They and others argued that while the current CDF has produced meaningful benefits, it also has badly overspent- thereby subtracting funding from other aspects of cancer care and other patient services. In part this has been because the current CDF has led to potentially inflated drug prices for sometimes limited efficacy, offering poor value for money. With the approval of the Board, we therefore initiated a joint 12 week public consultation with the National Institute for Health and Care Excellence (NICE) regarding a new CDF operating model, and this closed on 11 February 2016. In the light of this consultation, the Board is asked to consider and agree proposals for a phased and managed transition from the current CDF to a new operating model. NICE will be taking those aspects of the proposals that are relevant to their processes and methods to their Board for approval on 17 March 2016.

2. The consultation report at **Annex A** includes details of the number of responses by stakeholder type and responses to each consultation question. The original text of replies is available to the Board on request. The published consultation document is included at **Annex B**.

OVERVIEW

3. The proposal, aspects of which have been amended in the light of consultation, is as follows:

   - **The CDF becomes a new managed access fund with clear entry and exit criteria**, in line with the proposals set out in the consultation document;

   - **The new scheme will 'go live' from 1 July 2016**. From this point, new drugs will be able to enter the CDF under the terms of the new scheme;

   - **The operational detail of the new scheme will be developed over the coming months, informed by further detailed analysis and consideration of the consultation responses received**. A new Standard Operating Procedure will be published by June;

   - **On 1 April 2016 the current CDF list will be rolled over but will remain closed to new drugs pending the start of the new scheme in July 2016**. All existing CDF drugs will continue to receive funding until the point that NICE has been able to appraise / re-consider them (unless their manufacturer/sponsor does not co-operate promptly with the appraisal process). Off-label drugs will also continue to receive funding until such time as a routine funding decision can be taken;

   - **Any existing CDF drug that is not recommended for continued use within the new CDF or for routine commissioning as a consequence of this appraisal / re-consideration process will be given a notice period. However, patients in receipt of those drugs will continue to receive them.** This period of notice will not be given any earlier than 1 July 2016. Ahead of these appraisal / re-considerations, relevant companies will have had, where clinically appropriate, the opportunity to review their pricing levels with a view to their product either continuing to receive CDF funding or being approved for "routine commissioning" by NICE;

- **In preparation for the operational start of the new scheme, NICE will begin using their proposed new methodology for appraisals from 1 April 2016**, subject to consideration and approval by the NICE Board; and

- **The fixed financial limit of £340m for the CDF will fund both new and transitional CDF drugs**. Should the need arise, the same financial control mechanisms will be applied to both new drugs and existing CDF drugs awaiting appraisal / re-consideration. Acceptance of those controls will be made a condition of existing drugs awaiting appraisal / re-consideration remaining in the CDF after 1 July 2016.

CONTEXT

4. The CDF was developed in 2010 to improve access to cancer drugs that had not been adopted for routine use in the NHS. It provided the capability to fund drugs that were not eligible to go through the NICE appraisal process, such as drugs for rare conditions. It was only ever intended as a temporary measure and, as such, no clear criteria to allow drugs to exit the fund were developed. The current version of the CDF was set to expire in April 2016.

5. The annual budget for the CDF has been increased from £200m in 2011/12 to £340m in 2015/16. Despite this, the CDF has exceeded its allocated budget each year since 2013/14, primarily because more and more drugs have entered but few have had issues of clinical and cost effectiveness uncertainty resolved. The National Audit Office and Public Accounts Committee have both criticised these overspends.

6. Two delisting exercises have been undertaken in order to help bring significant budget overspends under control, but these have not had sufficient impact and the current approach represents a completely unsustainable way of commissioning cancer drugs.

7. The NAO, Public Accounts Committee and the independent Cancer Taskforce have all recognised in recent reports that there is a need for the CDF to change. In particular, the PAC indicated that '*NHS England should set clear objectives for what the fund is seeking to achieve, and be prepared to take tough decisions to ensure the Fund does not overspend'*. This requires an improved methodology for budget management, an efficient means of evaluating the effectiveness of drugs and transparency for pharmaceutical companies.

8. In addition to CDF reform, the Department of Health is developing proposals for its Accelerated Access Review (AAR) which is seeking to develop sustainable ways of increasing the uptake of transformative drugs and technologies, including 'Breakthrough' drugs, across all conditions. There will be opportunities to further consider alignment here providing the conclusions and recommendations of the AAR are available in advance of finalising the new CDF Standard Operating Procedure.

CONSULTATION

9. An analysis of the consultation responses is provided at **Annex A.** We received 286 responses in total through both the consultation hub and written submissions. In addition we held four webinars for stakeholders (85 attendees) and two face-to-face events in London and Manchester (115 attendees) alongside a number of individual meetings with key stakeholder groups. The table below outlines the response rates for each question.

10. Three key themes emerged from the consultation:

- Firstly, there was significant support for change, including specifically a move to a managed access process;

- Secondly, stakeholders suggested that there was a need to conduct further work to refine

and clarify the operational detail with regard to the management of the new CDF, particularly entry & exit criteria and financial control mechanisms; and,

- Thirdly, respondents were keen to understand more about the transition between the old and new operating models.

11. Some consultees expressed concern about whether the proposals would lead to more or less access to cancer drugs for patients when compared to the current system. In contrast, the consultation also produced strong views from some respondents that prioritising cancer drugs through the CDF was not equitable when compared to other conditions. There were also calls for broader and more general reform of NICE in order to improve access to and evaluation of all treatments.,

12. 264 of the consultation responses provided quantifiable information. The breakdown of responses is summarised in figures 1 and 2 below (please note not all respondents replied to all 14 questions) and the list of questions asked is included at **Appendix 1**.

Figure 1: Overall summary of responses to all questions

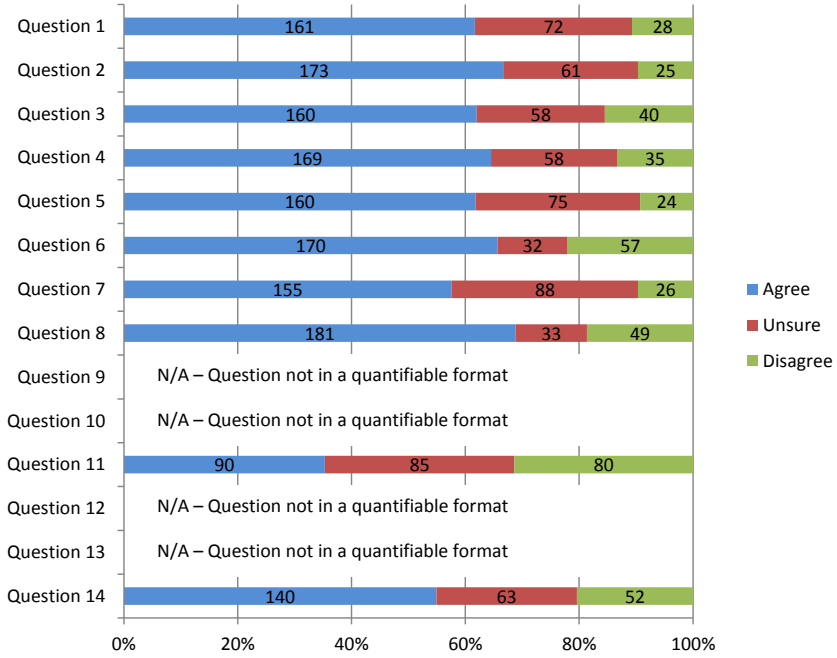
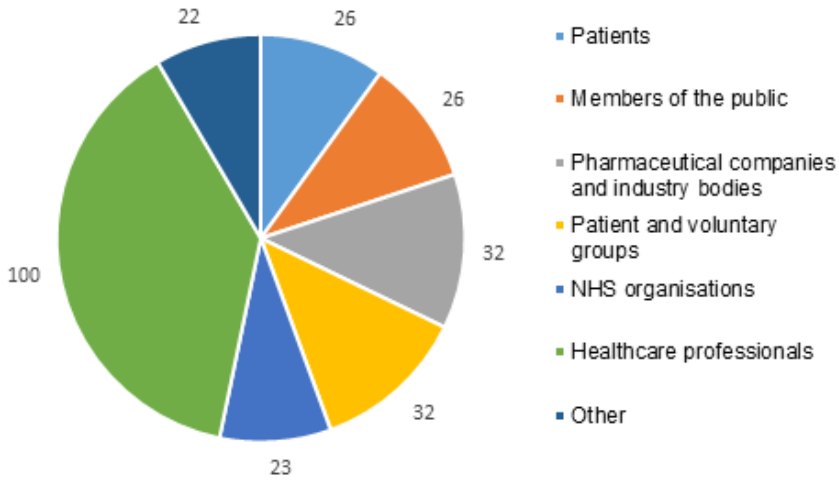


Figure 2: Breakdown of responses by stakeholder group



A NEW MANAGED ACCESS PROCESS

13. The consultation indicated support for the implementation of a managed access process. This process will utilise the clinical and health economics expertise of NICE and fulfil NHS England's requirement to manage the budget efficiently by providing a clear basis for decisions about what enters and exits the CDF. **Appendix 2** presents the new CDF process as a diagram with supporting narrative.

14. The key aspects of the managed access process are:

    - A change to existing process, meaning that all new licensed cancer drugs (including those that were previously not appraised due to small population size) will be referred to NICE for appraisal;

    - A much faster NICE process such that NICE will make a draft recommendation before marketing authorisation. Any drugs that receive either a draft recommendation for routine commissioning or, where uncertainty exists, a recommendation for use within the CDF will receive interim funding from the CDF from the point of marketing authorisation;

    - NICE will then normally issue final guidance within 90 days of marketing authorisation. If drugs are recommended for routine commissioning at this point, they will be funded by normal commissioning budgets. If drugs are recommended for substantive entry into the CDF, a joint NHS England and NICE CDF Investment Group will meet to agree the terms of any commercial access agreement, including evaluation criteria and a timescale for evaluation to complete;

    - At the end of the CDF evaluation period NICE will re-appraise the drug with the aim of making a final positive or negative assessment as to whether the drug should be routinely commissioned. Any patients still in receipt of treatment with drugs not recommended for routine commissioning at this point will continue to be treated, but with the funding provided by the relevant drug company until their treatment is completed.

15. The NICE process leading to guidance normally being issued within 90 days of receipt of marketing authorisation will be put in place for topics referred by Ministers after 1st April 2016. This will only apply to products referred to NICE early enough to allow the commitment to be met.

16. It should be noted that the new process will satisfy one of the ambitions of the Early Access to Medicines Scheme, which rests on the earliest possible start for NICE technology appraisal. This should have a positive impact in terms of ensuring earlier funding of and patient access to those drugs that NICE consider to be clinically and cost effective or demonstrate the potential to be clinically and cost effective. This benefit would also be felt by those cancer drugs that had obtained a 'Breakthrough' designation. However, this also represents an additional demand upon NICE and consultation respondents were concerned regarding the capacity within NICE to undertake assessments in a timely manner. Following detailed discussions between NHS England and NICE, resource is being put in place to ensure delivery of the number of additional appraisals required within the stated timescales, and NICE has committed to these timescales accordingly.

Recommendation 1: The Board agrees to establish the CDF as a new managed access process with clear entry and exit criteria as set out in the consultation document and summarised at Appendix 2.

DETAILED OPERATING PROCEDURE

17. The consultation identified a number of different opinions on how to operationalise a reformed CDF, and our stakeholder engagement events highlighted the need to take time to ensure an orderly transition to the new arrangements. As a result, we propose to implement a phased transition and begin the new CDF operating model from 1 July 2016. This gives NHS England and NICE time to refine and clarify the operational detail, taking into account relevant considerations arising from the consultation responses received during the consultation exercise just closed. The initial analysis indicates that it will be particularly important to consider carefully the following:

    - The practical aspects of publishing initial NICE recommendations prior to marketing authorisation. Several pharmaceutical companies indicated that these would need to avoid pre-empting final wording within the marketing authorisation.

    - The risk of providing interim funding for drugs from the point of marketing authorisation if a NICE draft recommendation has not been produced. Respondents indicated that this should be avoided by maintaining sufficient capacity within NICE. However, if a draft recommendation is not produced there is a danger of funding ineffective treatments which become difficult to withdraw from the CDF at a later date should the NICE evaluation be negative. This could create pressure on the CDF budget.

    - Ensuring that rare cancers are not overlooked. This includes recognising the importance of off-label drugs and considering what the role of NICE could be in the evaluation of off-label treatments in the future.

    - The proposal to limit funding to the number of patients required to support evaluation. Whilst this proposal was intended to maximise the number of drugs able enter the fund before further financial control mechanisms might need to be applied, 45 respondents questioned whether this was the best approach. Further consideration as to how best to maximise the breadth and depth of access, whilst remaining within the fixed financial envelope, will be important as the detailed operating procedure is developed.

18. Furthermore, the need for accurate data collection in terms of measuring key clinical outcomes will be critical to the success of the new CDF. The PAC has asked NHS England to report by June 2016 on what measures are being taken to improve data completeness. As such, and as part of the work to develop the operational detail, the Board is asked to note that it is our current intention that for drugs given substantive entry into the new CDF, any hospital trust wishing to administer such new CDF drugs must:

        - have electronic prescribing systems in place and used for the prescribing of all intravenous and oral chemotherapy; and,

        - be fully compliant with the collection of the Systemic Anti Cancer Therapy dataset for all its patients having intravenous and oral chemotherapy

19. A detailed standard operating procedure (SOP) will be submitted to the Specialised Services Commissioning Committee for approval and published by June 2016.

Recommendation 2: The Board agrees that the new scheme should go live from 1 July 2016, allowing time to work through important operational details, informed by relevant responses to the consultation, and that authority to agree the standard operating procedure and to make any other necessary arrangements and changes to the operational introduction or timing of the new scheme should be delegated to the Specialised Services Commissioning Committee.

TRANSITION ARRANGEMENTS – EXISTING CDF DRUGS

20. The consultation indicated that respondents wanted clear transition arrangements between the old and new CDF operating models. The following paragraphs set out the proposed arrangements, which will be further developed as part of the new Standard Operating Procedure between now and June.

21. In order to ensure a smooth and fair transition to the new scheme, it is proposed that the existing CDF list should "roll over" from 1 April 2016, with all drug indications continuing to receive funding until the point that NICE is able to issue a final appraisal / re-consideration decision (providing their manufacturer/sponsor co-operates with the NICE appraisal process). The current CDF scheme would continue to remain closed to new drugs pending the introduction of the new CDF operating model from 1 July 2016.

22. NICE will apply the new decision making methodology referred to in **Appendix 2** to existing CDF drug indications, and could propose to the CDF Investment Group that the drug should be considered for the new CDF. NICE will set out its final appraisals and re-considerations timetable in March.

23. It is proposed that two conditions should apply to existing CDF drugs receiving transitional funding from 1 April 2016:

    - Firstly, that reimbursement of each drug indication is no higher than the level as at 31 March 2016; and

    - Secondly, that from 1 July 2016 existing CDF drugs receiving transitional funding (i.e. pending NICE appraisal / re-consideration) will be liable to the same financial control mechanisms as new drugs entering the new CDF (see affordability and financial control section below).

24. Any existing CDF drugs that are subsequently recommended for routine commissioning will cease to receive CDF funding, as their costs will be picked up as part of routine baseline commissioning. For existing CDF drugs appraised or re-considered as part of transition that are not recommended for routine commissioning, having taken account of the pricing that drug companies propose, companies will be given a notice period, but all patients already in receipt of these drugs will continue to get them.

25. In addition to the above process, we recognise that for certain rare cancers the provision of off-label drugs remains an important issue. The work to clarify the operational detail will consider the views of consultation respondents on how this should be addressed, but in the meantime existing CDF off-label drugs will continue to receive funding.

Recommendation 3: The Board agrees that, from 1 April 2016, the current CDF list should be rolled over but remain closed to new drugs, with funding made available until the point of NICE issuing guidance. The Board also notes the broader transitional arrangements proposed for existing CDF drugs and agrees to delegate final decisions on their application to the Chief Executive, National Medical Director and Chief Financial Officer, in partnership with NICE.

AFFORDABILITY AND FINANCIAL CONTROL

26. The CDF needs to be affordable. We recognise that consultation responses were uncertain regarding the proposal to fix the annual CDF budget but most comments indicate recognition of the need for improved budgetary control and a requirement to limit the impact of the CDF on

wider NHS services. The operational detail regarding financial control is still to be finalised and will explain clearly how the arrangements will work.

27. Based on the above proposals there is a period between 1 April 2016 and 30 June 2016, where the only call on the fixed CDF budget will come from existing drugs (transitional drugs). Financial modelling indicates that this restriction, coupled with expected NICE appraisal recommendations over this period, will ensure there is sufficient funding available in the first quarter share of the annual CDF funding for all transitional drugs.

28. The operational mechanisms regarding financial control are being put in place to allow for the flow of eligible new drugs into the CDF whilst ensuring that the CDF does not overspend. The published SOP will clearly describe these financial control mechanisms based on the following principles:

    - the incremental cost effectiveness ratio of any new CDF drugs must potentially fall within the standard NICE range, if any reasonable uncertainty in the drug's cost effectiveness were eventually to be resolved in the drug's favour;

    - the total cost of each individual drug to the CDF will be limited via the terms of the commercial access agreement put in place;
    - a prospective contingency will be put in place whereby:

        - the amount paid by the CDF to all companies during the year is set at a consistent level below 100% of the sums otherwise due, with the remainder retained as a contingency;
        - if the CDF stays within the resulting net budget, the contingency will be released to companies;
        - if the CDF exceeds the net budget, the contingency will be retained as necessary to balance the budget, with the remainder paid to companies proportionately;
        - if the CDF exceeds the net budget and the contingency, the whole contingency will be used to reduce the overspend, and, exceptionally, a further across the board rebate for each CDF drug will be applied. (There are some parallels with the voluntary PPRS scheme rebate. However, at the request of the ABPI, spending on the CDF over and above £320m in 2016/17 will not be captured by the existing PPRS rebate mechanism, further underlining why other budget control mechanisms are needed).

29. The need to utilise the prospective contingency mechanism will depend on several factors, including the outcome of NICE appraisals on both existing CDF drugs and new drugs and the commercial arrangements put forward by drug manufacturers. Supplier agreement to all of the above mechanisms will be a condition of funding under the new arrangements from 1 July 2016 for both new drugs and those transferred from the existing CDF pending appraisal or re-consideration under the new scheme.

30. The operation of the financial control mechanisms will be the responsibility of the proposed CDF Investment Group, a joint committee of NHS England and NICE.

Recommendation 4: The Board approves the methodology for keeping CDF expenditure in line with the budget, including the transition from the old to new CDF operating models, confirms a fixed annual budget of £340m for the CDF, and agrees to delegate final decisions on the detail of the methodology to the Chief Executive, National Medical Director and Chief Financial Officer.

CONCLUSION

31. The Board is asked to consider and agree proposals for a phased and managed transition from the current CDF to a new operating model. In particular this includes:

    - Agreeing the implementation of a new managed access fund, with clear entry and exit criteria;
    - Agreeing that the new scheme should go live from 1 July 2016 to allow for further work on the operational detail;
    - Agreeing that existing CDF drug indications should continue to receive transitional funding, subject to certain conditions, from 1st April 2016 until the point that NICE is able to complete their appraisal or reconsideration of these drugs.
    - Agreeing the financial control mechanisms set out in this paper;
    - Agreeing the delegations of authority described above; and,
    - Confirming the overall budget for the CDF of £340m.

Author: **Bruce Keogh, National Medical Director / Paul Baumann, Chief Financial Officer**
Date: **25 February 2016**

Appendix 1 – List of Consultation Questions

Question 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?

Question 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?

Question 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?

Question 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.

Question 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?

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

Question 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?

Question 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?

Question 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?

Question 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?

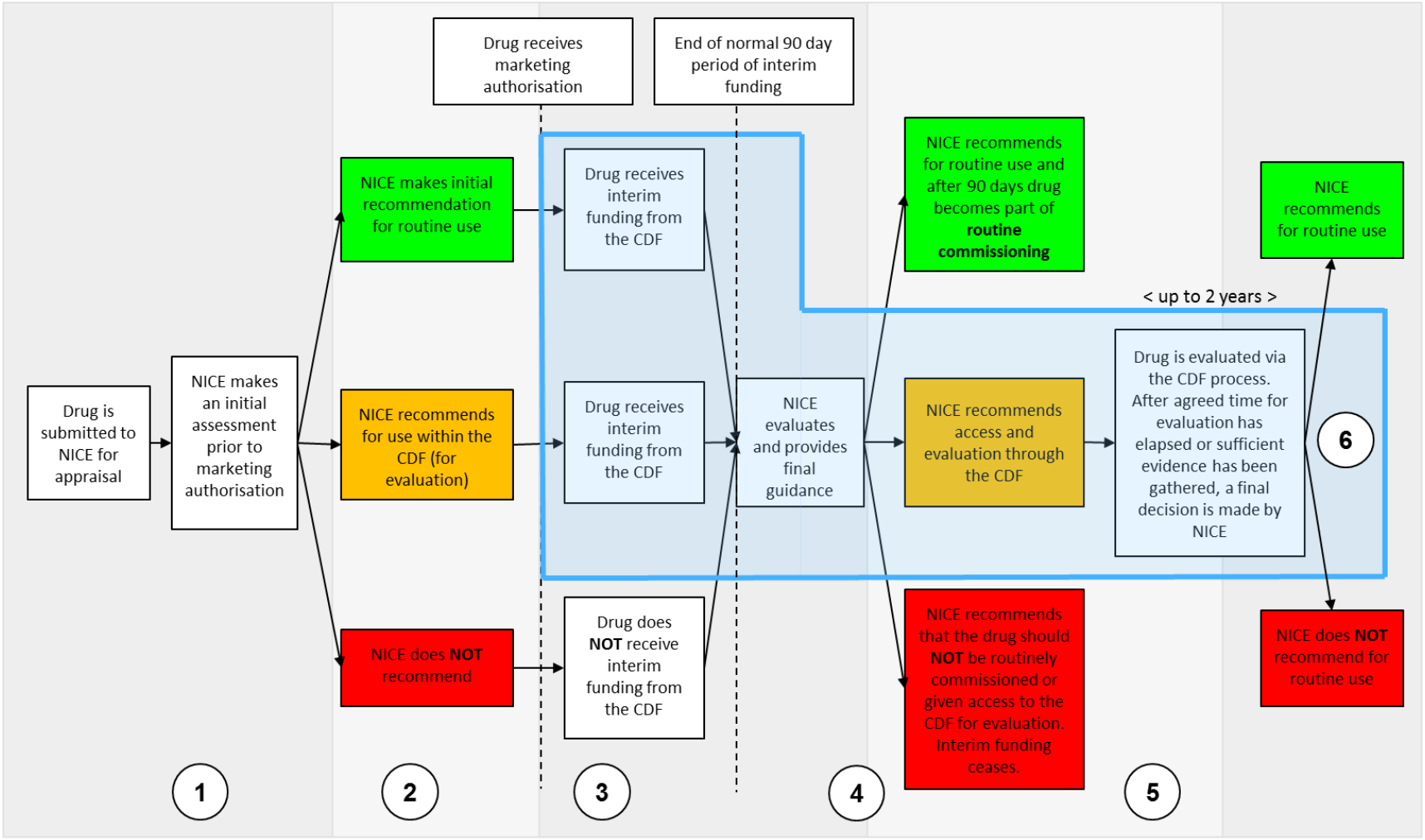
Question 11: Do you agree with the proposal to fix the CDF annual budget allocation and apply investment control mechanisms within the fixed budget as set out in this consultation document?

Question 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?

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

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

Appendix 2 – The Managed Access Process



1: All new cancer drugs expecting to be licensed are referred to NICE for appraisal prior to marketing authorisation

2: NICE makes an initial recommendation based on clinical and cost effectiveness

3: Interim funding is provided by the CDF whilst NICE undertake their full appraisal

4: NICE makes a final recommendation. Drugs recommended to enter the CDF must meet NHS England commercial requirements

5: CDF drugs are evaluated against specific criteria for a set period. After the evaluation period they are given a 'yes' or 'no' recommendation by NICE

6: Light blue shading denotes where CDF funding applies (subject to financial controls & £340m budget)

CONSULTATION ON PROPOSALS FOR A NEW CANCER DRUGS FUND (CDF) OPERATING MODEL FROM 1 APRIL 2016

Analysis of responses to the consultation

24 February 2016

Contents

1. Introduction

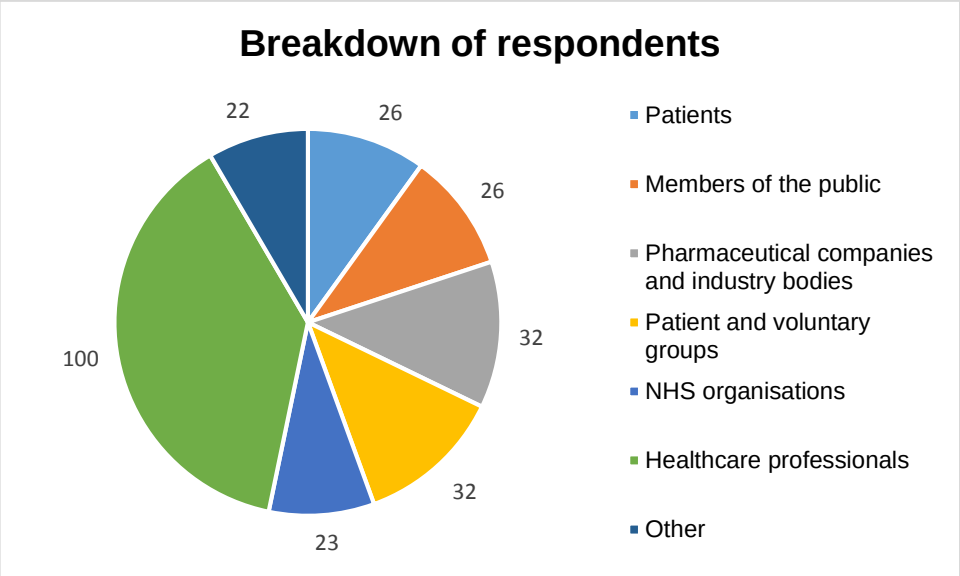
This report covers the responses received to the consultation on the proposals for a new Cancer Drugs Fund operating model which ran from 19 November 2015 to 11 February 2016.

The use of quotes throughout the document is to illustrate some of the main issues raised. They do not necessarily reflect a balance of opinions.

2. The consultation in numbers

The consultation received 264 online responses and 22 written submissions. We are aware that there is some duplication; for example organisations which responded online, but also sent in a written submission.

3. Who responded to the consultation?



Responses were received from:

- 23 pharmaceutical companies, including[1]:
  - AbbVie
  - Amgen
  - ARiAD
  - Astellas
  - Astra Zeneca
  - Baxalta
  - Baxter
  - Bayer
  - Boehringer Ingelheim
  - Bristol-Myers Squibb
  - Celgene
  - Eisai
  - Eli Lilly
  - Janssen
  - Merck
  - MSD
  - Novartis
  - Pfizer
  - Roche
  - Sanofi
  - Sobi
  - Takeda UK

[1] This list contains the names given by respondents identifying themselves as Pharmaceutical Companies. Not all supplied their names.

- Nine professional and industry bodies, including:
  - Association of the British Pharmaceutical Industry
  - British Association of Urological Surgeons
  - British In Vitro Diagnostics Association
  - British Oncology Pharmacy Association
  - The Ethical Medicines Industry Group (EMIG)
  - Royal College of Radiologists
  - Royal College of Physicians
  - Royal College of Surgeons
  - UK BioIndustry Association

- 29 responses from the patient and voluntary groups[2], including:
  - Action on Smoking and Health
  - Bloodwise
  - Cancer Research UK
  - Beating Bowel Cancer
  - Breast Cancer Care
  - Breast Cancer Now
  - The Blood Cancer Alliance
  - Cancer52
  - The Chronic Myeloid Leukaemia Support Group
  - CLIC Sargent
  - CLL Support Assocciation
  - Genetic Alliance UK
  - Leukaemia CARE
  - Lymphoma Association
  - Myeloma UK
  - Ovarian Cancer Action
  - Pancreatic Cancer UK
  - Prostate Cancer UK and Tackle
  - Rarer Cancers Foundation
  - Target Ovarian Cancer

- NHS organisations:
  - Nine NHS acute trusts
  - Two NHS community organisations

- Two trade unions

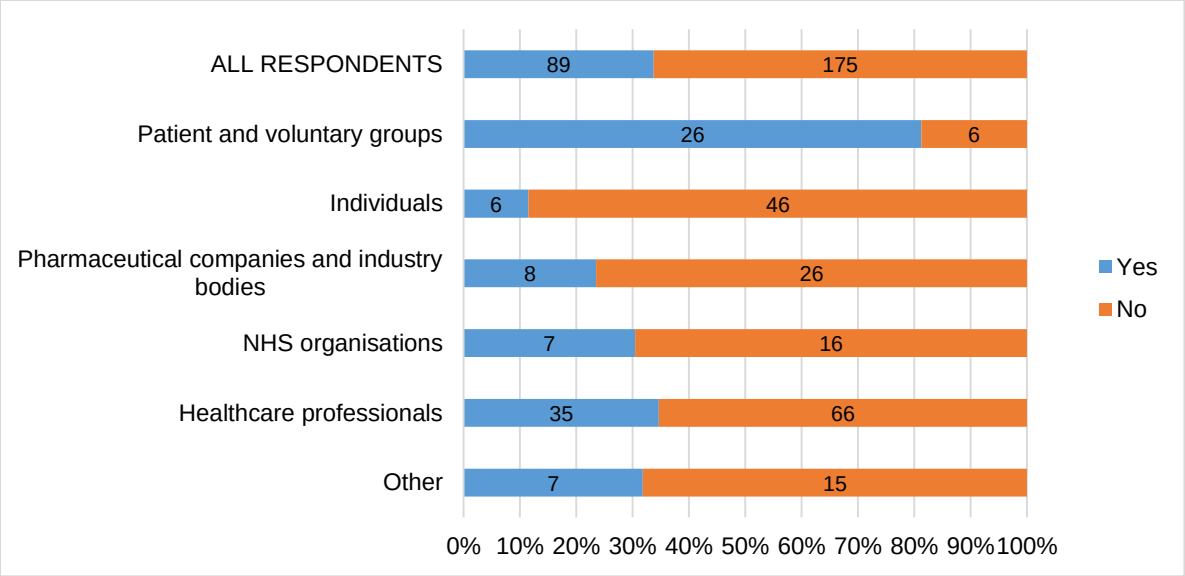
- Other organisations and individuals, which include:
  - All Party Parliamentary Group on Pancreatic Cancer
  - All Party Parliamentary Group on Cancer
  - Brain Tumour Research
  - Clinical Commissioning Groups and other NHS organisations
  - London Cancer
  - Members of Parliament
  - NHS England
  - Public Health England

- Three educational establishments

[2] This list contains the names given by respondents identifying themselves as patient and voluntary groups. Not all supplied their names.

‘Sunshine’ provision/conflict of interest disclosures

Respondents were asked whether they had received any payments, grants or other funding from the pharmaceutical industry in the last three years.

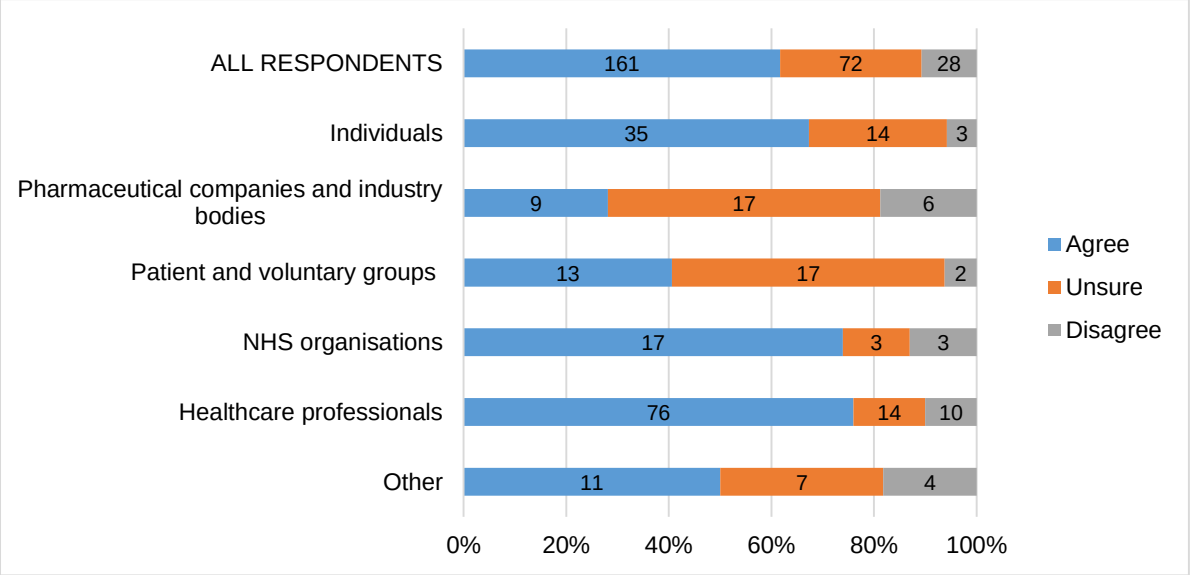


Overall, 34% of respondents declared they had received payments from drug companies.

The highest percentage of respondents affirming that they had received such payments were patients and voluntary organisations, 81% of whom said they received drug company funding.

4. Analysis of responses to the questionnaire by question

Question 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?



61 per cent of respondents agreed with the proposal, with 27% saying they were unsure, the majority of whom wanted clarification and transparency on the entry and exit criteria.

Patients and the public

61.5% of patients agreed with the proposal. In support, they said it was common sense and addressed inequity. They described the approach as evidence-based which they said would address inequity and be less likely to fund ineffective treatment. Several people commented that it should include diseases other than cancer.

> *I think it is appropriate to have clear criteria by which drugs are assessed and that data on their effectiveness is collected throughout their use.*
>
> **Female patient, aged 35-54**

Members of the public also agreed (73%); of the rest, most were unsure. Concern was expressed about a one-size-fits-all approach and not treating cases on an individual basis. There was also an anxiety that it would be more bureaucratic.

> *I am strongly in favour of an evidence based approach to the introduction of new medicines/technologies/procedures.*
>
> ***Member of the public, male, aged over 55***

Only three people disagreed from the two groups. There was a view that this would need wider reformation of NICE.

Organisations

Pharmaceutical companies and industry bodies were mainly unsure about this proposal (53%) rather than agreeing or disagreeing, wanting more detail of how it would work in practice.

In principle, […] agrees with the concept of a 'managed access' fund and the need for clear entry and exit criteria; however, we are concerned about the proposed criteria as set out in the consultation … Specifically, […] does not agree with the entry criteria whereby only patients for whom data needs to be collected will have treatment funded out of the CDF and other patients 'above this number will be paid for by the company', nor with the strict exit criteria with an arbitrary two-year timeframe in which to collect data.

Pharmaceutical company

Respondents from patient and voluntary groups were also unsure (53%).

We agree with the principle of a managed access fund … We also agree that any new medicines access scheme should aim to have clear entry and exit criteria... Whilst we welcome the principle of providing access to new treatments whilst awaiting further data and a final appraisal decision, we remain concerned over the lack of details on how NICE will operate when assessing medicines for routine commissioning …

Patient/voluntary organisation

NHS organisations were supportive with nearly 74% of respondents agreeing with the proposal.

This seems to provide a system which would be transparent and clear to manufacturers but also to patients and clinicians who currently have some difficulty understanding why some drugs are taken off the list. It also provides some reassurance for patients with other life-threatening conditions for whom no equivalent to the CDF exists.

NHS organisation

Healthcare professionals

Most healthcare professionals agreed with the proposal (76%); this included 80% of doctors who responded to this question. The main reasons for agreement were beliefs that the current system did not work well, was unfair or undermined the role of NICE.

I am satisfied that this will allow early and timely use of new agents, while monitoring their benefits and cost effectiveness

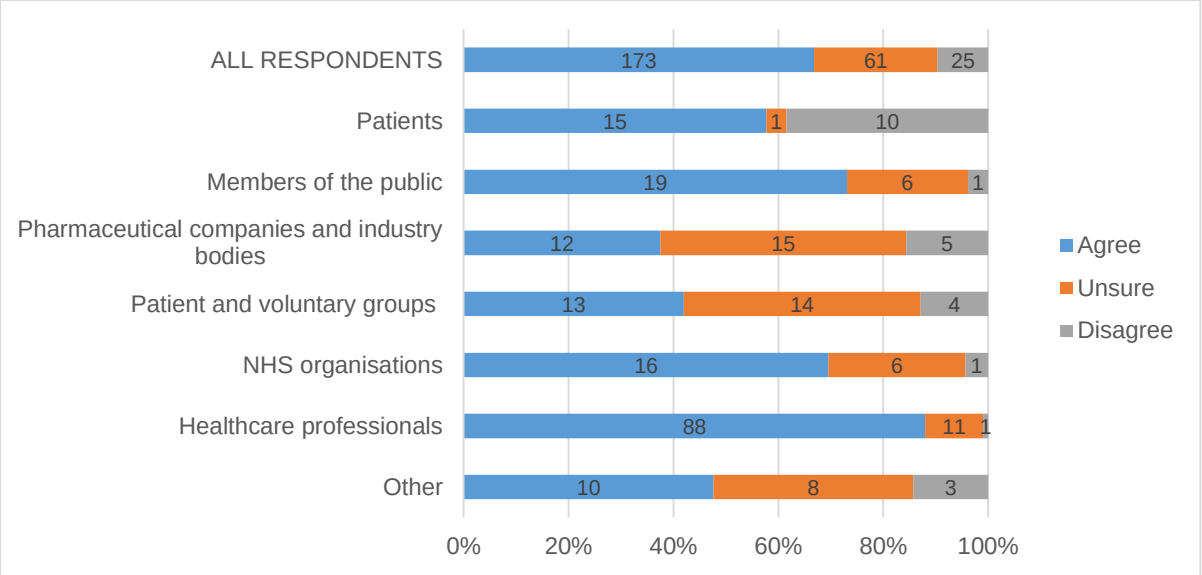
Female doctor, NHS acute trust

Some people agreed because they felt a new system could have a positive impact on the collection of data. Others agreed because they felt cancer drugs which had little or no proven benefit should not be funded.

Of those that disagreed, concerns raised were that a drugs fund should not be restricted to cancer treatments and that a CDF undermines NICE processes.

Those who felt unsure stated a range of reasons. Some felt unsure about what the new proposal would entail, and what the entry and exit criteria would be. Again, many did not support a fund specifically for cancer patients.

Question 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?



67% of respondents agreed with the proposal. However, there were concerns about the impact on NICE and its workload arising from implementation of this proposal.

Patients and the public

58% of patients and 73% of members of the public agreed with this proposal, stating that they felt that NICE committees have the expertise to do this. There was also some concern about the speed at which NICE could do this and whether it would add bureaucracy.

> *NICE is there to govern quality*
>
> **Male patient, aged over 55**

However, 38% of patient respondents disagreed, stating that they felt that NICE would not be able to cope and would take too long. There was also concern that NICE was financially-driven and did not have a good track record in funding new drugs and/or innovative treatments.

> *NICE does not have a good track record for the funding of new drugs/innovative treatments.*
>
> **Female patient, aged over 55**

Organisations

38% of pharmaceutical companies and industry bodies agreed and 16% disagreed with the remainder saying they were unsure.

> *[…]supports in principle the proposal that all new cancer drugs and indications be referred to NICE for appraisal but only if significant NICE reform takes place to create a broader value assessment for cancer medicines. It is clear through an analysis of the drugs currently in the CDF, that the existing NICE evaluation framework is not fit for purpose vis-a-vis cancer drugs in general and a fair process needs to be established for medicines for rarer cancers specifically.*
>
> **Pharmaceutical company**

There was strong support from NHS organisations (67%) for the proposal, while patient and voluntary organisations were split between agreeing and being unsure (42% and 45% respectively).

> *We agree that there should be a single body responsible for assessing new cancer drugs, and that NICE is the most appropriate body to be able to do this. However NICE needs the resources so that decisions are made in a timely manner. Until now the CDF has provided a stop-gap, but it will be a better system if NICE can publish their 1st appraisal in a timely manner. We need to be assured that NICE have the capacity to be able to do this.*
>
> **NHS acute trust**

> *We support a system for the evaluation and commissioning of medicines and services which would allow each to be assessed on its own merits by balancing the benefit it offers to patients against the cost and then funded accordingly … However, we are concerned that there has been no indication that the capacity of NICE will be increased to handle this increased number of cancer medicine appraisals.*
>
> **Patient/voluntary organisation**

Healthcare professionals

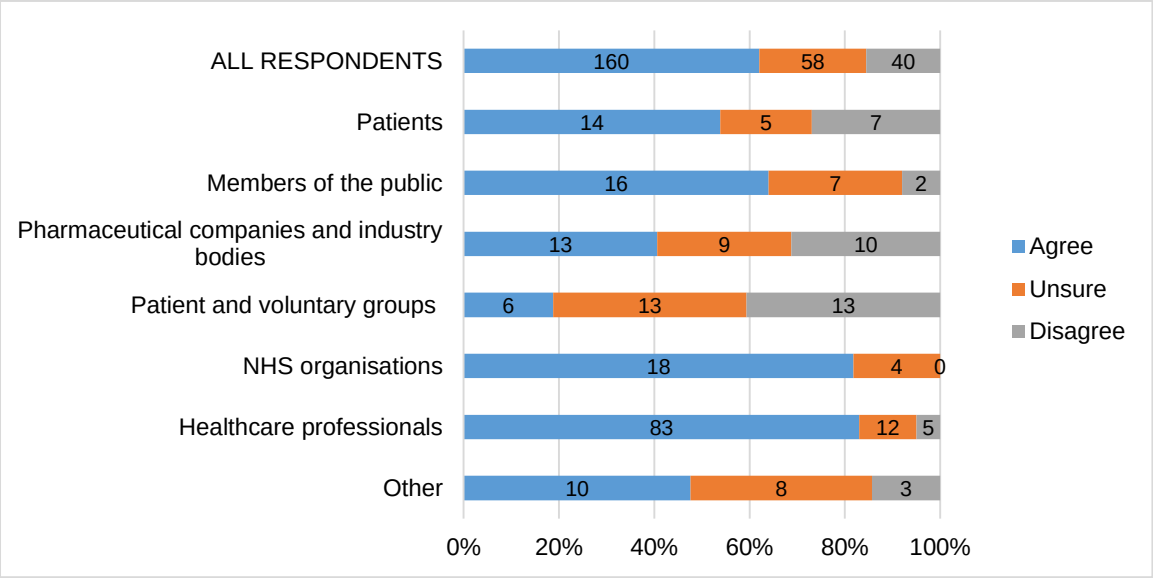
The majority of healthcare professionals agreed with this proposal (88%).

> *Yes, we agree with this process. It is essential that all new drugs or new indications should go to NICE for appraisal and final approval … However it is essential that this process will be carried out in a reasonable time frame and not significantly delay the access*
>
> **Male doctor, health advisory group, aged 35-54**

However, some people highlighted that this would have an impact on the workload of NICE, and wanted assurance that this would be managed. Others referred to timescales, and had concerns they would be too long.

A small number of people disagreed or were unsure; mostly this was due to concerns over the ability of NICE to take on the extra work. There were also some concerns raised in relation to 'off-label' cancer drugs, and that they needed to be included in this process (in particular for rare cancers).

Question 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?



Over 60% agreed with the proposal, with the highest level of approval coming from healthcare professionals. Those most likely to disagree were pharmaceutical companies and patient and voluntary organisation organisations.

Patients and the public

54% of patients agreed with the proposal. There was some concern about the capacity of NICE, the speed of the process and whether the evaluation board would include cancer specialists.

> *As long as this does not have a negative impact on the availability of treatments or the quality.*
>
> **Male patient, over 55**

> *I am surprised that all drugs aren't already evaluated by some regulatory body already.*
>
> **Female patient, aged over 55**

However, 27% disagreed. They felt the process was too slow.

> *When you have cancer, you just want to be given the opportunity to try any suitable available process.*
>
> **Male patient, over 55**

There was also some concern about whether this would be suitable for appraising patients with rarer cancers.

19% were unsure. Again they felt the process needed to be quick as well as transparent and consistent. Some respondents also felt they did not have enough information or knowledge to give a view on this question.

Organisations

While 41% of pharmaceutical companies agreed with the proposal, there was support from the professional bodies with none disagreeing with the proposal (only two of the 10 who responded were unsure).

> *We agree with this proposal and feel that it builds on 15 years of the technology appraisal process used by NICE which is much more robust than creating a parallel system just for cancer.*
>
> **Professional body**

> *Whilst we agree that the modified NICE appraisal process should be used to evaluate all new licensed cancer drugs, the modifications suggested do not fundamentally alter the appraisal process and they are often unclear.*
>
> **Pharmaceutical company**

Patienta nd voluntary organisations were equally split between disagreeing and not being sure (41% each), with 19% agreeing.

> *There needs to be a quicker and more transparent way of appraising and assessing new cancer drugs but as stated above, the criteria goes against what we need to see happening for less common cancers. We are aware of the disproportionate funding through the CDF between common and less common cancers.*
>
> **Patient/voluntary organisation**

> *Our support for NICE appraising all cancer drugs is contingent on NICE being reformed to an extent where we feel new treatments for rare cancers and cancers of unmet need will receive a better chance of being recommended for commissioning.*
>
> **Patient/voluntary organisation**

NHS organisations were supportive with 82% agreeing with the proposal.

> *There needs to be a single assessment process, using a standard health economic model. NICE has been around for many years and has developed expertise and skills in this field.*
>
> **NHS acute trust**

Healthcare professionals

Most healthcare professionals agreed with this proposal (83%).

> *The process is not perfect but it has stood the test of time and is clear.*
>
> **Female doctor, NHS acute, aged 35-54**

Like patients and the public, there were some who wanted further assurances about the process, in particular that it would not take too long.

> *… needs to be a much faster process than currently, and has to have much more specific criteria about when it can be used and what can be used before and very importantly, as this is not currently looked at, later lines of treatment.*
>
> **Female pharmacist, NHS acute, aged 35-54**

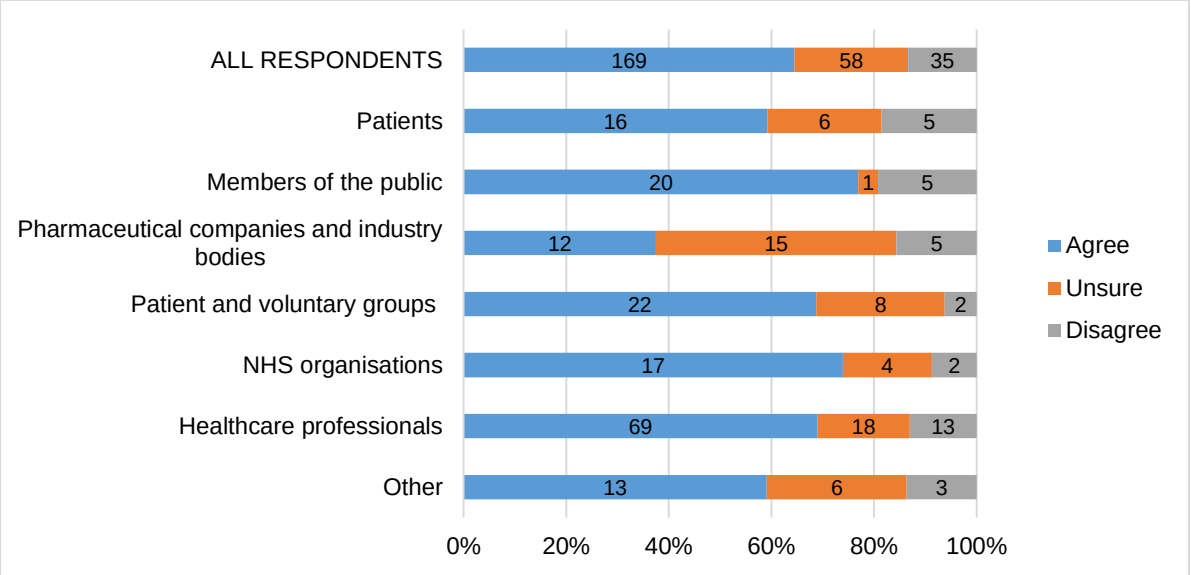
A small number of people were unsure. Again, some of the concern was about the process taking too long and the capacity of NICE. Others felt unsure about the terminology and implications of 'appropriate modification' and 'significant licence extensions'.

> *Makes sense to have a single process, however there needs to be consideration of how the process will manage multiple products with same indication, launched in a sequential manner…The existing multi technology appraisal route takes significantly longer than other options, so how will this be managed to fit time frame. Concerned that NICE has the capacity to deal with the volume of applications in a timely manner without reducing the responsiveness for non cancer TAs and other publications.*
>
> **Male pharmacist, NHS acute, aged 35-54**

A small number of people (5) disagreed. Again, the main reason was concern about the time this process would take.

Question 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.



65% of respondents agreed with the proposal, but 22% said they were unsure (of those, pharmaceutical companies and industry bodies were the most unsure).

Patients and the public

59% of patients and 77% of members of the public agreed with the proposal. One person described it as the most important part of the proposal. Another said the cost and benefit needed to be weighed against the overall constraints of the NHS. The importance of streamlining systems and having clear criteria was stressed.

> *In theory, this looks sound. However, there needs to be clarification on what specific feedback the CDF requires while the companies have time to submit evidence. Where will patients be involved in the appraisal?*
>
> **Female patient, aged over 55**

> *This seems to be a fairer way of assigning categories however the costs of these drugs and the potential benefit does need to be balanced against overall constraints that exist in the NHS.*
>
> M**ember of the public, male, aged over 55**

Of those disagreeing, the view was expressed that this would undercut NICE's bargaining power because it would allow drug companies to maintain anti-competitive prices. Another respondent said that if a drug was deemed cost-effective then it should be approved and if not, then it should not.

Organisations

38% of pharmaceutical companies and industry bodies which responded supported this proposal, with 47% unsure.

> *… is supportive of the proposals' aim to resolve uncertainty but more specificity is required to guide appraisal committees in their decision making around the parameters of uncertainty that need to be considered and broadened. What level of uncertainty would be acceptable? How would the Committee determine whether the uncertainty has the potential to be resolved within additional evidence? What timeframes for data generation would be considered acceptable?*
>
> **Pharmaceutical company**

69% of patient and voluntary organisations agreed with this proposal, with only 6% disagreeing.

> *We agree with this proposal. This proposal could allow greater flexibility as promising treatments that have insufficient evidence to gain a positive NICE recommendation have the opportunity to remain on the CDF whilst additional data are collected on their effectiveness. However …there is a need for greater transparency and patient involvement in the IFR process so that patients are able to understand the basis on which decisions are made.*
>
> **Patient/voluntary organisation**

Similarly NHS organisations showed agreement, with 74% agreeing with the proposal, with 17% unsure.

Healthcare professionals

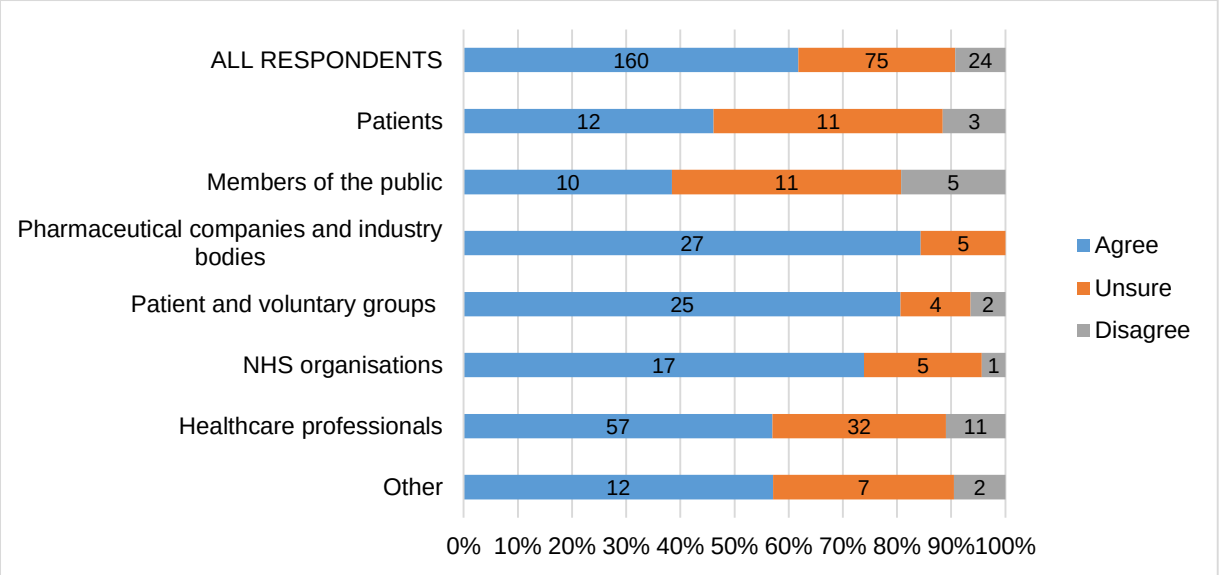
Most healthcare professionals agreed with this proposal (69%). There were some provisos, largely around what criteria would be used in the decision to assign one category or another.

18% of respondents were unsure, mainly because they felt that this option feels ‘half-hearted’ and they had concerns around data collection, evaluation and the criteria used.

> *This categorisation looks reasonable but it has to be clear what drives such a judgement.*
>
> **Male doctor, health advisory group, aged 35–54**

13% of healthcare professionals disagreed, mainly because they felt that cancer should not be treated differently, and/or there should not be a Cancer Drugs Fund. Some of those who disagreed suggested that they would agree, if changes were made.

Question 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?



Patients and the public

Around 46% of patients and 38% of members of the public agreed with the proposal, saying this was 'overdue' and 'statistical common sense' and that everyone should have the same access to treatment, regardless of the type of cancer or its rarity.

The same proportion – about 43% – was unsure for both groups. This was primarily because they felt the question was not clear.

Of those that disagreed, most did not give a reason but one person said the proposal seemed to be based on numbers rather than clinical need or efficacy. Another said more generous access to drugs should be given to those with a rare condition.

Organisations

85% of pharmaceutical companies and industry bodies agreed with this proposal. 16% stated they were unsure, mainly because of a lack of clarity around the question.

81% of patient and voluntary organisations also agreed with the proposal, with 13% saying they were unsure.

74% of NHS organisations agreed with the proposal, with only one organisation who disagreed with the proposal.

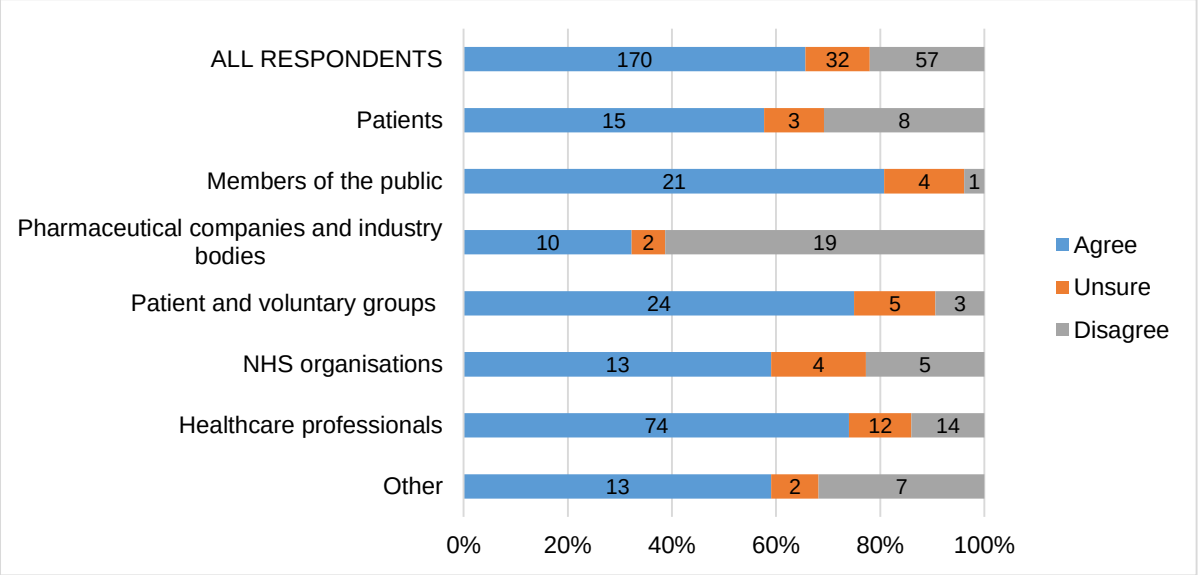
Healthcare professionals

Just over half of healthcare professionals agreed with this proposal (57%), giving as their main reason that they felt there should not be a cap, and that it was 'arbitrary'.

Those that disagreed (5%) had various concerns about changes to the criteria, mostly due to the impact on people with rarer cancers.

Of those who selected unsure some were unclear why that criteria was included in the first place. Others felt some sort of limit was needed, or questioned what would replace the current part of the criteria.

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



Nearly two-thirds agreed with this proposal. However over 60% of pharmaceutical companies and industry bodies disagreed.

Patients and the public

About 58% of patients and 81% of members of the public agreed with this proposal. They said it was common sense and transparent, and would help people to make decisions. Several people caveated this by saying as long as it did not delay the process.

> *It needs to be completed and published very swiftly so that unnecessary delays are avoided.*
>
> **Member of the public, male, over 55**

Eight patients and only one member of the public disagreed with the proposal, because they felt the process would take too long and this would be another hurdle to people receiving life-saving drugs. There was also concern about the implications of approving drugs before they receive marketing authorisation.

Seven were unsure because they did not feel they had sufficient understanding of the effect of this proposal, for example whether it would actually delay treatment.

> *Anything which speeds patient access to medicines is positive. I agree with this proposal as long as it does not mean having to produce data early when it is not ready for appraisal and will not be considered as mature enough to meet NICE's clinical and cost effectiveness ratios. This, rather than improving access, has the potential to deny patients access to very effective new medicines which could save their lives.*
>
> **Male patient, aged 35-54**

Organisations

61% of pharmaceutical companies and industry bodies disagreed with the proposal, citing issues such as:

- how realistic the proposal was e.g. NICE Technology Appraisal process would need to be reformed and this will not be possible in the timeframe; UK pricing is not set until immediately prior to licensing, so how would NICE undertake assessment of cost effectiveness
- inequity e.g. companies and products may be penalised if they do not have the resources to submit early
- inefficiencies e.g. as new data becomes available after the original submission this will need to be inputted.

Those that did agree (32%) generally assumed a new NICE process would be put in place.

75% of patient and voluntary organisations agreed with the proposal, with 16% saying they were unsure.

> *Facilitating earlier access to new, potentially life-saving, drugs is a key strength of the CDF and should continue as part of the new fund.*
>
> **Patient/voluntary organisation**

Healthcare professionals

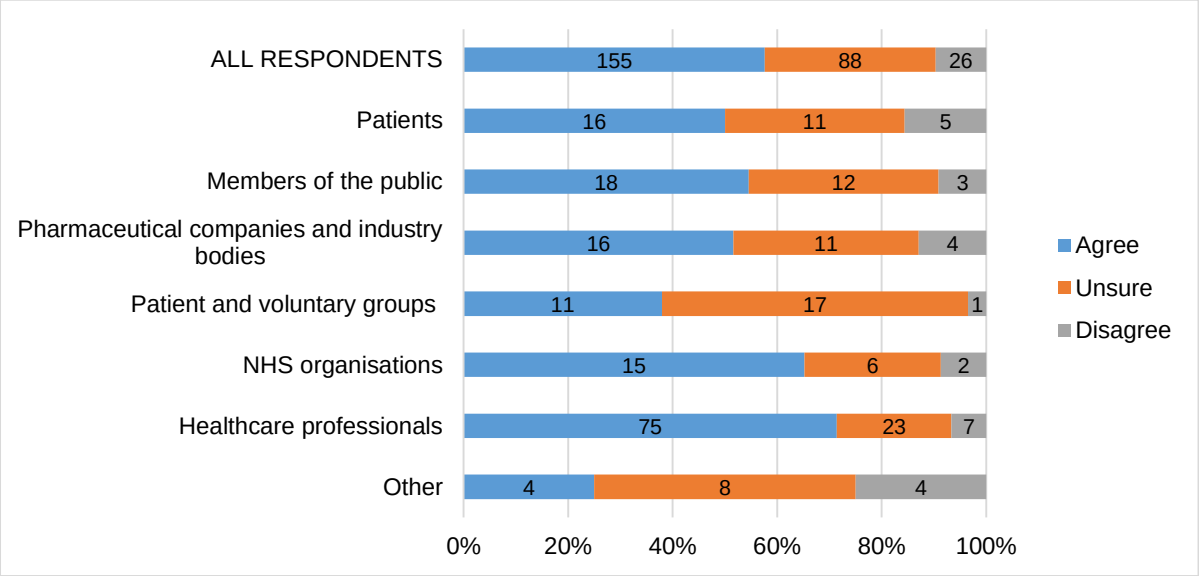
Over half of healthcare professionals agreed with this proposal (59%). Those who disagreed (23%) were mostly concerned about marketing authorisation happening at the right time, and what would happen if a drug did not then receive marketing authorisation.

> *What will NICE do if the drug fails to receive marketing authorisation? Simply not recommend it? In that case all the preliminary work will be wasted.*
>
> **Female doctor, acute trust**

Some of those who were unsure (18%) cited pros and cons of this proposal, or gave provisos.

> *I think the MA should come first but if a drug appears to have solid clear evidence of efficacy then it would be in everyone's interest for a draft proposal to be released prior to MA.*
>
> **Female pharmacist, acute trust**

Question 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?



Over half the respondents agreed with this proposal, with healthcare professionals and NHS organisations showing the highest level of approval. Over 30% of respondents were unsure.

Patients and the public

50% of patients and 55% of members of the public agreed with the proposal, saying this was common sense and anything that speeded up the process had to be good. One person said 90 days seemed reasonable, however another wanted it to be shorter.

Around 16% disagreed (5 people), because they felt that they should be available at the same time as marketing authorisation was given.

Nearly 35% said they were unsure, with concerns being primarily that 90 days was too long. There was also concern about the ability of NICE to cope and make decisions in a timely manner.

Organisations

Just over half of pharmaceutical companies and industry bodies agreed with the proposal, with 36% being unsure.

Where they were unsure of their support or disagreement, the concerns centred around:

- Inequity e.g. there may be instances where companies with limited resources might not be able to support the NICE process or where a global company's headquarters has not set a price. Companies wanted to see a process that did not penalise companies that missed submission at earlier times if submission timelines have been agreed with NICE and NHSE.
- Deliverability e.g. whether NICE will be provided with the capacity to achieve the aim.

38% of patient and voluntary organisation organisations agreed, and 59% said they were unsure about this proposal, highlighting their concern over the ability of NICE to deliver.

NHS organisations showed nearly two-thirds support for the proposal, with a quarter being unsure. Two organisations disagreed.

Healthcare professionals

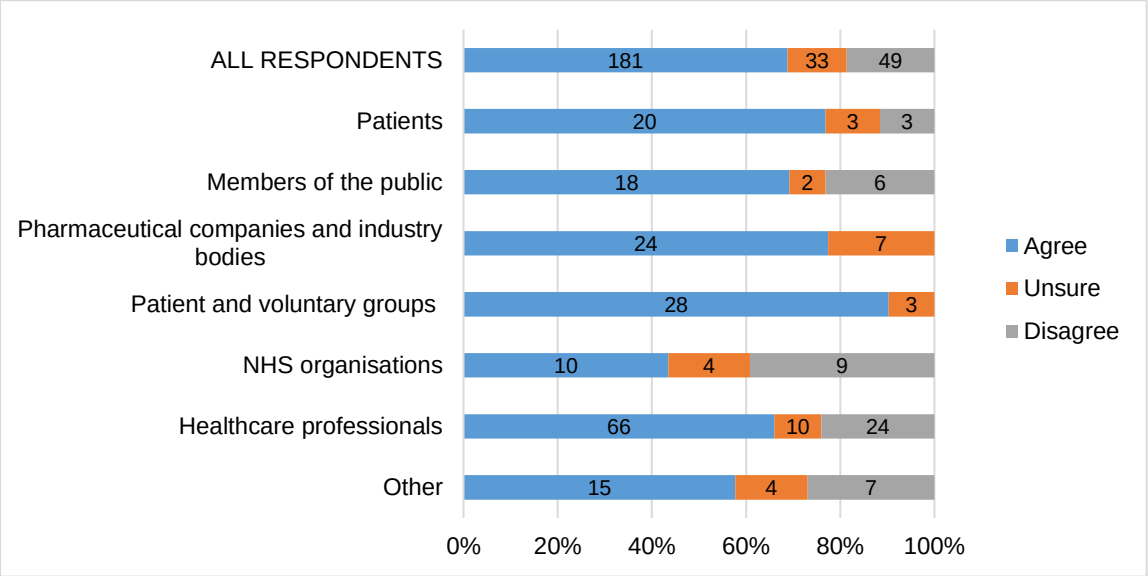
Most healthcare professionals agreed with this process change, though some expressed concern over the impact on NICE and its ability to put the changes in place within 90 days.

> *Early guidance is essential and hence the process needs to change to enable such guidance to be produced. The proposed changes seem sensible on terms of achieving the output of the early guidance.*
>
> **NHS manager, acute trust**

Those who disagreed (7) were generally concerned about the process being rushed, and some asked for flexibility in the time allowed.

Those who were unsure generally felt they needed more information or more detail, or assurances around the decision-making process.

Question 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?



Two-thirds of respondents agreed with this proposal with a high level of support across all categories of respondents, except for NHS organisations where 44% agreed.

Patients and the public

These respondents showed the highest level of agreement in response to this particular question, with 77% of patients and 69% of members of the public agreeing. Where a reason was given, it was that it was important to give access to drug treatment as quickly as possible.

11% and 23% respectively disagreed and several suggested that these drugs could be made available prior to the final appraisal decision but funded by the pharmaceutical company or industry, which would have access to the research data.

Nine percent were unsure, but seemed to think that 90 days was a reasonable length of time.

> *Whilst it is important to get decisions made quickly, it would be unfortunate if a drug that was given interim funding was later refused. Therefore, a wait of maximum 90 days does not seem unreasonable in order to get things right.*
>
> **Male patient, over 55**

Organisations

77% of pharmaceutical companies and industry bodies agreed with this proposal, and patient and voluntary organisation organisations showed the greatest support with 90% agreeing. Nobody from these two categories disagreed with the proposal.

> *We support earlier access to treatment that interim funding would provide for drugs that receive a draft recommendation for routine or conditional use.*
>
> **Patient/voluntary organisation**

> *… supports this concept to ensure that NHSE patients receive access to innovative cancer treatments as soon as they are available.*
>
> **Pharmaceutical company**

44% of NHS organisations agreed and 39% disagreed. Of those that disagreed, concerns raised were about how funding would work during the period.

> *This would result in churning of drugs between a number of different short-term funding streams which would be very difficult to manage in an operational setting. This would be very challenging for both providers and NHS England.*
>
> **NHS acute trust**

Healthcare professionals

The majority of healthcare professionals agreed with this proposal (66%).

Those who agreed generally felt the proposal was 'reasonable'. There were some caveats, and one person wanted to know what happens if the manufacturer does not agree to data collection:

> *The requirement for this needs to be dependent on the receipt of data into the SACT dataset to support this. There does not appear to be an incentive or penalty for supplying the data (or not) to SACT at the moment…*
>
> **Pharmacist, NHS acute trust**

Those who were unsure or disagreed cited a range of reasons, including: difficulties for CCGs and trusts handling complex budgets, and that stopping treatment once it has started would be difficult. Others felt drugs should not be funded on an interim basis and that cancer drugs should not be treated differently to other drugs.

Question 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?

Patients and the public

Generally respondents in this group did not agree with providing interim funding if this would affect funding for other drugs that had already been assessed as worthwhile.

> *It risks funding ineffective treatments, which are a loss to the British taxpayer and should not be entertained, even temporarily.*
>
> **Member of the public, male, aged 35-54**

One person said this could incentivise manufacturers to drag out appraisals that would be negative.

A number of people expressed concern about NICE's ability to make decisions in a timely manner and said that resources should be put into this.

> *I agree, NICE need more people and work faster!*
>
> **Member of the public, female, aged 35-54**

The point was made that drugs should be funded if they were already being used in the EU or the US.

Organisations

There was general disagreement with this proposal because of the perceived negative impact on risk, financial arrangements, patient communications and expectations; and a view that sufficient funding and arrangements should be put in place at NICE to avoid the scenario occurring.

> *Strongly disagree with this approach. In effect it would mean that neither NICE or NHS England would be able to control the CDF spend and the threshold for funding would be lowered. The careful appraisal which the CDF has had to date and is expected from the main consultation proposals would be redundant.*
>
> *If interim funding is given, then a NICE TA process suggests that a drug is not cost effective and should not be funded – either in routine commissioning or the CDF – then invariably there will be political and other pressure to keep the drug in the system.*
>
> **NHS organisation**

Pharmaceutical companies were more supportive of the proposal (but not exclusively so) whilst at the same time calling for a flexible CDF budget. For instance:

> *If a delay in completing the appraisals is a direct result of inadequate resources at NICE then, NHS England should ensure that additional interim funding is granted. This will ensure that patients can continue on treatment and companies are not penalised financially because of NICE delays.*
>
> **Pharmaceutical company**

Healthcare professionals

Healthcare professionals offered a range of views on this scenario.

Those who responded positively felt this was fair for various reasons, including the likelihood of there being solid evidence already and for those with rare cancers. One respondent supported it because the point of marketing is to have a clear time point from which some form of access/appraisal is needed.

Many who responded more negatively felt that a NICE recommendation was needed before funding, with more resources given to NICE to issue draft guidance promptly if necessary.

Other comments included:

- *Drugs could be funded which are later found not to be cost-effective*
- *Funding for drugs that have already been approved by NICE should not be impacted by this process*
- *Concern over starting treatment for a patient and then withdrawing it*
- *Pharma companies should cover the interim drug cost*
- *The availability of funding should be clear and transparent and controlled centrally*
- *There should be a maximum cost for this group of drugs per patient treated*
- *Depends on the reason the guidance has not been produced*
- *Drug companies and NICE should co-ordinate marketing and recommendations simultaneously.*

Question 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?

Patients and the public

About half the respondents in this group did not comment or said that they were insufficiently informed to be able to comment.

Most of those who commented thought that *some* consideration should be given to the CDF taking account of off-label drugs

> *NICE, whether via CDF or routine use decisions, should be able to consider off-label use where requested by clinicians or commissioners. This would be a valid use of the CDF and would be preferable to not progressing such appraisals, although identifying other sources of research funding - such as through NIHR - would be preferable.*
>
> **Member of the public, male 35-54**

Respondents were keen that there should be some evidence of potential benefit:

> *NICE is currently not allowed to assess the use of drugs off-label. This has been a real disadvantage for patients who could benefit from the off-label treatment but could not have the advantage of the level of evidence needed to meet NICE standards on efficacy & adverse effects. There would need to be recourse to a body of clinicians who could recommend that a drug has good potential for off-label use, and then put the drug through the NICE appraisal procedure.*
>
> **Member of the public, female, over 55**

Organisations

This question drew a wide range of responses from those respondents who felt that use of off-label drugs is only going to increase in future and they should be treated in the same (or a similar) way as licensed drugs, through the CDF.

> *existing funding of CDF medicines which are used off-label should continue to be made available but that further consideration of additional off-label treatments should be put on hold prior to the evaluation of all currently licenced cancer medicines being completed. Recognising that off-label usage is important in the oncology treatment setting, and is indeed often a lever to innovation, in the future, NICE and NHSE may wish to consider some off-label medicines being selected for evaluation via, for example, the existing NICE Evidence Summaries for Unlicensed and Off Label Medicines Programme.*
>
> **Pharmaceutical company**

However there was also a belief (primarily of pharmaceutical companies) that, given the capped nature of the fund, off-label drugs should not be CDF funded.

> *There has been a steady stream of ICDFRs for off-label use of the CDF since its inception. Decisions on clinical exceptionality for these requests have been made by regional expert panels with a response time standard of 10 days. The option of putting ICDFRs through the same IFR process as all non-cancer treatments would mean that requests for cancer drugs would be handled in the same way as all other*

treatments, including non-drug treatments for cancer. This is more equitable. However, the response times for ICDFRs and IFRs are discordant.

NHS organisation

Healthcare professionals

Healthcare professionals offered a range of views on this scenario.

Many responded positively. Some felt this would ensure rare cancers get treatment. Some felt NICE needed to be involved. Suggestions for how this could work included:

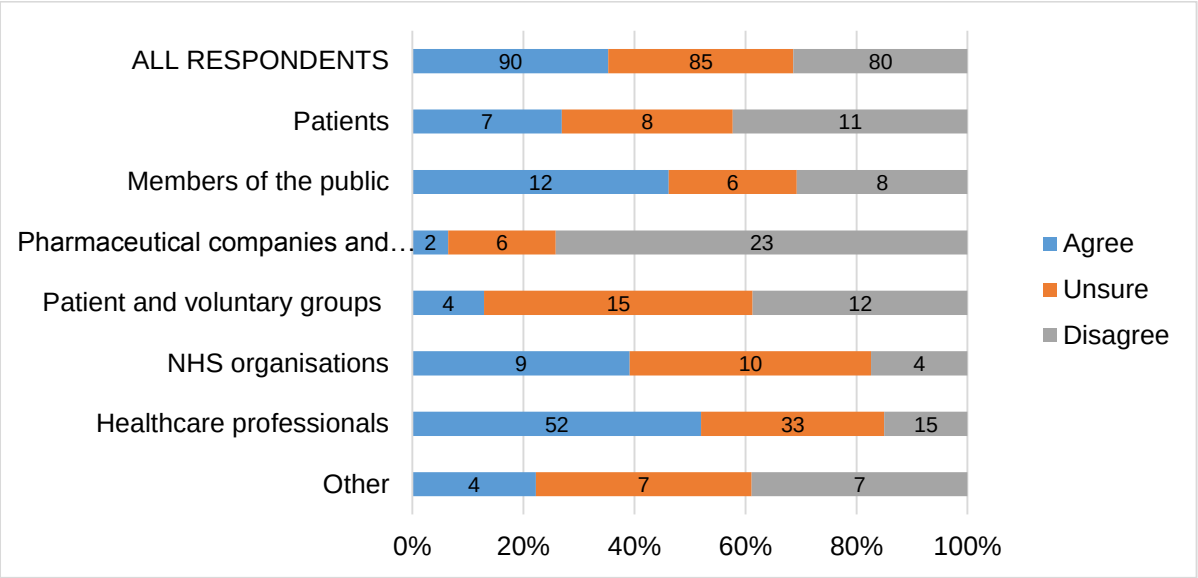
- Clinicians could submit suggestions for indications to be considered and have some sort of prioritisation/voting system
- NICE could commission trials or systematic observational data collection during the period of interim funding, to reduce uncertainty for unlicensed drugs.
- When a 'clinical' body of experience has been built up (using Individual Funding Request) the CDF could then act to collect data over a 24 month period to enable NICE to determine whether a benefit is actually being achieved.
- Create a 'Rarer Cancers Group' within the CDF to evaluate requests for funding of off-label uses of drugs.

Some gave the caveat that there needs to be basic levels of evidence that a drug has some action in a disease.

Others responded more negatively or had concerns. Some felt it would be too complicated. Others felt a separate process was needed, such as assessment by NICE, or by a small panel.

Some said the Cancer Drugs Fund should not be involved at all. Others suggested that off-label decisions should continue to be made as they are now.

Question 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?



Respondents were fairly equivocal about this proposal, with a high degree of uncertainty across most groups.

Patients and the public

27% of patients and 46% of members of the public agreed with this proposal. 42% of patients and 31% of members of the public disagreed.

Of those that agreed with the proposal, respondents stated there must be limits placed on the fund.

> *I think that this is necessary for the fund to operate effectively. My only concern is that it does not impede cancer treatment deemed necessary by the clinician for their patients.*
>
> **Member of the public, male, over 55**

For those expressing their disagreement, respondents thought that it was a mistake to fix the amount in the fund because it should be based on the needs of the population.

> *The drugs should be available on the basis of clinical need and evidence based efficacy only.*
>
> **Member of the public, male, over 55**

Those who were not sure stated that they could see both sides, but were concerned about patient care:

> *I do agree that the budget needs fixing and that there is a contingency; however, there has to be flexibility in how drugs are assessed within the fund with greater emphasis being given to expertise of consultants' knowledge of their patients.*
>
> **Female patient, over 55**

Organisations

22 out of the 23 pharmaceutical companies that responded to this question disagreed with this proposal with one responding as 'unsure'. There was a high degree of uncertainty across most groups. However 'NHS' aligned bodies were generally in favour of fixing the budget (although some NHS Trusts questioned how the system would practically work).

Disagreement from pharmaceutical companies centred around:

- Calendar funding restrictions e.g. penalisation of products brought to market in a busy year or towards the end of the year when budget has run out.
- The financial risk to pharmaceutical companies
- The likelihood that this might mean companies view the UK as too challenging an environment in which to launch a product.

> *rather than a complete payback by the company, this rebate should be weighted and based on the difference in price based on an agreement of the incremental c-e ratio compared to BSC, as some value may have been gained for some patients (observed in registries and trials). Ultimately some benefit for patients must exist for the treatment otherwise there would be no grounds for granting a license in the first place.*
>
> **Pharmaceutical company**

NHS and other organisations were concerned about how technically the process would work

> *The system that is proposed in this consultation appears very complicated, and will be complex for pharmacy departments to administer. Perhaps a better system is for NHSE to be responsible for paying the manufacturers directly, depending on which particular scheme the patient is receiving the drug. As long as the patient has been registered appropriately, there is only 1 organisation having to deal directly with the manufacturer, which will be far simpler.*
>
> **NHS acute trust**

> *The principles are sound as the budget needs to be managed, but the mechanism for these needs to be clarified. How can NHS England/CDF freeze what it pays to manufacturers if Trusts have already paid manufacturer? How will the invoicing be managed, experience has shown that invoicing old cancer drug fund is complex and needs regular local scrutiny. There are problems with reclaiming VAT and use of third party dispensing. There are risks with use of PAS schemes which may not realise expected benefits and are difficult to manage and track.*
>
> **NHS acute trust**

Healthcare professionals

Over half of all healthcare professionals agreed and of those, many cited the need to remain within an allocated budget and to ensure it is spent wisely.

> *This has to happen. It is unfair that patients with cancer get special treatment over other NHS patients.*
>
> **Doctor, NHS acute trust**

However some felt it could be difficult to maintain, and felt there was a risk of overspend.

Many of those who disagreed or felt unsure had concerns over a fixed budget and how control mechanisms would work in practice.

Question 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?

Patients and the public

About half the respondents in this group did not comment or said they did not know. Almost a quarter explicitly expressed support.

Of the small number of people who disagreed, this was on the grounds that cost should not be a factor.

There was support for removing any decisions from the political arena. There was also support for ensuring transparency with suggestions that companies needed to be transparent about development costs and proposed return on investment and that information from all trials would need to be published before approval could be given.

Organisations

Overall, organisations felt they needed to understand the operational detail. There was some belief that the proposals were an improvement on the current system. But 74% of pharmaceutical companies and industry bodies disagreed with the proposal, while appreciating that it is not viable to have a limitless fund. They cited in particular the unknown variable of the number of potential cancer medicine launches in any one year.

Healthcare professionals

52% of healthcare professionals agreed with the proposal, with some feeling the investment control arrangements were appropriate. Others felt they were not, for various reasons such as a perception that it over complicates the control mechanism.

Alternative mechanisms included:

- *Offer funding for a fixed number of cycles of treatment then apply for extension of treatment funding*
- *Use NICE, and their standard appraisal procedures*
- *Rigorous audit of clinical progress of cases accepted for CDF funding*
- *Price cap arrangements as per current PPRS*
- *Manufacturers to supply CDF drugs to be supplied to Trusts at zero cost, under a managed access scheme, with tracking patients*
- *The NHS receives shares in the marketing company in response to an agreement and invests profits into prevention e.g. smoking cessation*
- *Value based pricing should be employed based on QALY*
- *Negotiation with companies for drugs that are out of the boundaries set by either budget or NICE, could be provided in a discount price to make them financially friendly for the organisation. Also, if drugs are used for more (new) indications, the logical process should be to force companies to reduce the price (as the market will be bigger).*

There were also a number of comments about the need for transparency. One respondent felt the arrangements would undermine the subsequent NICE process. Another felt it was inappropriate to consult on these matters, and the cost/benefit should be decided by NICE.

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

Patients and the public

Most respondents in this group raised other issues. Several people stressed the importance of ensuring that decisions were evidence-based and would meet the population's needs. The importance of engaging more widely with the public and patients was also mentioned by a number of people, as was the importance of handling data properly and ensuring IT systems could communicate with each other.

Other issues raised were:

- The need for an appeals process
- Ensuring there were systems to evaluate clinical and cost effectiveness
- Providing more clarity about the relationship between the NHS, NICE, the CDF and the government
- That rarer cancers should not be ignored
- The fund should be widened to include conditions other than cancer.

Organisations

Respondents from organisations felt there was a need to look at:

- Impact on NHS trusts e.g invoicing
- Evidence gathering and data collection issues e.g. the funding of this
- Patient information about how the system works
- Ethical issues e.g. how the fund could be broadened to include all innovative medicines
- Transition arrangements and reviews of drugs previously removed from the CDF
- Timing of any changes
- Changes to the NICE technology appraisal process to assess the impact of proposed CDF changes
- How the CDF could consider a more holistic view rather than just cost-effectiveness
- The recruitment and resourcing of the CDF Investment Group and NICE Technology Appraisal Committee and patient involvement
- Alignment with the Accelerated Access Review (AAR)
- Quality of, and interrelationship with the SACT dataset and with the IFR process
- The consultation process e.g. the lack of a patient friendly guide.

Some pharmaceutical companies said they did not support the proposal that the CDF should only fund the minimum number of patients required to generate the data needed for further NICE review, and for companies to pay for all other NHS patients.

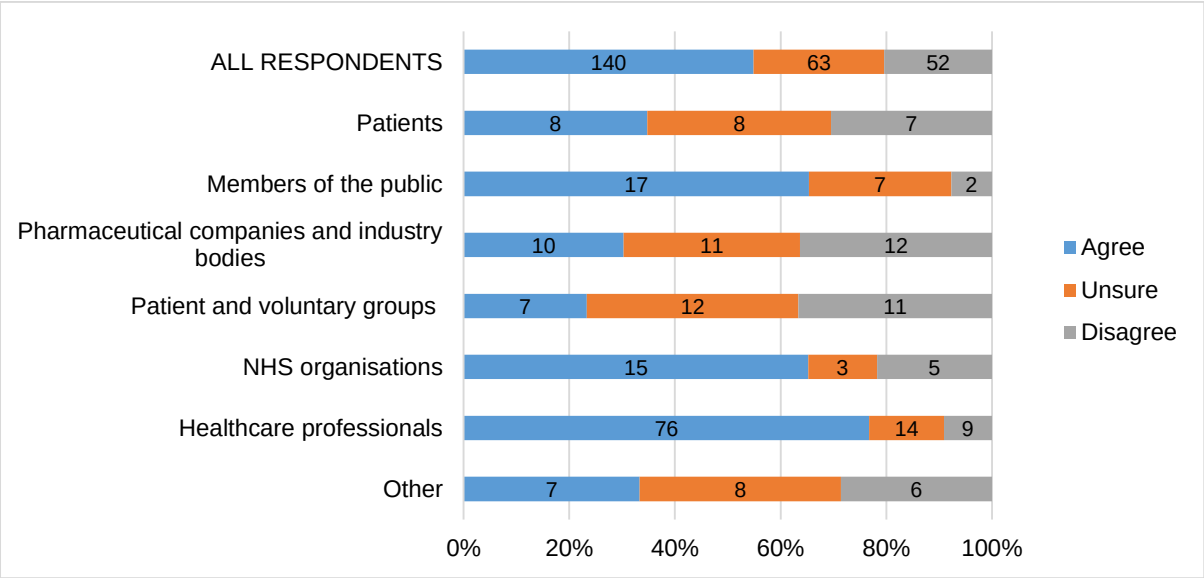
Healthcare professionals

Key issues stated by healthcare professionals included:

- The high-profile of the CDF and some cancer treatments; 'it seems that those who shout loudest will be listened to'. Patient expectations need to be managed better
- The process for dealing with rare and ultra-rare cancers needs to be better
- More onus needed on clinician to provide information on effectiveness of drugs used within CDF

- Appropriate realistic reference data is needed for end of life care that can be applied systematically across all appraisals
- The approval of drugs for the CDF for a period of 24 months only may not be sufficient to generate new data. We need to specify what type of data is acceptable and provide tools to have the data available.
- Rather than just seeking support for applications, perhaps there should be arguments against applications as part of the process
- Declarations of all negative data/trials associated with the product, as well as positive
- It should be easy to access the fund; simple on-line applications and rapid decisions

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



On balance, respondents do agree that the new CDF arrangements are preferable to existing arrangements. However, nearly a quarter said they were unsure, citing lack of detail and unease over future financial sustainability and system bureaucracy.

Patients and the public

Respondents in this group were split about whether the new arrangements would be better than existing arrangements, with no majority opinion: 35% agreed they would be better, 30% disagreed with the remained unsure.

In agreeing, respondents felt that the proposed system would be more sustainable and less political. It would deal with current inequity within the system.

> *It seems to me that the current mechanism consists of an extra fund for drugs that have essentially a poor cost/benefit ratio and are not approved by NICE for general use but are then simply funded from another source which is also not (and never can be) bottomless.*
>
> **Member of the public, male, over 55**

Reasons for disagreeing included that this would be returning to the pre-CDF system and that it would discriminate against some cancers.

Those who were unsure thought there would be positive and negative impacts.

> *Bringing down costs is good, making new drugs available is good, limiting the application of those drugs by budget is not.*
>
> **Patient, male, over 55**

Organisations

Pharmaceutical companies and industry bodies also showed a split across the three options, with 30% agreeing, 36% disagreeing and the remainder unsure.

> *Whilst this is likely to create a few short-term problems in the transition, it is the right approach to address the long-term affordability of cancer care.*
>
> **Professional body**

Of those patient and voluntary organisation that disagreed (37%), some felt that there was not enough detail in the proposal; others felt that key enablers were not in place e.g. reform of NICE.

Healthcare professionals

77% of healthcare professionals agreed that the new CDF arrangements are preferable to the current ones.

> *The existing system is not satisfactory, and is also not sufficiently transparent. Taking drugs off the CDF has been fraught because of the lack of clear, robust criteria. This proposal should be a significant improvement.*
>
> **Pharmacist, NHS acute trust**

Those who disagreed (9%) expressed a range of concerns including; the new arrangements will mean fewer available treatments; a more holistic approach is needed, including spending money on surgery and radiotherapy; there are risks around the flow of data from providers; there is a lack of mention of PASLU, IFRs and managing combinations of new expensive drugs. Others felt more clarity was needed in various areas, such as who will set criteria for use of drugs.

Most of the people who were unsure felt it was too early to answer this question, or had concerns about the existence of the CDF.

Appendix A – Demographic information

The demographic information below relates to individuals who completed the questionnaire, as those who sent in letters or emails did not give us these details about themselves. Percentages are given after the actual numbers. Where these do not total 108 (100%), the remainder are those who did not respond or preferred not to say.

Gender of respondent

	Number	% of overall total
Female	93	35.23%
Male	116	43.94%
Neither	3	1.14%
Prefer not to say	35	13.26%
Not answered	17	6.44%
Total	**264**	

Age of respondent

	Number	% of overall total
Under 18	1	0.38%
18-24	2	0.76%
25-34	25	9.47%
35-54	112	42.42%
Over 55	71	26.89%
Prefer not to say	36	13.64%
Not answered	17	6.44%
Total	**264**	

Sexual orientation of respondent

	Number	% of overall total
Bisexual	1	0.38%
Gay man	2	0.76%
Heterosexual/ straight	183	69.32%
Prefer not to say	58	21.97%
Not answered	20	7.58%
Total	**264**	

Ethnic group

	Number	% of overall total
African	1	0.38%
Bangladeshi	1	0.38%
British	164	62.12%
Chinese	2	0.76%
Indian	8	3.03%
Irish	3	1.14%
Pakistani	2	0.76%
White and Asian	4	1.52%
White and Black Carribean	1	0.38%
Other	17	6.44%
Do not wish to disclose	42	15.91%
Not answered	19	7.20%
Total	**264**	

Religion

	Number	% of overall total
Buddhist	1	0.38%
Christian	89	33.71%
Hindu	2	0.76%
Muslim	5	1.89%
None	87	32.95%
Other	6	2.27%
Prefer not to say	49	18.56%
Not answered	25	9.47%
Total	**264**	

Person with disability

	Number	% of overall total
No	193	73.11%
Yes	16	6.06%
Prefer not to say	37	14.02%
Not answered	18	6.82%
Total	**264**	

NHS England

NICE National Institute for Health and Care Excellence

CONSULTATION ON PROPOSALS FOR A NEW CANCER DRUGS FUND (CDF) OPERATING MODEL FROM 1ST APRIL 2016

NHS England INFORMATION READER BOX		
Directorate		
Medical	Commissioning Operations	Patients and Information
Nursing	Trans. & Corp. Ops.	Commissioning Strategy
Finance		

Publications Gateway Reference:	**03873**
Document Purpose	Consultations
Document Name	CONSULTATION ON PROPOSALS FOR A NEW CANCER DRUGS FUND (CDF) OPERATING MODEL FROM 1ST APRIL 2016
Author	NHS England/NICE
Publication Date	19 November 2015
Target Audience	All NHS organisations, Patients and the Public, Department of Health, Other Government Departments, Cancer Charities, Members of the Pharmaceutical Industry
Additional Circulation List	Any additional interested stakeholders who wish to respond to the consultation
Description	Arrangements for the current Fund are due to end on 31st March 2016. Consulting now allows sufficient time for responses to be analysed, for an orderly transition from the existing scheme to be implemented and for the new CDF to be operational from 1st April 2016. Following this consultation, All feedback received will be reviewed and a report published on the NHS England website.
Cross Reference	N/A
Superseded Docs (if applicable)	N/A
Action Required	Response to consultation
Timing / Deadlines (if applicable)	**Responses to arrive no later than midnight on 11 February 2016**
Contact Details for further information	Clinical and Scientific Policy and Strategy Team NHS England, Medical Directorate 5W06 Quarry House Quarry Hill LS2 7UE 0113 825 1125 england.futurecdfconsultation@nhs.net

Document Status

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Promoting equality and addressing health inequalities are at the heart of NHS England's values. Throughout the development of the policies and processes cited in this document, we have:

- Given due regard to the need to eliminate discrimination, harassment and victimisation, to advance equality of opportunity, and to foster good relations between people who share a relevant protected characteristic (as cited under the Equality Act 2010) and those who do not share it; and

- Given regard to the need to reduce inequalities between patients in access to, and outcomes from healthcare services and to ensure services are provided in an integrated way where this might reduce health inequalities

CONTENTS

HOW CAN I MAKE MY VIEWS KNOWN?

NHS England and NICE are opening a public consultation for twelve weeks from 19th November 2015 until midnight on 11th February 2016. This is in line with Cabinet Office guidance on consultations. Comments must be received by midnight on 11th February 2016 to be considered.

You can respond to this consultation in one of the following ways:

- Complete the online consultation at **www.engage.england.nhs.uk**
- Download and print a copy of the consultation response form at **www.engage.england.nhs.uk** Send your responses to Clinical and Scientific Policy and Strategy Team, NHS England, 5W06, Quarry House, Leeds, LS2 7UE
- Alternatively, you may request a copy of the consultation response form to be posted to you. Please contact: **england.futureCDFconsultation@nhs.net**

This summary document can also be requested in alternative formats, such as easy read, large print and audio. Please contact: **england.futureCDFconsultation@nhs.net**

Any general queries relating to the consultation should be sent to: **england.futureCDFconsultation@nhs.net**

We would like to hear from anyone with an interest in the subject matter of the consultation. We are committed to involving patients, and potential future patients, in the planning and consideration of the future sustainability of the CDF, and we are particularly keen to hear from as many patients, carers and patient representatives as possible to inform decisions on proposals concerning the fund.

Responses will be public documents and all, or any part, of a response may be put in the public domain. If you wish to refer to any confidential information in your response, it must be included in a separate document which is very clearly marked as confidential on each page. NHS England and NICE are subject to the Freedom of Information Act. While they would seek to respect the confidentiality of any information provided to them, respondents should be aware that they may be obliged to release even confidential information under that Act. Please try not to include sensitive personal data in a response unless you feel this is absolutely essential to the point you are making.

Any comments that relate to services or issues outside of the scope of this consultation will be noted and passed on accordingly.

POST-CONSULTATION

Following this consultation, NHS England and NICE will review all relevant feedback received. Due to the likely volume of responses, feedback is likely to be presented in the form of a report capturing all material issues. The report will be published on the NHS England and NICE websites.

Due to the likely number of responses to this consultation, NHS England and NICE will not be able to provide individual replies to any submissions.

QUESTIONS

There are a total of 24 questions to answer. These are included at **Appendix C**.

There are 14 consultation questions, and a further 10 questions regarding information about you or your organisation.

Please tick one box only per question (Questions 15 – 24)

If you require more space than provided for your comments, please continue on a separate sheet, clearly referencing the question number.

INTRODUCTION AND CONTEXT

1. The Cancer Drugs Fund (CDF or Fund) was developed by the Government to improve access to treatment for patients. Since its inception in 2010, it has provided access to treatment for more than 72,000 patients whose individual circumstances suggest that they will benefit from drugs that have not been adopted for routine use in the NHS. This includes drugs which have not been recommended by the National Institute for Health and Care Excellence (NICE), those used for rare cancers not selected for NICE appraisal, or those which are being used for unlicensed indications.

2. NHS England is committed to ensuring quick and effective access for patients to newer and better drugs. The proposals in this consultation are aimed at delivering this through a new clinical assessment framework. We need to ensure that the right patients gain access to better drugs, within a process which is fully aligned with the evolving health and care system and which can rise to the challenge faced by all advanced economies in dealing in an affordable way with the increasing pace of biomedical discovery of new, targeted and often expensive drugs.

3. The CDF was originally intended to be a bridge to a new approach to the adoption of new drugs into the NHS, using 'value based pricing'. As circumstances changed and alternative methodologies were explored, this approach was not, in the end, adopted. One of the barriers to the routine commissioning of some cancer drugs is the uncertainty about their clinical benefit, and therefore their cost effectiveness, at the time they are licensed. Under the current arrangements, although the CDF provides the means for temporary funding to be made available, there is no process through which the NHS can resolve some or all of that uncertainty through a systematic approach to collecting relevant data. Resolving uncertainty is essential to enable a clear decision to be taken about whether to make a new drug available routinely or to restrict its use to individual patient requests.

4. The budget for the CDF was initially set at £200m; however, this has been increased twice, most recently to £340m for 2015/16. The budget for 2015/16 will not be affected by the arrangements proposed in this consultation. The CDF has increased access to cancer drugs; however, as the Independent Cancer Taskforce's report ("*Achieving World Class Cancer Outcomes: A Strategy for England 2015-2020")* noted, the current arrangements are not designed to reduce uncertainty about the benefits of new treatments or to make a decision about their long term use. As a result, the NHS in England is currently allocating an increasing share of the cancer budget to treatments of uncertain value, and the impact of this is being felt in other cancer services and in other parts of the NHS.

5. The CDF was considered by the Independent Cancer Taskforce, which was chaired by the chief executive of Cancer Research UK and drew on expertise from clinicians and patient groups. The Taskforce made the following recommendations on the CDF, which we propose to accept:

"Section 5.3.3.1 Access to innovative drugs

The Cancer Drugs Fund has helped more than 72,000 cancer patients in England access the drugs their doctors think they need in the absence of NICE approval. It has enabled pull through of innovative drugs into routine NHS use. However, because it has also enabled some pharmaceutical companies to bypass NICE cost-effectiveness assessments, it is widely acknowledged that it is no longer sustainable or desirable for the Cancer Drugs Fund to continue in its current form. In its place a solution is needed that ensures patients have routine access to a greater range of cancer drugs, including earlier access to innovative drugs, while ensuring that cost-effectiveness is maintained. A process is under way to find such a solution and it is anticipated that this will be agreed by summer 2015. Part of the solution will continue to be a national fund to make new cancer treatments available prior to NICE assessment or which are subject to a conditional approval."

And:

"Recommendation 31: NHS England should work with NICE, the Government, the pharmaceutical industry and cancer charities to define a sustainable solution for access to new cancer drugs. This updated process should enable NHS England to confirm clinical utility, whilst managing within a defined budget, and should be aligned with NICE appraisal processes. The new process should be published for consultation in summer 2015, with a view to implementation from April 2016. The solution should set out reforms to NICE processes to make them more flexible for cancer drugs."

6. The arrangements for the current CDF are due to end in March 2016. In light of this, and the increasing budgetary pressure on the CDF, the NHS England Board requested that proposals be developed for a new CDF operating model, to be introduced from April 2016 following a public consultation. The proposals set out in this consultation document are consistent with the recommendations in the Independent Cancer Taskforce report.

7. These proposals will provide access to medicines while data is collected to inform a decision on whether to adopt the drugs for routine commissioning. It provides the means for selected cancer drugs with apparent clinical promise but uncertain value to move into and out of the CDF, which will become a transitional fund to facilitate patient access with tightly focussed research and a fixed cost for the NHS, aimed at securing the best outcomes for patients.

8. It should be noted that an independent review of access to innovative treatments (the Accelerated Access Review or AAR) is currently underway. The aim of this review is to identify options for speeding up access to transformative innovative drugs, devices and diagnostics for NHS patients. The review is considering the long term landscape for innovation adoption. The proposals for the new CDF are consistent with the emerging conclusions of the AAR.

WHY ARE WE CONSULTING?

9. Arrangements for the current Fund are due to end on 31st March 2016. Consulting now allows sufficient time for responses to be analysed, and for the new CDF to be operational from 1st April 2016, with a target to complete the full transition by the end of March 2017.

AN OVERVIEW OF THE PROPOSED NEW CDF PROCESS

10. The proposal is that the CDF should become a 'managed access' fund for new cancer drugs, with clear entry and exit criteria. It would be used to enable access to those drugs which appear promising but where NICE indicates that there is insufficient evidence to support a recommendation for routine commissioning. These drugs would be given a conditional recommendation by NICE and their use enabled by the CDF for a pre-determined period whilst further evidence is collected. At the end of this period the drug would go through a short NICE appraisal, using this additional evidence. It would attract either a NICE positive recommendation, at which point it would move out of the CDF into routine commissioning, or a NICE negative recommendation, at which point it would move out of the CDF and become available only on the basis of individual patient funding requests. This approach will enable the money in the CDF to be more effectively managed, as well as providing a new pathway for innovative drugs to be assessed and made available to patients.

11. The key features of the proposed new model are as follows:

- NICE will appraise all cancer drugs that are expected to receive a Marketing Authorisation
- NICE will normally issue draft guidance prior to Marketing Authorisation
- NICE will normally publish their final guidance within 90 days of Marketing Authorisation
- NICE will make a recommendation falling into one of 3 categories:
  - Recommended for routine use
  - Not recommended for routine use
  - Recommended for use within the Cancer Drugs Fund
- At the point of Marketing Authorisation, all drugs with a draft recommendation for routine use, or a draft recommendation for conditional use within the CDF will receive interim funding from the CDF budget
- An additional option would be to provide interim funding for drugs or indications that NICE has not been able to produce an interim recommendation for at the time of Market Authorisation. This would have consequences for continuity of care and the use of funds in the CDF, should NICE issue negative draft guidance.

12. A summary of key decision making points in the proposed process is included at **Appendix A**.

13. NHS England will retain overall legal responsibility for, and governance of, the CDF, given it is responsible for the NHS budget and commissioning the use of cancer drugs. The impetus for this consultation is the need to ensure the CDF budget is used effectively and that those drugs that have demonstrated their clinical and cost

effectiveness become available through routine commissioning. NICE has the technical expertise and capacity to undertake the assessment of the benefit to be gained from these drugs, and to advise on when more data is necessary to reach a definitive view on their clinical and cost effectiveness. NHS England will therefore ask NICE to identify those drugs which are appropriate for time-limited funding under the CDF. The recommendations of the NICE Appraisal Committees for drugs they consider suitable for entry into the Cancer Drugs Fund will be received by a joint committee of NHS England and NICE (the Cancer Drugs Fund Investment Group), which will be responsible and accountable for confirming that an acceptable commercial access arrangement (the financial arrangements which determine the cost of the drug to the NHS, agreed between the company and NHS England) and data collection arrangements, which together form the managed access agreement, are in place before accepting the drugs into the Fund.

BENEFITS OF THE NEW ARRANGEMENTS

14. Patients will benefit from access to treatments for which there are insufficient data to support routine use but which nevertheless may represent a significant improvement on the treatment they are currently receiving.

15. The NHS will benefit from a careful process which will select only those drugs for which there is reason to believe that additional data collected, either through the CDF or from clinical studies already underway, will provide the basis for a clear decision as to whether a drug is clinically and cost effective and thus whether to move it into routine commissioning or not. Given the increasing overspend under the current arrangements, mechanisms are described in this consultation to contain the cost of the new CDF arrangements in line with the 2014 Pharmaceutical Price Regulation Scheme between the Government and the Association of the British Pharmaceutical Industry.

16. Pharmaceutical companies will benefit from a transparent and contestable process, managed by NICE, which will make clear the basis on which their products will be selected for use in the NHS, including the circumstances in which they may be eligible for time limited access to funding through the CDF.

THE NEW CDF PROCESS – PROPOSALS FOR CONSULTATION

Topic Selection and Appraisal Timescales

17. The Cancer Reform Strategy, published in 2007, stated that '*in future the default position for all new cancer drugs and significant new licensed indications will be that they will be referred to NICE, providing that NICE agrees that there is a sufficient patient population and an evidence base on which to carry out an appraisal and that there is not a more appropriate alternative mechanism for appraisal*'. Since then NICE has appraised virtually all new cancer drugs, excluding a small number (three to four each year), usually because of small population size.

18. From April 2016, it is proposed that all new cancer drugs and significant new licensed cancer indications will be referred to NICE for appraisal. Extending NICE's remit across all cancer drugs will make the scheduling of drugs for appraisal quicker and more efficient. It is also proposed that all cancer drugs will receive draft guidance from NICE before marketing authorisation, and final guidance within 90 days of marketing authorisation being granted (subject to appeals).

19. Cancer drugs that go through the normal European Medicines Agency licensing process will be scheduled into the NICE work programme such that final guidance can normally be produced within 90 days of marketing authorisation. In order to achieve this, the first Appraisal Committee meeting will be held before an opinion of the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has been published. The NICE Appraisal Committee will meet in private at this stage, as no public regulatory decision will have been made. An Appraisal Consultation Document (ACD) or Final Appraisal Determination (FAD) will only be released when the CHMP has published a positive opinion. Where a second Committee meeting is needed (when an ACD has been issued), it will be held when the product has received its Marketing Authorisation.

20. For NICE to publish guidance within 90 days of marketing authorisation, companies must provide their best estimate for the date of the expected CHMP opinion to *UK PharmaScan* and to the NICE Topic Selection and scheduling teams. Companies will be invited to submit evidence for clinical and cost effectiveness of their products to NICE at the same time as they submit to the European Medicines Agency. Evidence submissions will have to include all evidence the company intends to submit to NICE, including any patient access scheme.

21. NICE will, in each case, provide the company a date by which they will be required to provide their evidence submission to NICE in order for a draft recommendation to be available at the point of marketing authorisation. If companies do not provide their evidence submission by this date, it will not normally be possible to ensure a draft recommendation is available at the point of marketing authorisation which, in turn, may impact on the provision of interim funding (see paragraphs 35 - 38) and the ability to issue a final decision within 90 days of marketing authorisation.

Appraisal of Drugs for entry into to the CDF

22. The NICE technology appraisal process, appropriately modified, will be used to evaluate all new licensed cancer drugs and significant licence extensions for existing drugs.

23. The outcome of a NICE appraisal for a cancer drug will be a recommendation falling into one of the following three categories:

    - **Recommended for routine use** and funded from the baseline commissioning budget (a drug which thus demonstrates both clinical and cost effectiveness)

- **Not recommended for routine use** and thus there is no baseline funding (a drug which thus does not demonstrate both clinical and cost effectiveness)

- **Recommended for use within the Cancer Drugs Fund** for evaluation within a pre-determined period of time and on the basis of an estimated number of patients required to be treated in England in order to gain further evidence to address the uncertainty in the key outcomes which determine whether a drug is clinically and cost effective.

24. All access to the CDF will use the same pricing control mechanism.

25. *Recommended for routine use* would require the incremental cost effectiveness ratio to fall within the standard NICE range (£20,000 to £30,000 per QALY gained), taking into account the application of the End of Life criteria where appropriate.

26. *Recommended for use within the Cancer Drugs Fund* would require the drug to display plausible potential for satisfying the criteria for routine use, taking into account the application of the End of Life criteria where appropriate. Entry into the CDF would be subject to the company agreeing to fund the collection of a pre-determined data set, during a period normally lasting no longer than 24 months, and a commercial access arrangement which is affordable within the available CDF budget.

27. *Not recommended for routine use* would indicate that the drug is unable to satisfy either of the first two conditions.

End of Life Criteria

28. As is currently the case, it is proposed that the appraisal of certain cancer drugs will be modified by the application of the 'End of Life' criteria, which recognise the particular features of drugs designed to extend life, at the end of life. The current End of Life criteria are as follows:

    - The treatment is indicated for patients with a short life expectancy, **normally** less than 24 months; and

    - There is sufficient evidence to indicate that the treatment offers an extension to life, **normally** of at least an additional 3 months, compared with current NHS treatment; and

    - The technology is licensed, or otherwise indicated, for small patient populations **normally** not exceeding a cumulative total of 7000 for all licensed indications in England.

Proposed changes to NICE's End of Life criteria

29. In order to allow for uncertainty in the clinical benefit of cancer drugs with incremental cost effective ratios in excess of NICE's standard range (£20,000 to £30,000 per QALY gained) to be explored in the context of recommendation for use within the Cancer Drugs

Fund, NICE proposes to make the following changes to the End of life criteria (see **Appendix B, Technology Appraisal Methods, section 6** for further details):

- Removing the restriction of the cumulative patient population from the current End of Life criteria to recognise that it has been rarely engaged; and

- Amendments to emphasise the discretion that exists for NICE Appraisal Committees to interpret the uncertainty criteria when considering a drug for inclusion in the Cancer Drugs Fund.

Determining Recommendations for use within the Cancer Drugs Fund

30. When the evidence for the clinical and cost effectiveness of a drug has been assessed, including, where appropriate, the application of the End of Life criteria, the NICE Appraisal Committee will decide whether the drug can be recommended for routine use or not.

31. If the NICE Appraisal Committee cannot recommend a drug for routine use, it will consider whether the drug is eligible for recommendation for use within the Cancer Drugs Fund. The Appraisal Committee will apply its usual technology appraisal methods and process, subject to the amendments set out in **Appendix B**. To inform this decision, the Committee will take into account the following factors:

    - Whether the incremental cost effectiveness ratio considered has the potential to lie within the thresholds specified in the NICE technology appraisal methods; and

    - The extent and nature of the uncertainty in the clinical effectiveness of the drug; and

    - The likelihood that the timeframe for data collection (including research already underway) will be able to inform a subsequent NICE appraisal, normally within 24 months.

32. Drugs whose potential range of cost per QALY does not include £30,000, taking into account any QALY weight applied in line with the End of Life criteria where appropriate, will not be accepted into the CDF.

33. The duration for which each drug is to remain in the CDF will be determined at the point at which it enters the Fund. This will depend on the arrangements agreed for the data collection exercise and will normally be for a period up to 24 months. An interim review of the data collected will, where appropriate, be conducted, which may accelerate earlier transition of a drug through the Fund, where sufficient data has been collected before the predetermined end date. At the end of the data collection period, NICE will undertake a review of its original recommendation and will issue either a 'recommended' or 'not recommended' for routine use decision. This review will be undertaken through a short technology appraisal process which will normally take either 17 weeks, if the Appraisal Committee recommends that the drug should move into routine commissioning, in which case there will be no public consultation, or 26 weeks, if it does not and public consultation is therefore required. (See Figure a, Appendix B). The review will take into account only those data which have become available since the original appraisal,

together with any change to the commercial access arrangement proposed by the company.

34. The data collection specification, process and funding, forming the managed access agreement, will need to be clearly identified before a drug enters the fund, and the protocols and resources will need to be in place to manage it. The company will be required to agree to these arrangements before its drug can enter the CDF.

Interim Funding

35. It is proposed that all drugs that receive a draft recommendation for routine use from NICE will receive interim funding (out of the CDF budget) from the point of marketing authorisation. Normally within 90 days of marketing authorisation, final NICE guidance will then determine whether funding moves into baseline commissioning (recommended for routine use), stops altogether except for individual funding requests (not recommended), or is funded for use within the CDF.

36. Furthermore, it is proposed that all drugs that receive a draft recommendation for conditional use within the CDF from NICE will also receive interim funding from the point of marketing authorisation. Normally within 90 days of marketing authorisation, final NICE guidance will then determine whether funding moves into baseline commissioning, stops altogether except for individual funding requests (not recommended), or is funded for use within the CDF.

37. Drugs that are not recommended in draft NICE guidance will not receive interim funding.

38. A variant of this approach could be to provide interim funding for any new cancer drug or indication where the manufacturer has submitted the necessary information to NICE on a timely and comprehensive basis (including in accordance with paragraphs 20 and 21) but where NICE has not been able to make an interim decision at the point of marketing authorisation. This interim funding might continue until such time as NICE is able to issue draft guidance at which time the arrangements in paragraphs 35, 36 and 37 would apply, with draft recommendations for ‘routine use’ or ‘conditional use’ enabling the continuation of CDF funding until final appraisal, and draft ‘not recommended’ drugs or indications ceasing to be funded by the CDF. The disadvantage of this approach would be the potential provision of interim funding for drugs that subsequently receive a draft 'not recommended for routine use' decision from NICE. In such circumstances, these drugs might only receive interim funding for a very short period of time if the interim appraisal decision was 'not recommended for routine use'. Furthermore, widening the provision of interim funding in this way would also reduce the amount of funding available, from the fixed CDF budget, for more clinically and cost effective drugs. It would also be necessary for manufacturers to agree that they would continue to fund patients in receipt of their drug initially funded under this variant option if at the time of subsequent interim assessment it is not recommended.

Funding after Exit from the Fund

39. If and when NICE determine that a drug should not be recommended for routine commissioning, that drug will cease to receive funding from the CDF, with the company expected to pay for the drug for those patients who had previously received it. The exception to this will be for those drugs that remain in the CDF as at 31st March 2016. Should one of these drugs receive a 'not recommended' decision at first appraisal, then funding for existing patients will continue to be met from the CDF budget.

Off-Label Cancer Drugs

40. It is recognised that the potential provision of off-label drugs is an important issue for certain rare cancers, and we wish to invite views, through this consultation, on how this can be addressed.

COSTS OF OPERATING THE NEW PROCESS

Investment Control Arrangements

41. Companies will be asked to propose a commercial access arrangement when their drug is identified by the NICE Appraisal Committee as a candidate for the CDF. The cost of the drug in the commercial access arrangement may not exceed what would otherwise have been necessary for NICE to have recommended the drug for routine commissioning. Acceptance of the company's proposal will be conditional on these costs being acceptable to NHS England, in the context of the investment control arrangements set out below.

42. To ensure the financial sustainability of the CDF, investment control mechanisms will be put in place to enable it to operate within a fixed budget. These measures will ensure that companies are encouraged to develop the most competitive commercial access arrangements and that there is an incentive for companies to generate and publish the required data as quickly as possible. The measures are aimed at ensuring that the NHS can secure maximum benefit for patients from its expenditure on these drugs while more data is obtained on their effectiveness.

43. As a general principle, the allocation of funds from the CDF to an individual drug/indication will be influenced by the number of patients in the UK necessary to collect the data required by the NICE Appraisal Committee and the cost effective price of the drug implied by the NICE appraisal. These factors will be taken into account in agreeing the commercial access arrangement.

44. A range of budget control measures, which could be applied singly or in combination, depending on the circumstances, has been considered. In the light of this evaluation, the proposal is to introduce a prospective contingency provision and a cost cap for the total cost of each drug.

Prospective Contingency Provision

45. During the year, the amount paid out by the CDF to all companies will be set at a consistent level below 100% of the sums which would otherwise be due under the commercial access arrangements. The remainder will be retained until the end of the year as a contingency. At the end of the year:

- if the CDF has stayed within the budget (net of the contingency), the retained contingency will be released to companies (the sums being paid in proportion to the payments already made during the year);

- if the CDF expenditure has exceeded the net budget, the retained contingency will be retained to the extent necessary to balance the budget and any remaining amount will again be paid to manufacturers proportionately;

- if the amount by which the net budget is exceeded is more than the retained contingency, the whole contingency will be used to balance the budget as far as possible and an across the board reduction in the price paid for each CDF product will also be used to bring the total expenditure within budget.

Capping the cost of the drug aligned to prospective maximum patient numbers needed for data collection

46. The application of the CDF budget needs to be closely associated with the number of patients in England required to generate the data needed for NICE to review clinical and cost effectiveness. NICE will provide advice on the likely numbers of patients required for the data collection exercise, and the maximum cost borne by the NHS in each financial year the drug is in the CDF will be closely linked to this requirement. Access to the drug by eligible patients will not be restricted to the number of patients considered necessary for data collection, but any costs for treatment over and above this number will be paid for by the company.

47. Other ways of managing the CDF budget that were considered included a 'queuing' approach, in which a new drug would not be approved to enter the CDF if projected expenditure on it would result in the Fund exceeding its annual budget, and options for placing global caps on total expenditure on any one drug/indication or with any individual supplier. However, it was concluded that these alternative approaches would not be as effective as the options selected in achieving transparency, equity of access, fair treatment for manufacturers and operational effectiveness, while also containing the budget.

CDF Investment Group

48. A CDF Investment Group (a joint committee of NHS England and NICE) will be established, consisting of staff from NHS England and NICE. The Group will be responsible and accountable for ensuring that the CDF is managed within its budgetary limits. It will receive and make decisions on recommendations from the NICE Appraisal Committees for drugs to enter the Fund, determine the managed access agreement in each case and monitor the use of the CDF. To achieve this, both NICE and NHS England will establish new operational teams and mechanisms. The main day to day liaison at the operational level will be via these teams, with strategic level liaison between NHS England and NICE occurring through the CDF Investment Group.

CONSULTATION AND TRANSITION ARRANGEMENTS

49. This consultation will take place for a 12 week period, beginning on 19th November 2015. The results of this consultation will be received by both NHS England and NICE Boards. Both NHS England and NICE will agree a decision on the shape of the new operating model, having taken into account the consultation responses, and the new CDF will become operational from 1st April 2016 with a target to complete the full transition by the end of March 2017.

50. Transition arrangements are not included in the current consultation, as the nature of the arrangements required will depend on the substantive decisions to be taken about the new CDF framework following consideration of responses to this consultation.

51. Once the consultation on the new CDF arrangements has started, and without prejudging the outcome of that consultation, NHS England and NICE will have provisional discussions with companies about the implications of the new framework including existing individual commercial access arrangements or need for data collection, for those products remaining in the CDF in November 2015. The process of appraising drugs currently in the CDF in line with the new CDF criteria will be completed during the course of 2016/17.

52. All patients receiving treatment funded through the CDF on 31st March 2016 will continue to receive treatment until the point that they and their consultant agree that it is appropriate to stop.

APPENDIX A – SUMMARY OF KEY DECISION POINTS

Initial appraisal at grant of Marketing Authorisation		
Outcome	**Effect for new patients**	**Effect for any patients already receiving the drug**
Draft recommendation for routine commissioning	Drug immediately available to patients at the point of Marketing Authorisation (receiving interim funding from CDF budget)	Drug continues to be available
Draft recommendation for use within the CDF	Drug immediately available to patients at the point of Marketing Authorisation (receiving interim funding from CDF budget)	Drug continues to be available
Draft "not recommended" guidance	Drug only available if an individual funding request is made and succeeds	Patients may continue their course of treatment until they/their clinician agree it is appropriate to stop. Funding from original source.
Guidance within 90 days of grant of Marketing Authorisation		
Outcome	**Effect for new patients**	**Effect for any patients already receiving the drug**
Recommended for routine commissioning	Drug immediately available to patients, (funded from the CDF budget for 90 days before moving to baseline commissioning budget)	Drug continues to be available
Recommendation for use within the CDF	Drug immediately available to patients(funded from CDF budget)	Drug continues to be available
Not recommended	Drug only available if an individual funding request is made and succeeds	Patients may continue their NHS funded course of treatment until they/their clinician agree it is appropriate to stop. Funding to be provided by the company.

Final decision after CDF funded evaluation period		
Outcome	**Effect for new patients**	**Effect for any patients already receiving the drug**
Recommended for routine commissioning	Drug immediately available to patients (funded from the CDF budget for 90 days before moving to baseline commissioning budget)	Drug continues to be available
Not recommended	Drug only available if an individual funding request is made and succeeds	Patients may continue their NHS funded course of treatment until they/their clinician agree it is appropriate to stop. Funding to be provided by the company.

APPENDIX B - Proposed amendments to the NICE technology appraisal processes and methods guides to support the proposed new Cancer Drugs Fund arrangements

Technology Appraisal Processes

This document sets out the proposed changes to the Guide to the Processes of Technology Appraisal necessary to support the joint NHS England and NICE proposals for the management of the Cancer Drugs Fund from April 2016.

Only relevant sections of the Guide are shown. Therefore the sections below need to be read in conjunction with the Guide to the Processes of Technology Appraisal.

New text proposed to be inserted into the guide is shown below in *italics.*

2. Selection of technologies

2.3 Prioritisation

2.3.3 All new cancer drugs and significant new licensed indications for cancer drugs will be referred to NICE for appraisal.

The Appraisal Process for Cancer Drugs

In order to be able to publish guidance on cancer drugs within 90 days of the marketing authorisation, NICE will hold the first Appraisal Committee meeting for a cancer drug before the CHMP opinion is published, ideally at or about the 180 day point in the regulatory process. Because the drug will not, at this stage, have received a regulatory opinion, this Appraisal Committee meeting will be held in private, in order to preserve the confidentiality of the data submitted by the company. Patient, clinical and commissioning experts, and company representatives will be invited to participate in the meeting under normal confidentiality arrangements.

After this Appraisal Committee meeting, an Appraisal Consultation Document (ACD) with a preliminary recommendation, or a Final Appraisal Determination (FAD) will be developed. As soon as the CHMP opinion has been published, NICE will establish whether the CHMP opinion is the same as, or similar to, the indication provided in the company submission. If it is, the ACD and the committee papers will be sent to consultees, commentators, the clinical

experts, NHS commissioning experts and patient experts for consultation (or consideration of appeal where a FAD is produced). In cases where the CHMP opinion is substantially different from the indication provided in the company submission, a further Appraisal Committee discussion may be necessary. An ACD or FAD is confidential until NICE publishes it on its website, normally 5 working days after it has been sent to consultees.

Where an ACD has been produced, the subsequent Appraisal Committee meeting will be held in public shortly after the publication of the Marketing Authorisation.

Consultation on the Appraisal Consultation Document (ACD) (if produced)

3.7.26 When a cancer drug is recommended for use within the Cancer Drugs Fund (CDF), the Appraisal Committee will state the conditions for its use in the Appraisal Consultation Document (ACD) and will identify the nature of the clinical uncertainty which should be addressed through data collection. Details of data collection, including the protocol and the analysis plan, will be set out in a 'managed access agreement'.

3.7.27 *The data collection arrangements will be developed, during the consultation period, by the company, NHS England, and NICE with input from clinicians and patients, and on advice from NHS England's Chemotherapy Clinical Reference Group and NICEs Observational Data Unit (ODU). It will be completed before the final guidance is published. Funding for data collection and analysis will be provided by the company holding the marketing authorisation for the product.*

5 Patient access schemes, flexible pricing and commercial access arrangements

5.2 *In the context of the Cancer Drugs Fund, companies agree 'commercial access arrangements' with NHS England. Such arrangements will be considered in the NICE technology appraisal.*

Definitions

5.5 *A commercial access arrangement is a proposal from a company to NHS England to manage the cost of a drug to the NHS. Commercial access agreements support the inclusion of cancer drugs in the CDF and facilitate patient access to a medicine through the CDF where NICE technology appraisal, on the current evidence base, is unlikely to support a recommendation for routine use.*

5.6 NICE can only consider patient access schemes (see figure 5) and flexible pricing proposals (see figure 6) after these have been formally approved by the Department of Health.

Commercial access arrangements

5.31 When the Appraisal Committee decides to recommend a technology for use within the CDF, the company will be invited to propose a commercial access arrangement, or amend an arrangement that has already been proposed.

5.32 In order for a cancer drug to be recommended for use through the Fund, it must display plausible potential for satisfying the criteria for routine use, taking into account the application of the End of Life criteria where appropriate.

5.33 Companies should work with NICE and ask for advice about the assumptions used in the consideration of clinical and cost effectiveness by the Appraisal Committee, which must form the basis of their proposal for a commercial access arrangement.

6 Reviews

Updating technology appraisals after inclusion in the Cancer Drugs Fund

6.22 NICE will normally review its guidance for a cancer drug funded through the CDF within 24 months of publishing it. The aim of the CDF guidance review is to decide whether or not the cancer drug can be recommended for routine use. The drug (or indication) may not remain in the CDF once the guidance review has been completed

6.23 Progress with data collection will be reviewed regularly. An annual report, provided by the company or the organisation collecting the data, will be submitted to NICE to check whether the data collection is on track, and to establish whether any additional action is needed. This will be coordinated through the NICE Observational Data Unit. Guidance may be considered for review before the published review time when there is significant new evidence that either supports the original case for clinical and cost effectiveness, or when the evidence points to the likelihood that the original recommendations are not valid. The steps involved are shown in table 8, 9 and figure a.

6.24 The published guidance will be withdrawn, and the drug removed from the CDF, if the company stops data collection for reasons other than an early guidance review.

6.25 Review of guidance for cancer drugs funded by the CDF will be scheduled into the technology appraisal work programme to coincide with the end of the data collection period determined at the point of entry of the drug into the fund. This will normally not be longer than 24 months. If NICE considers it reasonable to review the published guidance earlier than at the designated data collection period, the decision to do so will be subject to consultation.

6.26 The guidance review will be undertaken through a shortened technology appraisal process, which will normally take a maximum of 6 months. The company will have 4 weeks to submit the new evidence from data collection, and the ERG will have 4 weeks to critique the new evidence (see table 8).

6.27 The CDF guidance review will take into account the data that have become available since the original appraisal, together with any change to the patient access scheme or commercial access arrangement proposed by the company. No changes to the scope of the appraisal will be considered.

6.28 Companies must provide an evidence submission to support the CDF guidance review. The managed access agreement signed at the time of the original appraisal will include this obligation.

6.29 After the first committee meeting for the guidance review, a Final Appraisal Determination (FAD) will be produced if its recommendations are consistent with the original conditions for use in the Cancer Drugs Fund. In all other circumstances, an ACD will be produced.

Table 8 Expected timelines for the Cancer Drugs Fund guidance review - shortened technology appraisal process

		Weeks (approx.)
Step 1	*NICE invites organisations to participate in the guidance review as consultees or commentators*	*0*
Step 2	*NICE receives evidence submission from company*	*4*

	holding the marketing authorisation	
Step 3	*NICE requests clarification from the company on the evidence submission*	*5*
Step 4	*NICE invites selected clinical experts, NHS commissioning experts and patient experts to attend the Appraisal Committee meeting*	*7*
Step 5	*NICE sends the ERG report to the company for fact checking*	*8*
Step 6	*NICE compiles a review summary report and sends it to the Appraisal Committee*	*10*

**Timelines may change in response to individual appraisal requirements.*

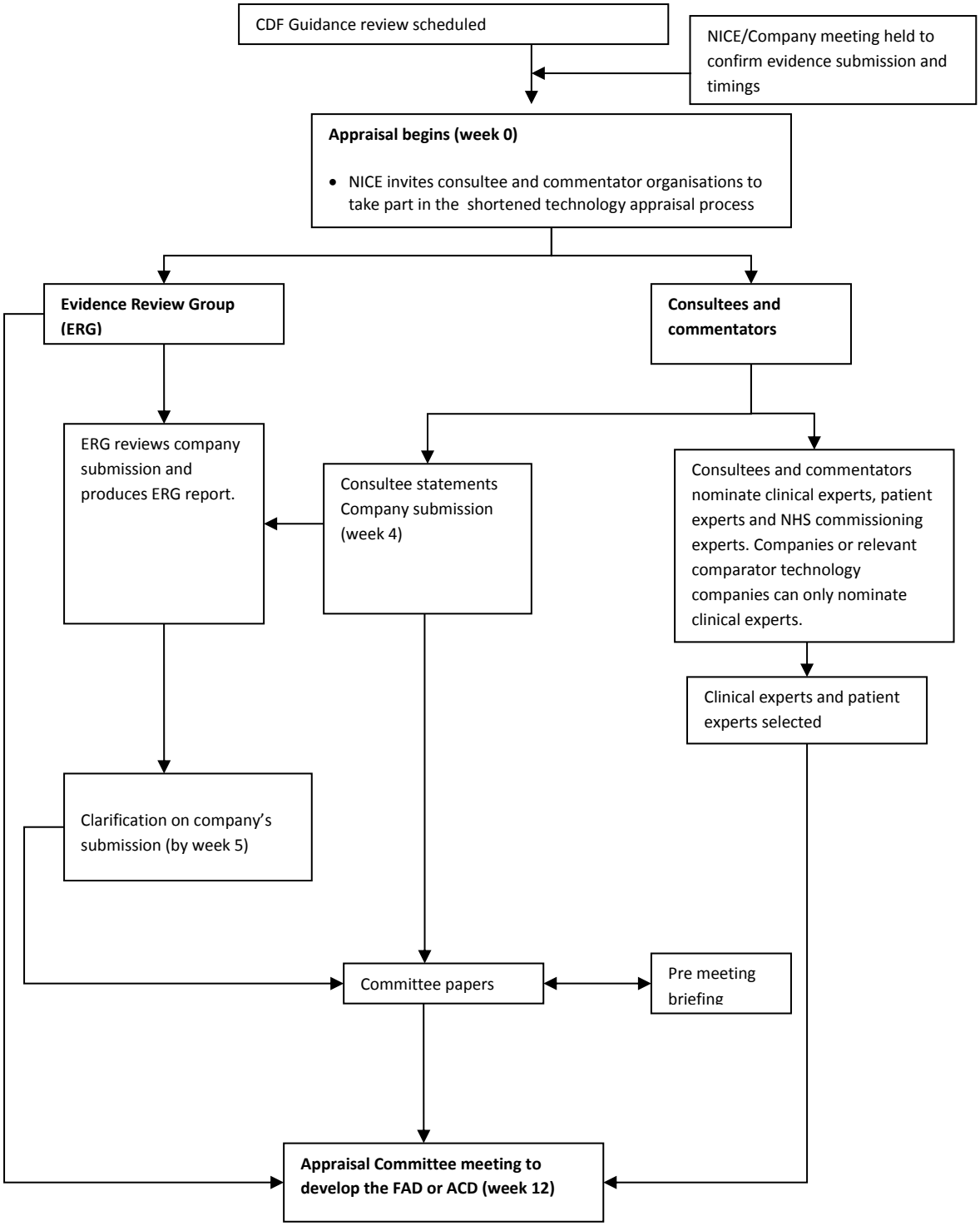
Table 9 Expected timelines for the Cancer Drugs Fund guidance review using the shortened appraisal process if an ACD is produced*

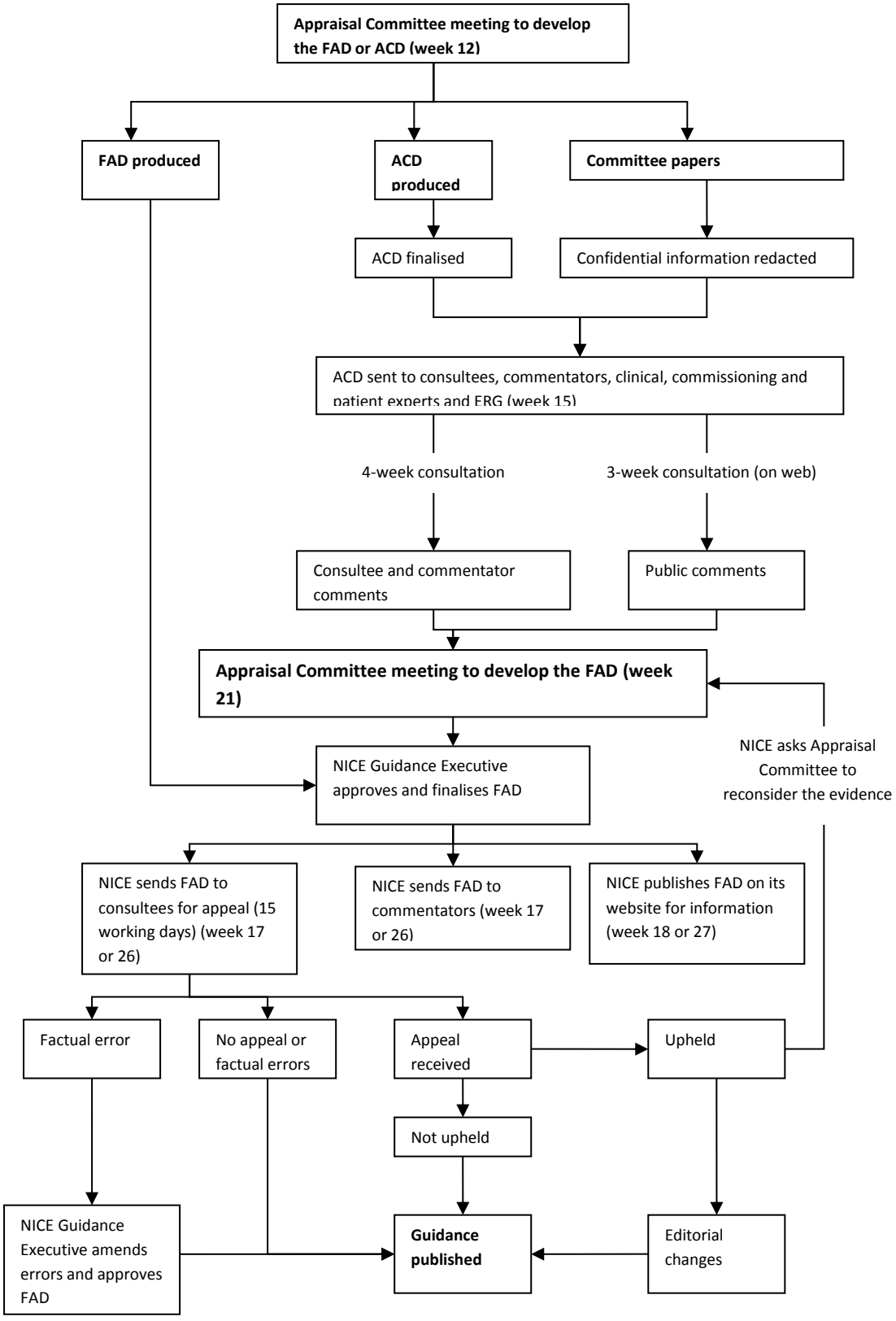
		Weeks
Step 7	*Appraisal Committee meeting*	*12*
Step 8	*The ACD is produced. NICE distributes the ACD and publishes it on the website 5 working days later*	*15*
Step 9	*Fixed 4-week consultation period on the ACD*	*15-19*
Step 10	*Appraisal Committee meeting to consider comments on the ACD from consultees and commentators, and comments received through the consultation on the NICE website. Appraisal Committee agrees the content of the FAD*	*20/21*
Step 11	*The FAD is produced. NICE distributes the FAD and publishes it on the website 5 working days later*	*26*
**Timelines may change in response to individual appraisal requirements.*		

Table 10 Expected timelines for the Cancer Drugs Fund guidance review using the shortened appraisal process if an ACD is not produced*

		Weeks
Step 7	*Appraisal Committee meeting to develop a FAD*	*12*
Step 8	*The FAD is produced. NICE distributes the FAD and publishes it on the website 5 working days later*	*17*
**Timelines may change in response to individual appraisal requirements.*		

Figure a Summary of the Cancer Drugs Fund guidance review using the shortened technology appraisal process




Appraisal Committee meeting to develop the FAD or ACD (week 12)
FAD produced
ACD produced
Committee papers
ACD finalised
Confidential information redacted
ACD sent to consultees, commentators, clinical, commissioning and patient experts and ERG (week 15)
4-week consultation
3-week consultation (on web)
Consultee and commentator comments
Public comments
Appraisal Committee meeting to develop the FAD (week 21)
NICE asks Appraisal Committee to reconsider the evidence
NICE Guidance Executive approves and finalises FAD
NICE sends FAD to consultees for appeal (15 working days) (week 17 or 26)
NICE sends FAD to commentators (week 17 or 26)
NICE publishes FAD on its website for information (week 18 or 27)
Factual error
No appeal or factual errors
Appeal received
Upheld
Not upheld
NICE Guidance Executive amends errors and approves FAD
Guidance published
Editorial changes

Technology Appraisal Methods

This document shows all proposed changes to the Guide to the Methods of Technology Appraisal 2013.

Only relevant sections of the Guide are shown. Therefore the sections below need to be read in conjunction with the Guide to the Methods of Technology Appraisal

New text proposed to be inserted into the guide is shown below in *italics.*

The text ~~scored out~~ is proposed to be deleted from the current Guide.

6 The appraisal of the evidence and structured decision-making

Structured decision-making: clinical effectiveness and health-related factors

6.2.10 In the case of a 'life-extending treatment at the end of life', the Appraisal Committee will satisfy itself that all of the following criteria have been met:

- the treatment is indicated for patients with a short life expectancy, normally less than 24 months and
- there is sufficient evidence to indicate that the treatment has the prospect of offering an extension to life, **normally** of a mean value of at least an additional 3 months, compared with current NHS treatment.

~~and~~

~~the technology is licensed or otherwise indicated, for small patient populations normally not exceeding a cumulative total of 7000 for all licensed indications in England.~~

In addition, the Appraisal Committees will need to be satisfied that:

- the estimates of the extension to life are *sufficiently r*obust and can be shown or reasonably inferred from either progression-free survival or overall survival (taking account of trials in which crossover has occurred and been accounted for in the effectiveness review) and
- the assumptions used in the reference case economic modelling are plausible, objective and robust.

6.2.11 When the conditions described in section 6.2.10 are met, the Appraisal Committee will consider:

- the impact of giving greater weight to QALYs achieved in the later stages of terminal diseases, using the assumption that the extended survival period is experienced at the full quality of life anticipated for a healthy individual of the same age and
- the magnitude of the additional weight that would need to be assigned to the QALY benefits in this patient group for the cost effectiveness of the technology to fall within the normal range of maximum acceptable ICERs, with a maximum weight of 1.7.

6.2.12 Treatments recommended following the application of the 'end-of-life' criteria listed in section 6.2.10 will not necessarily be regarded or accepted as standard comparators for future appraisals of new treatments introduced for the same condition. Second and subsequent extensions to the marketing authorisations for the same product will be considered on their individual merits.

6.5 Making recommendations for use through the Cancer Drugs Fund

6.5.1 *When the evidence for the clinical and cost effectiveness of a drug has been assessed, including, when appropriate, the factors described in 6.2.10–17, the Appraisal Committee will decide whether the drug can be recommended for routine use.*

6.5.2 The Appraisal Committee will determine whether the estimates of the extension to life are sufficiently robust.

6.5.3 If the Appraisal Committee concludes that estimates of the extension to life are not sufficiently robust, such that the uncertainty in the clinical and cost effectiveness data is too great to recommend the drug for routine use, the Committee can consider a recommendation for use within the Cancer Drugs Fund if the following criteria are met:

- *The incremental cost-effectiveness ratios (ICERs) presented have the plausible potential for satisfying the criteria for routine use, taking into account the application of the End of Life criteria where appropriate. (see sections 5.8.10 and 6.3.2–5 of the guide to the methods of technology appraisal).*

- *It is possible that the clinical uncertainty can be addressed through collection of outcome data from patients treated in the NHS.*
- *It is possible that the data collected (including from research already underway) will be able to inform a subsequent update of the guidance. This will normally happen within 24 months.*

6.5.4 *The arrangements for data collection will be part of the managed access arrangement to be drawn up between the company, NHS England, and NICE with input from clinicians and patients, and with advice from NHS England's Chemotherapy Clinical Reference Group and NICE's Observational Data Unit (see the guide to the processes of technology appraisal section 3.7.27) before final guidance is published.*

APPENDIX C

CONSULTATION QUESTIONS:

IMPORTANT NOTE: In line with standard requirements regarding transparency of payments by the pharmaceutical industry, all respondents should complete question 18, disclosing any payments, grants or other funding received by their recipient or their organisation from the pharmaceutical industry in the last three years, and specifying the source of funding and sums involved in each of the last three years.

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:

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:

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:

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:

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:

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:

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:

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:

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?

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?

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:

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?

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

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:

QUESTIONS ABOUT YOU:

15. Are you responding:
    - ☐ as a patient *
    - ☐ as a carer *
    - ☐ as a member of the public *
    - ☐ as a health or social care professional**
    - ☐ on behalf of an organisation ***

    * If you are responding as a patient, carer or a member of the public, please proceed directly to Question 18
    ** If you are responding as a health or social care professional, please go to the next question.
    *** If you are responding on behalf of an organisation, please only complete Questions 17 and 18.

16. Please indicate if you are a:
    - ☐ Paramedic
    - ☐ Radiographer
    - ☐ Other Allied Health Professional
    - ☐ Doctor
    - ☐ Nurse/Health Visitor
    - ☐ Pharmacist
    - ☐ Other Health and Social Care Professional

    If you selected 'Other Health & Social Care Professional', please specify.

17. If you are responding as a health or social care professional, or on behalf of an organisation, please indicate your primary area of work or the nature of the organisation you represent:
    - ☐ NHS Acute
    - ☐ NHS Community
    - ☐ Social Care
    - ☐ Private Health
    - ☐ Third Sector
    - ☐ Regulatory Body
    - ☐ Professional Body
    - ☐ Education
    - ☐ Trade Union
    - ☐ Local Authority
    - ☐ Independent Contractor to NHS
    - ☐ Pharmaceutical Company
    - ☐ Other Supplier
    - ☐ Other

    If you selected 'Other', please give details:

18. 'Sunshine' provision/conflict of interest disclosures: have you or your organisation received any payments, grants or other funding from the pharmaceutical industry in the last three years?

☐ Yes
☐ No

If yes, please specify the source of funding and sums involved in each of the last three years:

DEMOGRAPHIC QUESTIONS: EQUALITY MONITORING

19. How old are you?

☐ Under 18
☐ 18 – 24
☐ 25 – 34
☐ 35 – 54
☐ Over 55
☐ Prefer not to say

20. What gender do you identify yourself as?

☐ Male
☐ Female
☐ Neither
☐ Prefer not to say

21. Do you consider yourself as a person with a disability?

☐ Yes
☐ No
☐ Prefer not to say

22. What is your ethnic group?

☐ British
☐ Irish
☐ White and Black Caribbean
☐ White and Black African
☐ White and Asian
☐ Indian
☐ Pakistani
☐ Bangladeshi
☐ Caribbean
☐ African

☐ Chinese
☐ Other
☐ Do not wish to disclose
If you selected 'Other', please specify

23. What is your religion or belief?

☐ None
☐ Christian
☐ Buddhist
☐ Hindu
☐ Jewish
☐ Muslim
☐ Sikh
☐ Other
☐ Prefer not to say
If you selected 'Other', please specify

24. Which of the following best describes your sexual orientation?
Only answer this question if you are aged 16 years or over.

☐ Heterosexual / Straight
☐ Lesbian / Gay Woman
☐ Gay Man
☐ Bisexual
☐ Prefer not to say