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

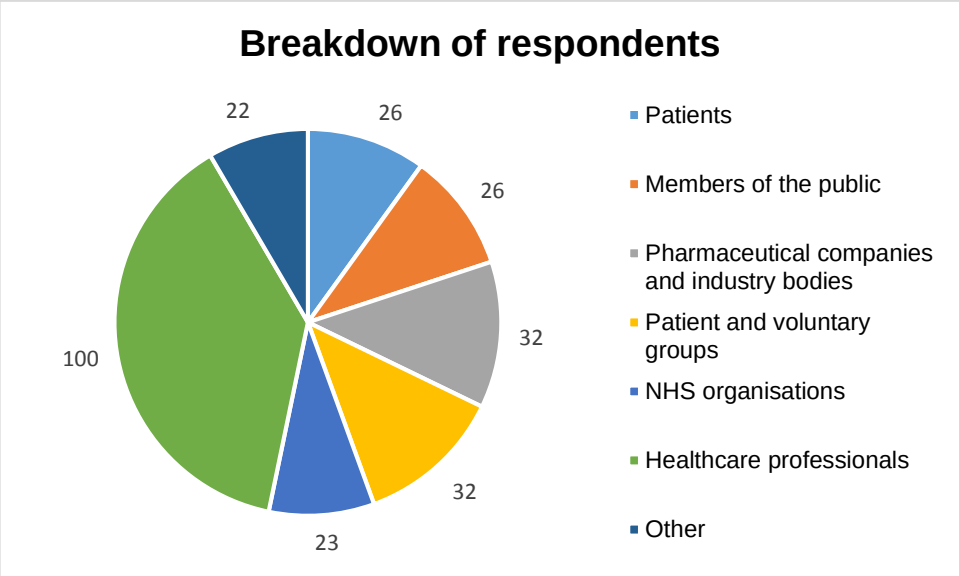
This report covers the responses received to the consultation on the proposals for a new Cancer Drugs Fund operating model which ran from 19 November 2015 to 11 February 2016.

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

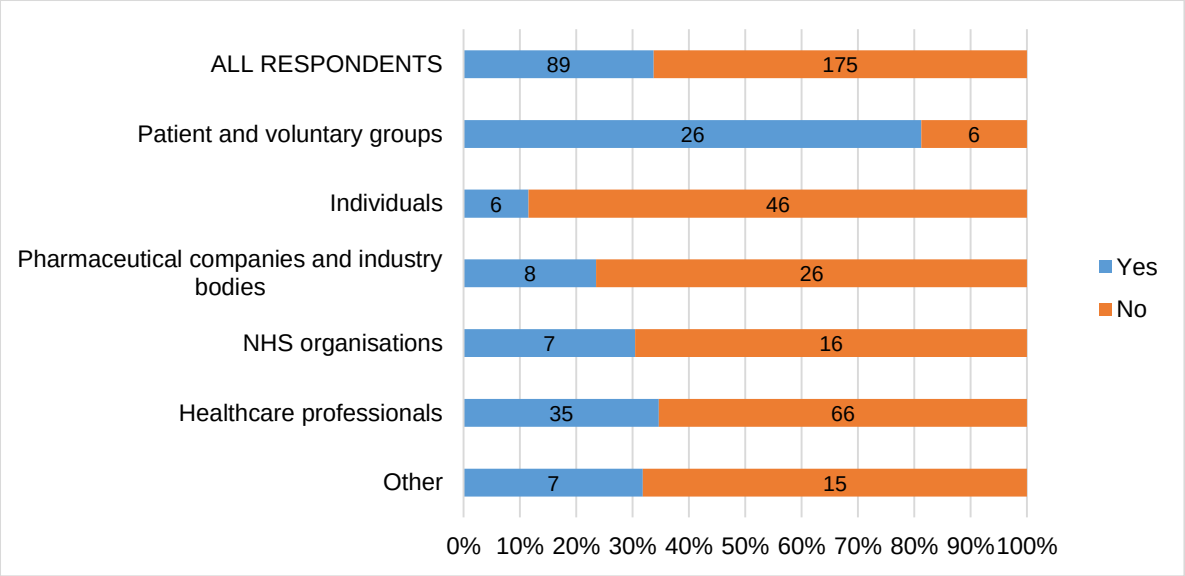
- Other organisations and individuals, which include:
  - All Party Parliamentary Group on Pancreatic Cancer
  - All Party Parliamentary Group on Cancer
  - Brain Tumour Research
  - Clinical Commissioning Groups and other NHS organisations
  - London Cancer
  - Members of Parliament
  - NHS England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

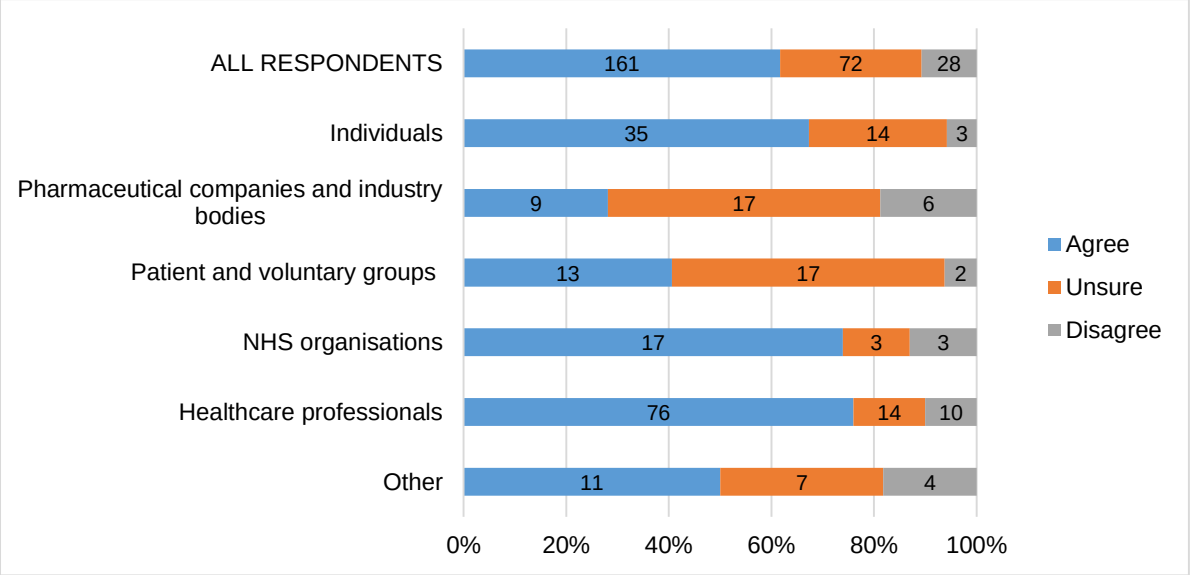


Overall, 34% of respondents declared they had received payments from drug companies.

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

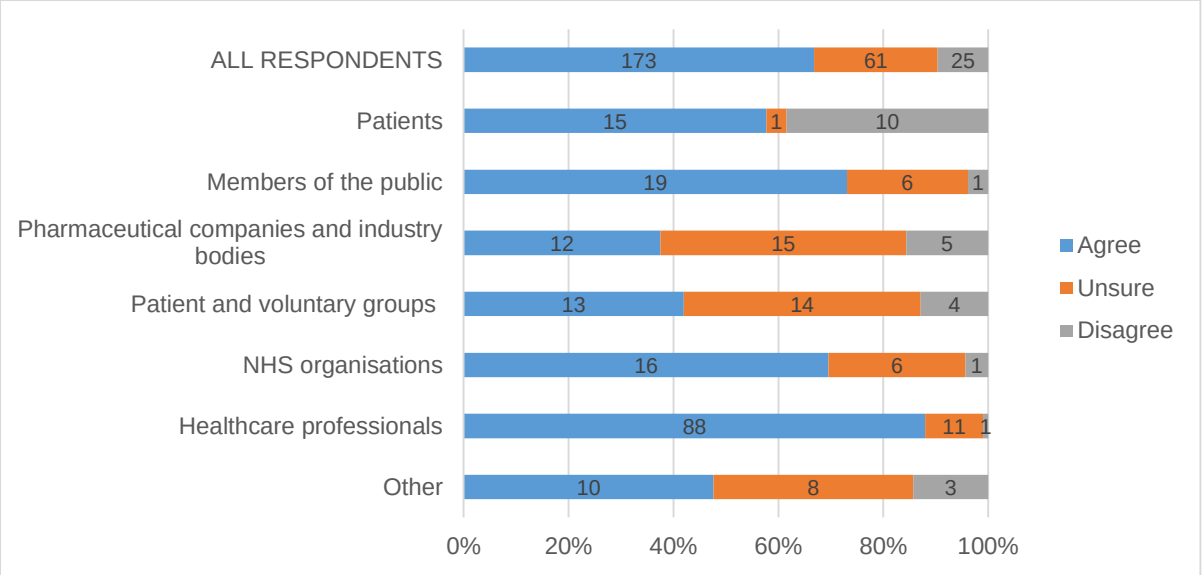
Female doctor, NHS acute trust

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

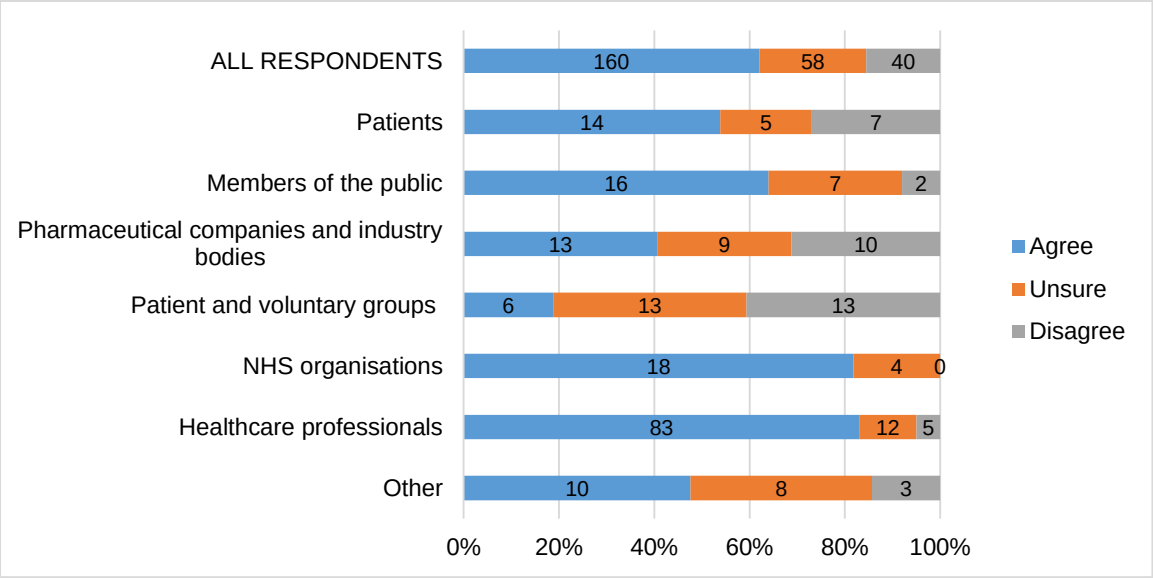
The majority of healthcare professionals agreed with this proposal (88%).

> *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**

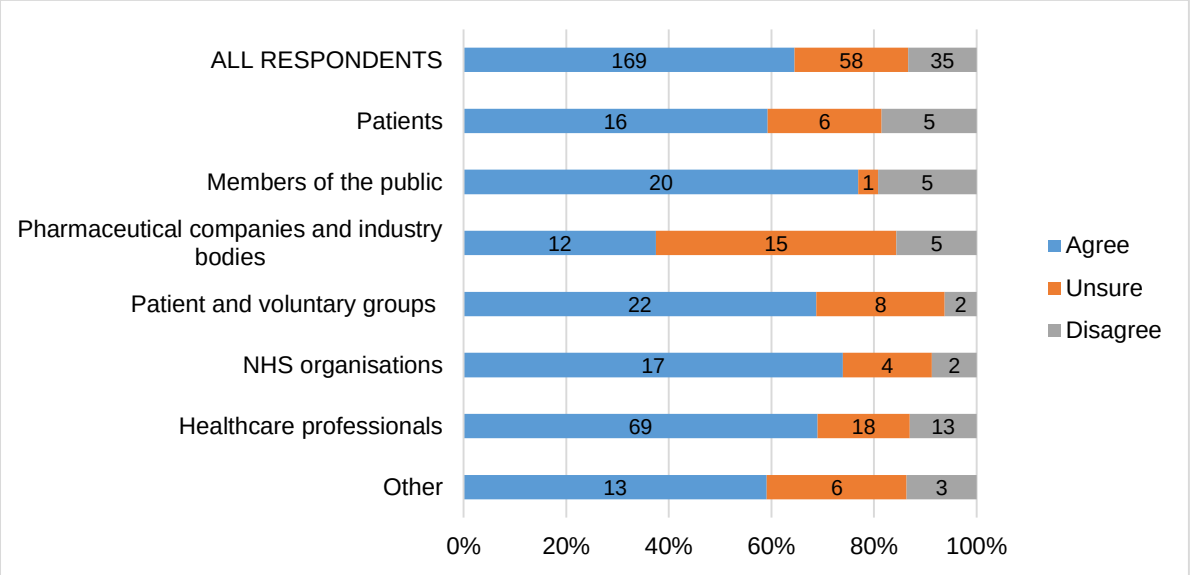
A small number of people were unsure. Again, some of the concern was about the process taking too long and the capacity of NICE. Others felt unsure about the terminology and implications of 'appropriate modification' and 'significant licence extensions'.

> *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

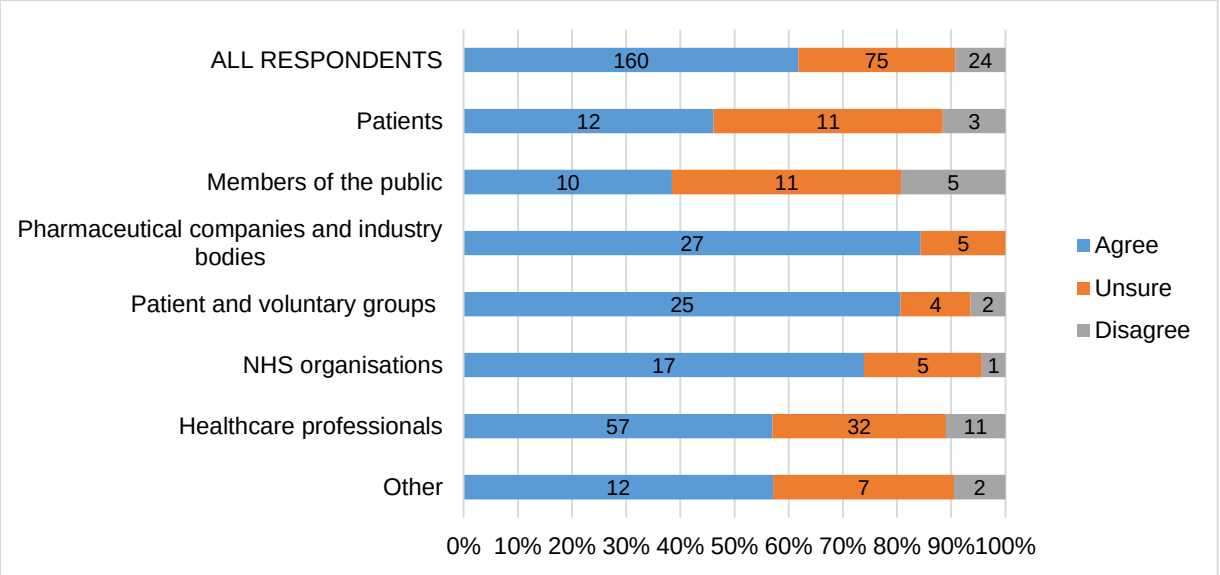
Most healthcare professionals agreed with this proposal (69%). There were some provisos, largely around what criteria would be used in the decision to assign one category or another.

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.

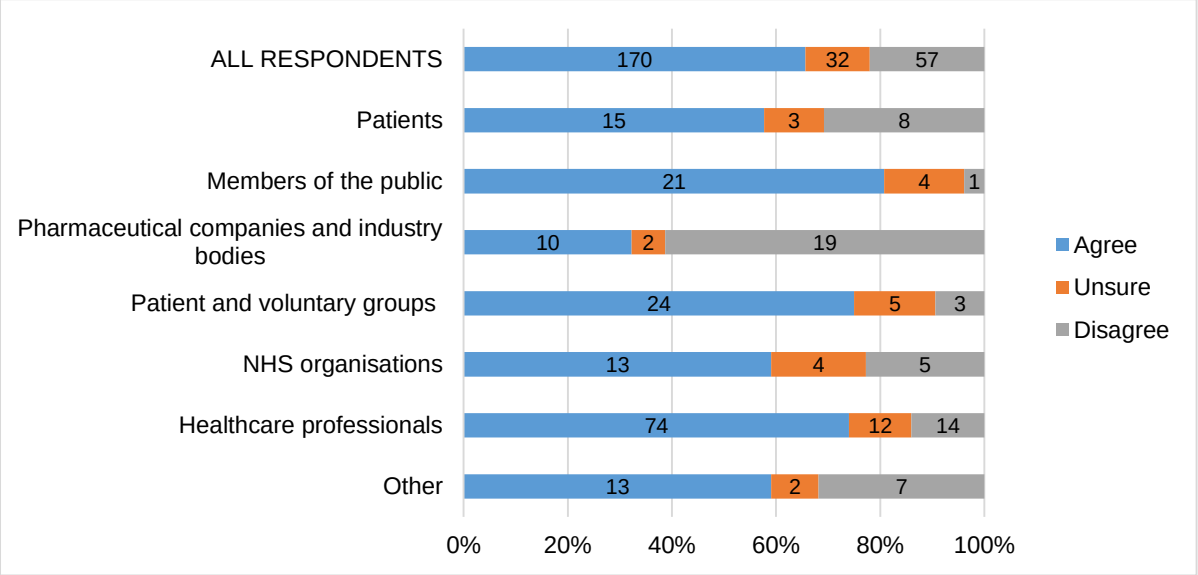
Healthcare professionals

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

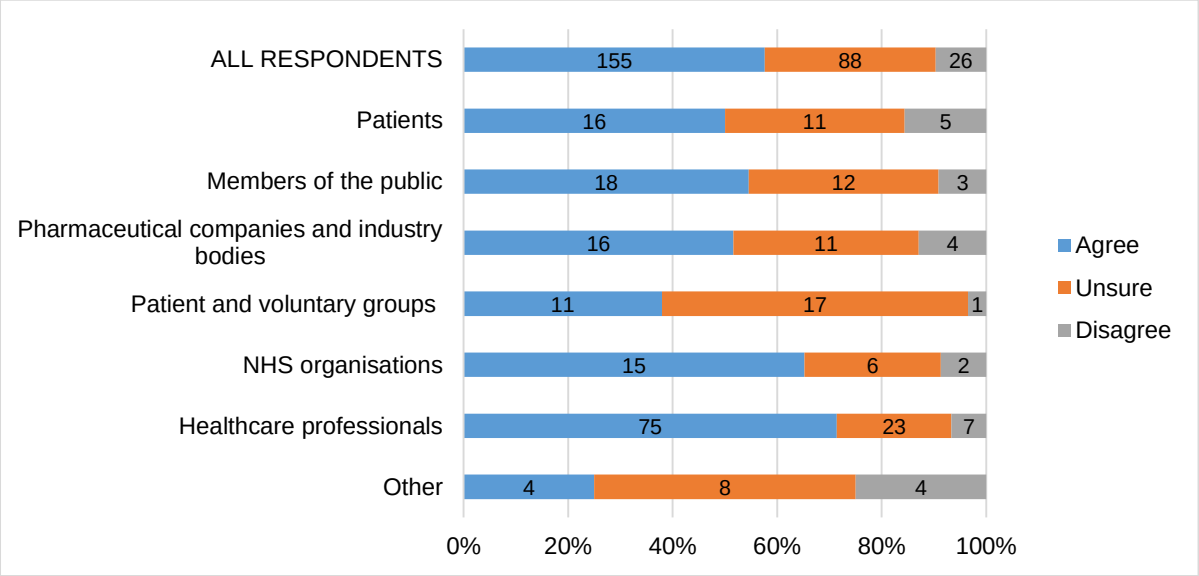
Over half of healthcare professionals agreed with this proposal (59%). Those who disagreed (23%) were mostly concerned about marketing authorisation happening at the right time, and what would happen if a drug did not then receive marketing authorisation.

> *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

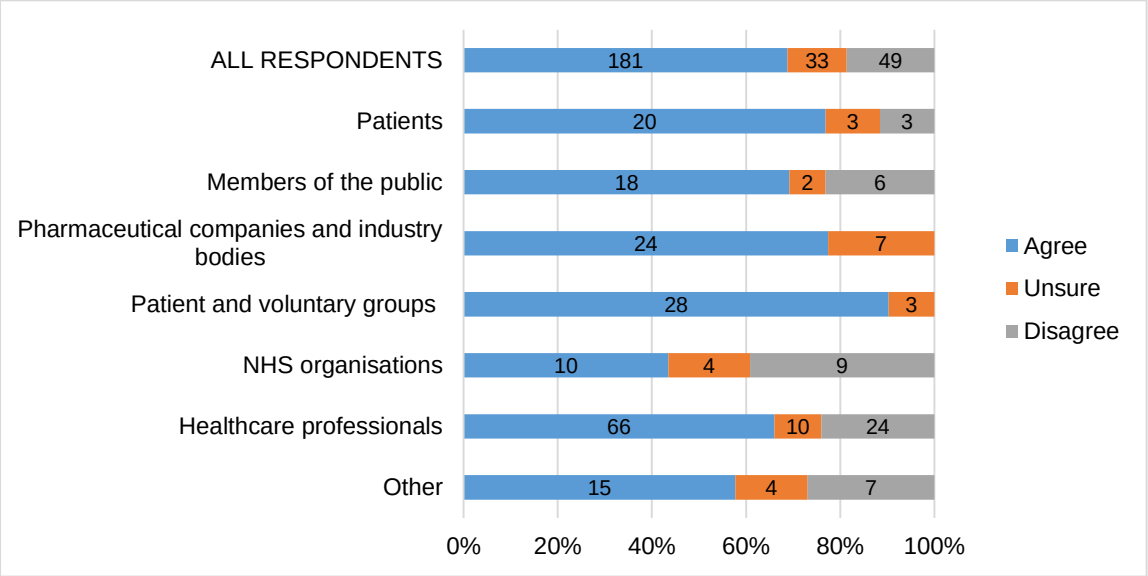
Most healthcare professionals agreed with this process change, though some expressed concern over the impact on NICE and its ability to put the changes in place within 90 days.

> *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:

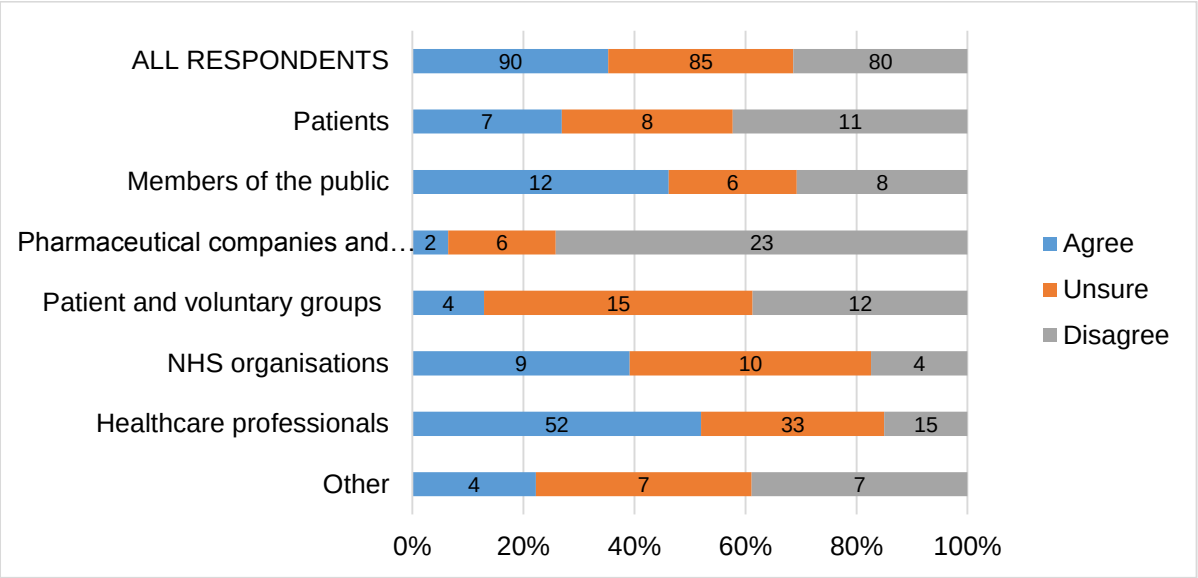
- Clinicians could submit suggestions for indications to be considered and have some sort of prioritisation/voting system
- NICE could commission trials or systematic observational data collection during the period of interim funding, to reduce uncertainty for unlicensed drugs.
- When a 'clinical' body 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.

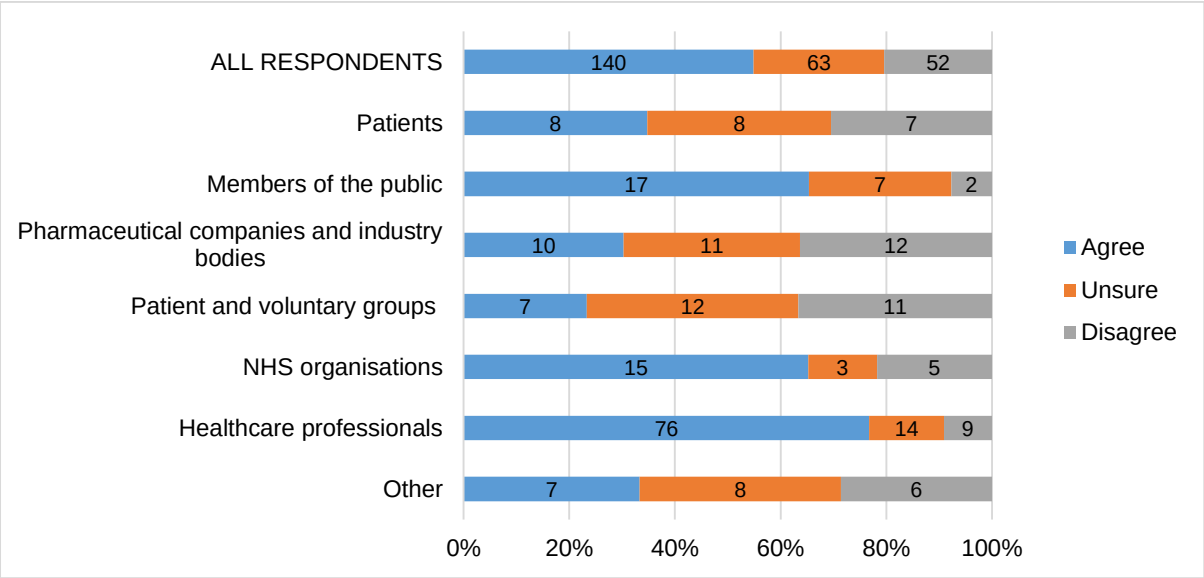
Healthcare professionals

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**	