Consultation on proposals for a new cancer drugs fund (CDF) operating model from 1st April 2016. Response from the All Party Parliamentary Group (APPG) on Pancreatic Cancer

Background - what is the APPG on Pancreatic Cancer?

The All-Party Parliamentary Group (APPG) on pancreatic cancer was established in 2012, by a leading group of parliamentarians with an interest in making a difference for pancreatic cancer. An All-Party Parliamentary Group is primarily an interest group and provides a forum for MPs and Peers to meet and interact with stakeholders, to share ideas about issues and discuss ways to change the political landscape for the subject they are concerned with.

The APPG provides an excellent forum for keeping pancreatic cancer high on the political agenda, for example through debates and oral and written questions. The purpose of the APPG is to raise the profile of pancreatic cancer in Parliament, to raise issues of relevance with government and policy makers; and to influence policy to improve life for pancreatic cancer patients and survivors.

The current officers of the APPG are: Nic Dakin MP (Chair), Baroness Morgan of Drefelin, Lord Aberdare, Lord Patel, Mark Durkan MP, and Stuart Andrew MP.

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**
X **Unsure**

Please provide comments to support your response:

We should stress at the outset that we are glad a way is being sought to continue with the Cancer Drugs Fund (CDF) beyond 31st March 2016. It is important to remember why the CDF was introduced in the first place.

The 2010 report *Extent and Causes of International Variations in Drugs Usage*[1] showed that the UK's take-up of new cancer drugs was just 45% of the average level of 13 countries similar to the UK. Furthermore, a National Audit Office report of 2015[2] showed that between 2007 and 2014, of the 102 cancer drug indications appraised by the National Institute of Health and Care Excellence (NICE) just 46% were recommended or partially recommended, well below the approval rate for non-cancer drugs (81%).

The CDF was brought in to help correct this disparity by operating outside of the NICE process. The APPG believes – and this was a belief shared by numerous MPs in a recent Westminster Hall debate on the CDF - that the CDF has led to a large number of patients being able to access drugs they would not otherwise have been able to since the CDF was launched in 2010. The National Audit Office report of September 2015[3] cites a figure of 74,000 patients falling into this category between October 2010 and March 2015.

---

[1] https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/216249/dh_117977.pdf
[2] https://www.nao.org.uk/report/investigation-into-the-cancer-drugs-fund/
[3] https://www.nao.org.uk/report/investigation-into-the-cancer-drugs-fund/

So for as long as the wider NICE scoring system which leads to fewer cancer drugs being approved – e.g. the use of QALYs and the current level of QALY threshold – remains in place, we believe that the CDF, in one form or another, needs to remain.

In terms of what that future CDF might look like, in various meetings since the APPG was launched in 2012 we have heard from experts who have supported the principle of a 'commissioning through evaluation' or 'managed access fund' approach when looking to assess the likely effectiveness, and cost-effectiveness, of new drugs. This is in large part because using such a system will allow 'real world data' to help inform decisions on which promising new drugs to fund, whilst also allowing faster and wider patient access to those drugs whilst a decision is reached.

However, whilst we agree that, *in principle*, a managed access fund (MAF) with 'clear entry and exit criteria' could be an appropriate way for the Cancer Drugs Fund (CDF) to be re-packaged in future, we do not believe that the information provided in either the consultation document itself (19th November 2015), or in the companion Q&A document produced much later (27th January 2016) provides that clear entry and exit criteria.

Indeed, overall, on the information available, we do not believe that the system proposed will lead to more drugs being made available to patients than the CDF has since 2010. **In fact, we believe it to be almost certain there will be fewer drugs made available**. The consultation Q&A document raises this prospect by stating that 'the proposals for the new CDF are not necessarily about more cancer medicines being recommended for use in the CDF, but about the right ones….' We do not agree with that statement and believe that the CDF should be about more cancer drugs being available to patients, especially those with conditions where there are very few treatment options already, as is the case for pancreatic cancer patients.

Specifically, we have concerns that:

- The changes to the 3 month rule are especially important for pancreatic cancer patients, where survival rates are extremely low and have remained virtually unchanged for 40 years, and where there are very few treatment options available. With average survival of between just 2-6 months for metastatic pancreatic cancer patients, additional survival gain of less than three months can still represent a relatively large increase in survival for patients to spend with their loved ones. The changes to the NICE end-of-life criteria seem unlikely to ensure more drugs for pancreatic cancer are provided with an entry route into the MAF, let alone given a full approval. We discuss this elsewhere in our response but suffice to say here that the re-wording to the end-of-life three month threshold contained in the consultation is minimal and unlikely to achieve the stated aim.

- Paragraph 31 of the consultation notes that only drugs that 'have the potential to lie within the thresholds specified in the NICE Technology Appraisal methods' will be considered for entry into the MAF scheme. However, how far outside the existing thresholds (£30,000 for most drugs and up to £50,000 for those meeting end-of-life criteria) a drug has to be to be considered to have that 'potential' is not specified. It is therefore unclear how many drugs may fall into this category.

- 24 months in a MAF may be long enough to resolve 'uncertainty' issues for some drugs, but those for rare cancers, or even for some less common cancers such as pancreatic cancer, this is not always going to be the case due to small population sizes of patients who might be eligible for treatment. The proposals leave some scope for TA committees to vary the length of a MAF period over which data is collected, but we believe that there should be a more

specific 'push' given to TA committees to consider giving longer periods of trial for new drugs for rare and less common cancers.

- We would question whether the proposals as set out will actually allow data to be collected in a genuine 'real world' setting. For instance, we know that drugs are sometimes not given to patients at full dose, or at the initially desired intervals, because different patients will react in different ways to them. However, this individualised approach would make it potentially difficult to collect data in a format that could be properly compared at the end of the MAF process. Clearly, we would like to see the individualised doses etc given, as this is likely to be best for patients.

- On the wider issue of data collection, there is a huge amount of uncertainty in the proposals, even as to the types of data that might be deemed to be collectable. Paragraphs 26 and 33 of the consultation simply state that data will be collected and analysed by NICE. It does not say who will collect it, although elsewhere it is made clear that the drug company will pay for the collection. This seems a key omission, as there could presumably be issues of ethical approval, patient consent and data protection if it is anybody other than the NHS collecting the data. If anything, that uncertainty only grows upon reading the Q&A document released on 27th January. We are told in Q12 that companies 'will be expected to be involved in the data collection in England' but not that they will be doing the collection. It goes on to say that it is 'hoped' the Systemic Anti-Cancer Therapy (SACT) database will 'also be able to collect' some data, although SACT is not fully up and running and many experts have concerns it will not ever be able to operate as intended. Q12 of the Q&A goes on to show that it is not yet known how this data collection process will actually happen when it notes that 'the organisation' collecting the data will need to collaborate with NICE, NHS England and the company. That this issue appears not to have been resolved at this stage is alarming.

- How the 'real world' data collected as part of the MAF will be analysed by a NICE TA at the end of the process is also not made clear. We are told in the Q&A, Q14, that the data collected will not be the only data looked at by the NICE TA committee when coming to a final decision when the MAF period is concluded. In addition, new trial data, follow up studies and 'other observational data' (Q5) will be considered. We are told that 'the weight to give to any particular trial data will be a matter for the expert judgement of NICE appraisal committees, as now.' This means that despite having spent two years paying for the collection of data in a real-world setting, that data could be 'trumped' by a trial carried out elsewhere over the same period. This could clearly work both ways- in favour of, or against giving a final positive recommendation to a particular drug – but not to spell out what weight should be given to the data collected as part of the MAF seems strange.

- The financial and cost-capping proposals set out elsewhere in the consultation document are complicated, uncertain, and possibly very costly. Whilst we support efforts by the NHS to get the best value for money possible from deals with pharmaceutical companies, there is a clear risk what is proposed might be a step too far and simply lead to many companies refusing to take part in the system and we end up with fewer drugs being made available to patients.

- There are questions whether all new patients with a particular condition will be able to receive a new drug if it has been entered into a MAF. This is because paragraph 46 of the consultation states that the CDF (i.e. the NHS) will only pay for drugs for the number of patients needed to generate the data required to make a final decision after 24 months on a MAF. The company will be expected to pay for the rest of the patients. Question 15 of the Q&A opens up the possibility that the company could decide not to fund the drug for the remaining patients: 'Whether that means there is no access for them [the other patients]

would depend on the specific arrangements made in the commercial access agreement.' This actually opens up a scenario of even greater inequality across the UK than there is at present.

- There is also a danger that NICE Technology Appraisal (TA) committees might prefer not to pass more definitive judgement and actually reduce the number of outright positive recommendations they give, opting to allow a drug to go down the MAF route instead. We hope that this would not be the case but without knowing more about how TA committees will be advised to define 'uncertainty' we cannot be sure.

- The document does not deal with transition arrangements but we are told in paragraph 49 of the consultation, and again in Q1 of the Q&A, the transition process should have been completed, and all drugs currently on the CDF reappraised by March 2017. However, as the CDF is currently still overspending, it is logical to presume that new drugs will have to be removed from the CDF before new ones can be funded through a MAF. If this cannot start until well into 2016, or maybe even as late as 2017, that is a significant gap where patients might not be able to access potential new treatments. In fact, since no new drugs have been added to the CDF since January 2015 – only de-listings have taken place in that time – there could be a gap of nearly two years where the CDF has not added new potentially beneficial treatments for cancer patients.

- Finally, we are disappointed that the transition arrangements are being looked at under a separate process. Whilst some drugs currently on the CDF will be absorbed into baseline commissioning, the transition clearly will lead to many more drugs being 'de-listed' from the CDF, and have a subsequent impact on patient access. This in turn will have a huge bearing on how much of the CDF will be available for new drugs, and from when.

We regret that so much has been left unanswered in the consultation document and that this leaves us unable to comment with certainty on whether we can support the proposal that the CDF should be turned into a MAF.

All this being said, we also believe that a much more thorough overhaul of the NICE appraisal process is required, if more cancer drugs are to be approved without having to go through this separate and possibly complicated CDF/MAF process. We urge Ministers at the Department of Health to instruct NICE to conduct a full review of its appraisal system at the earliest opportunity.

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**
X **Unsure**

Please provide comments to support your response:

Generally, we support the principle that all cancer new drugs should be assessed by one body and it makes sense that NICE is that body, given its expertise in the area.

However, despite the changes set out in the consultation document, including the introduction of a MAF and amendments to the end-of-life criteria, we still have concerns that the NICE process is not flexible enough when it comes to assessing new treatments for rare cancers and cancers of unmet need, and that these cancer types will not see an increase in the number of new treatments being approved.

As such, our support for NICE appraising all cancer drugs is contingent on NICE being reformed to an extent where we feel new treatments for rare and less common cancers, and cancers with substantial unmet need, stand a fairer chance of being recommended for commissioning.

It would have been helpful to understand better what 'new' and 'significant' mean in this context. Unfortunately, the Q&A document does not clarify the situation, with more uncertainty being introduced by Q6. The answer in the Q&A states 'it may be that NICE does not consider that an indication requires a full Technology Appraisal, and might channel it through another part of NICE. The exact arrangements are still to be developed….'

This begs the question 'what other parts of NICE.' Yet again, it is regrettable that there is such a lack of clarity at this late stage in proceedings and it means we cannot say we agree or disagree with the consultation as things stand.

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**
X **Disagree**
☐ **Unsure**

Please provide comments to support your response:

As already highlighted in response to question 1, we still harbour concerns that rare, and some less common, cancers will not be able to be properly assessed by NICE due to small population size and that even if TA committees refer them for participation in the proposed MAF, the amount of time the MAF would run for – usually up to 24 months – will not be long enough to generate definitive results. We also believe that the minor changes proposed to the end-of-life three month threshold do not go far enough to lead to the approval of new treatments for cancers with the very worst survival rates, where a modest life-extension of a couple of months represents a relatively large survival gain. This is discussed at more length elsewhere in the APPG's response.

In short, we believe that if *all* new cancer drugs are to be assessed by NICE, there needs to be a much greater modification of NICE processes above and beyond what is contained in the consultation proposals, for rare and less common cancers, and for cancer types with a clear and substantial unmet need, like pancreatic cancer.

We believe that a separate entry point into the NICE process for rare cancers and pre-determined cancers of unmet need (i.e. those with the worst survival rates and where a small amount of life-extension would be a relatively large survival gain for the condition concerned, like pancreatic cancer) would help resolve those problems. Entry under rare cancer, or cancer of unmet need route, should trigger an additional set of procedures to be followed by NICE, the results of which would have to be appropriately considered by TA committees.

For instance, for cancers of unmet need it would mean TA committees must take into account the *relative potential* survival gain of a new drugs, i.e. taking into account what the current average survival is for the relevant cancer type.

And for both rare cancers and cancers of unmet need, it should mean an additional phase of evidence gathering and consultation with patients and clinicians, which TA committees would be required to give due weight to, in addition to clinical- and cost-effectiveness evidence, as part of their deliberations.

We support the Patient and Clinician Engagement (PACE) approach introduced by the Scottish Medicines Consortium, which works on a similar basis for end-of-life and orphan drugs and which has started to see more drugs for rare cancers and cancers of unmet need approved for use in Scotland, e.g. the pancreatic cancer drug Abraxane. We would like to see NICE introduce a similar PACE process for cancers deemed to be rare or to meet a predefined definition of unmet need. This PACE process would then trigger a much more detailed stage of evidence gathering from patients, patient representative groups and clinicians, in order to build as broad a case both for and against the new drug to be appraised. The NICE TA committee would receive a PACE report and have to give due weight to the information it contains, alongside the usual consideration given to clinical and cost-benefit data.

Even if these additional modifications were made to the NICE process as part of this consultation, we would still urge that whatever emerges from these consultation proposals is not seen as a long-term solution to how cancer drugs are appraised. We maintain that a more thorough overhaul of the NICE system is needed, including a reassessment of the QALY system. Moreover, it is worth noting that patient engagement is barely mentioned in the consultation document at all. From our conversations with patients, carers, charities and others, we know how many of them feel that NICE is not responsive enough to their experiences and knowledge. The more thorough overhaul of NICE should also include changes to ensure that greater patient and carer input can be built into the system.

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**
X **Unsure**

Please provide comments to support your response:

As outlined in our response to question 1, we recognise there has long been agreement that drug assessment needs to take account of 'real world' clinical data and a MAF could allow data to be collected in that way. However, as also explained, we do not believe that the case has been made to be able to say the version of a MAF as contained in the consultation is workable or desirable.

Moreover, as a point of clarification, it is unclear from the consultation document as to what has happened to other outcomes currently available to NICE TA committees. E.g. 'optimised' or 'only in research.' Presumably these have been removed?

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?

X Agree
☐ Disagree
☐ Unsure

Please provide comments to support your response:

According to the National Audit Office report of September 2015 into the performance of the Cancer Drugs Fund, between 2009 and 2014, of the 39 cancer drug indications recommended for use by NICE, 38% were approved under the End of Life criteria.

The NAO report went on to note that just one drug, Avastin, accounted for almost 20% of patients supported by the CDF over the two years 2013/14 and 2014/15. It stated: *'In 2014/15 Avastin was approved for 4,520 patients, nearly two thirds of whom had colorectal cancer. Previously NICE had not recommended Avastin for a range of cancer indications, including breast, colorectal and ovarian cancer, on the grounds of a lack of robust data or the high cost per QALY gained.* ***Avastin was not eligible for consideration under NICE's end-of-life criteria,*** *across a range of cancer indications, due to the large number of patients affected.'*

The implication of removing the 7,000 population threshold for end-of-life consideration is that the number of cancer drugs given outright approval - recommended for routine use - at the first NICE TA committee meeting may increase, at least for more common cancers.

It may not, however, lead to drugs for more common cancers being assessed as part of a MAF. This is because paragraph 46 of the consultation makes clear that the CDF budget will only pay for the number of patients deemed necessary to generate the data required to make a decision at the end of the MAF process. The drug companies will be expected to make up the difference. If the total patient population were only a few hundred, it would be plausible that the company may choose to pay the difference in order to ensure their product is made available through the MAF. However, if the potential population size of the new drugs were several thousand, and NICE/NHS only deemed it necessary to collect data from 3-400 hundred patients in order to obtain enough data to make a decision at the end of the MAF process, that could mean the company having to pay for a lot more drugs. Companies may simply not choose to take part in the MAF at all. Or, as Q15 in the Q&A suggests, we may end up with a situation where not all patients would have access, only those deemed necessary to collect the data. This would widen inequality to drug access across the UK.

However, this change to the population threshold will be unlikely to make a difference for rare and less common cancers, including pancreatic cancer. Pancreatic Cancer UK believes that a wider range of reforms of the NICE appraisal process are needed to ensure rare and less common cancers, and cancers with unmet need, likewise benefit in future.

The APPG is particularly disappointed that the consultation does not also ask for comments on the other change to end-of-life criteria set out in the consultation document, namely changes to the 3 month threshold. Paragraph 29 states that there will be *'amendments to emphasise the discretion that exists for NICE Appraisal Committees to interpret the uncertainty criteria when considering a drug for inclusion in the CDF.'*

The changes to the 3 month rule are especially important for pancreatic cancer patients, where survival rates are extremely low and have remained virtually unchanged for 40 years, and where there are very few treatment options available. With average survival of between just 2-6 months for metastatic pancreatic cancer patients, additional survival gain of less than three months can still represent a relatively large increase in survival for patients to spend with their loved ones.

The APPG firmly believes that NICE TA committees must take into account this level of unmet need when deciding on whether to apply the 3 month rule and thus the end-of-life criteria and the higher QALY thresholds that means.

Instead of this much required change, the consultation simply proposes a very minor amendment, from:

'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'; (Para 28)

to

'There is sufficient evidence to indicate that the treatment ***has the prospect of offering an extension to life***, normally of at least an additional 3 months, compared with current NHS treatment.' (Appendix B, para 6.2.10).

How proof of prospective extension to life differs from the already existing wording, and what difference this might make to the number of positive recommendations is not clear.

Indeed, the wording seems to be pushing TA committees towards looking at the MAF/CDF route by talking about prospects.

Moreover, use of the word 'normally' can actually work both ways, acting as a disincentive to treat a drug as end-of-life, even if three month life extension were offered by a new treatments.

The APPG believes that there needs to be much greater clarity and guidance for TA Committees around what situations might lead to a drug with less than 3 months survival meeting the end-of-life criteria. In particular, we believe that it should be made clear that relative survival gain should be taken into account, together with the level of unmet need, including the average survival for patients of the cancer type the drug is being assessed for.

Guidance needs to make absolutely clear that TA committees can allow a drug to be considered under end-of-life rules even if it does not offer 3 months or more life extension. Moreover, guidance should specify that in cases where life extension of less than 3 months still represents a relatively large survival gain compared to the average survival rate for a given condition for which few treatment options are available, perhaps for a pre-determined set of conditions including as pancreatic cancer, they should <u>always</u> waive the 3 month rule. Perhaps a new threshold of 2 months should be set instead.

in short, we believe that the changes to end-of-life criteria as currently set out may lead to new cancer drugs for more common cancers being approved – which is welcome – but will not benefit rare and less common cancer types, nor cancers with a high level of unmet need.

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

X **Agree**
☐ **Disagree**
☐ **Unsure**

Please provide comments to support your response:

In addition to making sure more patients can access cancer drugs, the other key benefits of the CDF has been that it has allowed earlier access to new cancer drugs. A new NICE process to allow draft decisions to be made ahead of marketing authorisation, together with the CDF being used for interim funding until final guidance has been agreed, ensures that this early access to new, beneficial drugs for patients continues into the future. As such, we support this principle.

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**
X **Unsure**

Please provide comments to support your response:

In order to meet its new proposed timelines, the consultation suggests that draft guidance will need to be issued as early as possible with a NICE TA committee meeting before an *'opinion of the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has been published.'*

This will lead to the TA committee producing draft guidance on drugs before licensing. We support this draft guidance being issued as stated in our response to question 6.

More generally, we have concerns as to whether NICE has the capacity and resources to be able to deliver, in practice, the timelines as set out in the consultation. As the consultation does not deal with the transition process for assessing all drugs currently on the CDF, this lack of capacity could be even greater.

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?

X **Agree**
☐ **Disagree**
☐ **Unsure**

Please provide comments to support your response:

Clearly, if this interim funding is to come from the CDF, then it places pressure on the amount which can then be used as part of the MAF.

However, one of the benefits of the CDF was faster access to effective new drugs and we believe that this is a vital benefit to retain in the new system. Ensuring interim funding from point of marketing authorisation for those drugs that have already received a draft approval makes sense and acts as a backstop to concerns that NICE may not be able to make all final decisions within a 90 day period.

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?

It seems sensible not to provide interim funding for drugs that have not received a draft recommendation from NICE, as it could mean a drug might only be funded for a very short period of time. It would be hard for patients to accept that some patients were lucky enough to fall into a small window of uncertainty, and receive the treatment, only for the option to be removed within a matter of weeks.

However, this depends very much on the likelihood of NICE being able to carry out its appraisals and make its recommendations in a timely fashion. If it does not have the necessary resources to provide draft guidance, then it may well be the case patients will be missing out. Allowing interim funded as suggested in paragraph 38 might then have the effect of forcing NICE to make sure it does actually make its assessments in the required timeframe to avoid this from happening.

As regards the arguments put forward in paragraph 19 about the variant option having an impact on *'the fixed CDF budget'*, we do not feel able to comment with certainty on this aspect as nowhere in the document does it specify what that fixed budget is for future years.

On balance, we believe that the variant option should not be considered.

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?

We support moves to make off-label drugs available in appropriate cases and hope that a suitable process can be consulted on in detail in due course.

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**

X **Unsure**

Please provide comments to support your response:

As noted elsewhere, the consultation document does not give an indication as to what the 'fixed' level of the CDF budget will be. Being asked to comment on control mechanisms and their appropriateness is difficult without knowing what the size of the CDF might be and therefore how stringent control mechanisms might need to be.

Clearly there are issues around having a fixed level of CDF funding; these led to the old CDF having to increase the size of the Fund periodically, as well as to de-list drugs in order to stay within that budget. This new proposal makes clear drugs on a MAF will only be there temporarily and so there will be a constant refreshing of drugs paid for by the CDF

We support moves to require manufacturers to provide value for money and introducing some control mechanisms could help to achieve that, keep the CDF within budget and allow as many drugs as possible to be made available.

However, the methods outlined in the document seem complicated and offer little certainty for manufacturers: we would not want to see a system set up that led to manufacturers deciding not to submit drugs for NICE appraisal, or not to participate in the CDF, in future because of over stringent financial controls. From what we can see so far, this is currently a real risk/

We would expect that industry is heavily consulted throughout this consultation process in order to come to an agreement as to workable control mechanisms and minimise the risk of companies refusing to take part in the new system, which would be a big blow to patients.

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?

As per our reply to question 11, we support arrangements that will lead to manufacturers providing better value for money to the NHS.

However, the consultation proposals seem highly complex and we would suggest that complexity could make it more difficult for the system to operate in a transparent manner.

Moreover, as highlighted earlier, whilst we recognise the need for manufacturers to provide better value for money, we do not want to see a system so severe that manufacturers will opt out: this would be of huge dis-benefit to patients across the country.

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

We continue to have concerns about how new treatments for rare and less common and, especially, cancers of unmet need/with the worst survival rates are appraised. This consultation does not alleviate those concerns.

We also maintain that the usual 24 months for a drug to be appraised under a MAF is not long enough for drugs for rare conditions to be properly assessed. Even allowing flexibility in this area, some patient populations are so small that the level of evidence required by NICE might never be achieved.

Likewise, the changes to the 3 month threshold as part of the end-of-life criteria are minimal and will do little, if nothing, to ensure more drugs are recommended for use for cancers of unmet need.

As set out in previous answers, we therefore believe that there also needs to be a separate entry point into the NICE/CDF decision making process for drugs that might benefit rare cancers and for cancer types where there is a clear unmet need. This should mirror the PACE system employed by the SMC in Scotland. If a drug meets the rare/unmet need definition, then a more comprehensive patient and clinician engagement process should take place to delve deeper into the possible benefits the new drug might bring. The subsequent NICE TA committee should then have to give proper weight to the evidence emerging from that PACE process, together with the usual clinical- and cost-effectiveness evidence. This process should not add more than one month to the overall timetable, with the PACE process taking place BEFORE the first TA Committee meeting.

However, even if the appraisal mechanisms are altered to take into account our concerns above, we maintain that NICE needs a much fuller overhaul of its systems, including ICER/QALY system, if cancer drugs are to truly be approved on the scale patients need and deserve. This must include a way of enhancing the patient voice as part of the NICE process. From our conversations with patients, carers, charities and others, we know how many of them feel that NICE is not responsive to their experiences and knowledge at the present time.

As such we hope that whatever emerges from this CDF merger into the NICE system does not lead to that more comprehensive overhaul of NICE being delayed or even avoided.

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**
X **Unsure**

Please provide comments to support your response:

As noted throughout this document, we do not feel enough detail has been provided in the consultation proposals to give a definitive statement to this question.

However, overall, we do not believe that the system proposed in the consultation will lead to more drugs being made available to patients than the CDF has since 2010. In fact, it is almost certain there will be fewer drugs made available. The consultation Q&A document raises this prospect by stating that 'the proposals for the new CDF are not necessarily about more cancer medicines being recommended for use in the CDF, but about the right ones….'

We do not agree with that statement and believe that the CDF should be about more cancer drugs being available to patients, especially those with conditions where there are very few treatment options already, as is the case for pancreatic cancer patients.

Moreover, it is likely that as drugs currently on the CDF are reassessed by NICE under transition arrangements (outside the scope of this consultation), many will not receive funding in future, either by being moved into baseline commissioning or by being put on a MAF. And it is clear that this transition process will have to take place first before there is CDF funding available for new treatments to be considered for funding via the new MAF. As this process might not be complete until March 2017, this could potentially mean it will have been two years since new drugs were funded by the CDF – there have been only de-listings since January 2015.

We therefore continue to have serious concerns that the changes will not lead to more drugs - especially for rare and less common cancers, and for cancers of unmet need - being approved under the new system. We fear that for these cancer types, we may even return to pre-2010 levels of drug approvals.