Cancer Drug Fund Consultation Questions – *Responses in RED*

i. Proposal A – proposed change to the criteria for receiving a Quality of Life (QOL) score. (Appendix D: National Cancer Drugs Fund Prioritisation Tool; Section 2 – Quality of Life criteria, page 59)

Proposal A1

We propose changing the standard of evidence required for QOL scores. Under the current QOL scoring system, applicant drugs/indications can only receive a score of plus 2 or minus 2 if published QOL evidence is provided. The evidence criteria for scores of plus 1 or minus 1 is less clear and refer at different times to 'measureable evidence', 'clear evidence' and 'evidence'. We consider that this potentially leads to confusion and inconsistency.

To address this, we propose that only published and peer reviewed evidence be considered for the purposes of scoring QOL. Any evidence that is not published and peer reviewed will be scored 0.

We consider this will have two positive effects. Firstly, it will make clear and consistent the standard of evidence required for scoring QOL. Secondly, it will ensure that all QOL scores are underpinned by robust evidence.

Proposal A2

We also proposed amending the criteria for QOL scores of plus 1 and minus 1 to refer consistently to 'significant' rather than 'major' improvement/deterioration. We consider that this will ensure consistency.

Consultation questions

a. **Do you agree with, or have any comment on, proposal A1 – the requirement for evidence to be both published and peer reviewed in order for it to be given a QOL score?**
   I agree that evidence should be both published and peer reviewed in order for it to be given a QOL score.

b. **Do you agree with, or have any comment on, proposal A2 – the consistent use of 'significant' rather than 'major' as the standard for improvement/deterioration when scoring QOL?**

   I agree with the consistent use of the word significant, provided this refers to "clinical significance" rather than statistical significance. i.e. where rare cancer patients are concerned the numbers will always be low. The clinical significance of rare cancer patient's quality of life is the most significant feature. Please confirm this definition of significance.

ii. Proposal B – proposed change to the definition of rarity as used by the National CDF Panel when scoring the median drug cost per patient. (Appendix G: Terms of Reference of the NCDF Panel; Section 9 – NCDF Panel Review, paragraph 9.3, bullet point 4, page 81)

Certain drugs are developed specifically for treating rare cancers. We recognise that such drugs should receive a higher level of reimbursement, as they will only be used to treat a small number of patients. We therefore operate a two-tier system for scoring a drug's median cost per patient: one for drugs for rare cancers, and the other for all other drugs.

Currently a drug is considered to treat a 'rare' cancer if it is likely to be actually used to treat less than 100 patients per year in the NHS, taking into account all of its uses.

We propose amending the definition of rarity in two substantive ways (Proposals B1 and B2).

We consider that the proposed revised definition is more appropriate and more accurately determines which drugs have been developed to treat rare cancers.

Proposal B1

We propose replacing the reference to "likely to actually receive treatment" with "likely to be covered by the licensed indications". The effect of this is that all of the patients for whom the drug could potentially be used are taken into account, rather than just those who are likely actually to be treated by the drug.

We consider that potential use is a better measure of rarity than actual use. Drugs may have limited actual use for reasons other than the rarity of the cancers they treat. For example, a drug that could be used for non-rare cancer(s) may only have very limited actual use because of external factors.

Proposal B2

We also propose amending the definition so that a drug's use both within and outside the NHS will be considered when determining whether it is used to treat a rare cancer.

In assessing whether a drug is used to treat a rare cancer, we do not consider it appropriate to look only at its use within the NHS. This is because the use of such a drug, overall, may not be rare and so the financial considerations we are seeking to take into account do not apply.

Consultation questions

a. **Do you agree with, or have any comment on, Proposal B1 – the amendment of the definition of rarity to be used when scoring a drug's median cost per patient, so that a drug's potential use, rather than its actual use, is taken into account?**

I disagree. The wording used in the amendment of the definition of rarity is ambiguous and wonder if the CDF realise the devastating impact that such a definition will have on rare cancer patients?

I would like to propose an alternative phrasing option:

"Rarity is defined as less than 100 patients per year with a particular diagnosis, who are likely to be covered by the licenced indication(s) of the drug…."

Most rare cancers do not have drugs that are specifically designed for them. Rather, they take advantage of existing drugs that have been used in other indications, often more common cancers.

This being the case, if rarity is redefined by the CDF as the number of patients likely to be covered by the drug rather than the actual rare patient group numbers then very few rare cancers will be able to access drugs via the CDF.

This proposed classification seems to classify rare cancers as common because they are grouped with other diseases using the same drug?

This change will result in the majority of rare cancer patients not being able to access drugs via the CDF.

b. Do you agree with, or have any comment on, Proposal B2 – the amendment of the definition of rarity to be used when scoring a drug's median cost per patient, so that a drug's use, both within and outside the NHS, is taken into account?

I disagree with this amendment of the definition of rarity.

Most rare cancers do not have drugs that are specifically designed for them. Rather, they take advantage of existing drugs that have been used in other indications, often more common cancers.

This being the case, if rarity is redefined by the CDF as the number of patients likely to be covered by the drug rather than the actual rare patient group numbers then very few rare cancers will be able to access drugs via the CDF.

This proposed classification seems to classify rare cancers as common because they are grouped with other diseases using the same drug?

This change will result in the majority of rare cancer patients not being able to access drugs via the CDF.

e.g. GIST is rare and in the case of regorafenib there were 19 GIST patients prescribed this drug in the last year. Regorafenib is also licenced for colorectal cancer, a much more common cancer. Grouped in this way we will find ourselves no longer classified as rare and able to access regorafenib.

This proposal seems to formalise the mistake that we pointed out to the CDF in our previous appeal.

iii. Proposal C – proposed simplification of the processes for challenging decisions made by the NCDF Panel (Appendix G: Terms of Reference of the NCDF Panel; Section 9 – NCDF Panel Review, Section 10.11, page 86)

There are currently two parallel processes for challenging decisions made by the NCDF Panel:

- a complaints process for applicants and clinicians who are dissatisfied with the outcome of the NCDF Panel's decision to either include or exclude a drug from the national CDF list;

and

- a review process by which pharmaceutical companies that applied for inclusion of their drugs on the national CDF list can challenge the NCDF Panel's decision-making process.

We have extended the scope of the review process so that now both pharmaceutical companies and patient groups can challenge the NCDF Panel's decision-making process.

We now propose having a single process for challenging decisions made by the NCDF Panel. We consider having two, overlapping processes is unnecessary.

Consultation questions

Do you agree with, or have any comment on, proposal C – the creation of a single process for challenging decisions made by the NCDF Panel, as described in paragraphs 10 and 11 of Appendix G, of the proposed SOP for 2015/16?

I am delighted that the CDF appeals process now includes the opportunity for patients to challenge NCDF Panel decisions.

Please can you confirm that clinicians also have the opportunity to challenge NCDF Panel decisions along with applicants and patients as part of the single process for challenging decisions.