Response to Cancer Drugs Fund Consultation
February 2016

Background
The Cancer Drugs Fund (CDF) has provided access to treatments for around 84,000 cancer patients who may not otherwise have had access to them through the NHS. It is vital that the proposed changes to the CDF continue to deliver access to the best treatments for all patients diagnosed with cancer on an equitable and sustainable basis.

The CDF has been a positive development for sarcoma patients, providing access to new treatments not routinely available through the NHS. However, recent changes to the CDF have resulted in significantly reduced access to treatments for sarcoma patients.

Our position
Sarcoma UK – the bone and soft tissue cancer charity - welcomes this consultation on the future of the Cancer Drugs Fund.

Whilst we welcome a number of elements within the proposals, we believe that the proposals in their current form are unlikely to improve sarcoma patients' access to new and innovative treatments, and may even return to a pre-CDF situation. The proposals do not sufficiently address the challenges of appraising treatments for rare cancers such as sarcoma.

We welcome:
- The principle of faster access.
- A single system.
- Introduction of real world evidence.

Our concerns:
- Insufficient detail around the proposed new system.
- Insufficient adjustments to NICE Technology Appraisal processes to accommodate treatments for rare cancers such as sarcoma.
- NICE capacity to deliver.
- Lack of clarity about real world evidence and the level of discretion that can be used when considering data relating to rare cancers such as sarcoma.
- The consultation process for these changes to the CDF - too short and complex.

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?**
   Unsure.

   We welcome the principle of a managed access fund as a means of increasing both the availability of cancer drugs and the speed of access to new therapies. However, there is insufficient detail on how the fund will operate in practice, in particular around the new entry and exit criteria for drugs selected for conditional CDF funding. Clarification is also needed about how the processes will address the challenges of rare cancers such as sarcoma.

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.

   We welcome the intention of the proposals to make treatments routinely available more quickly to patients, and we support faster access through the CDF via NICE.

   However, further changes must be made to the NICE appraisal processes to better address the challenges of appraising treatments for rare cancers such as sarcoma. In particular, we would like more clarity around the level of discretion the Technology Appraisal committee will have and how this will work; whether there is involvement of clinical expertise from the specific cancer field under consideration; and how meaningful public and patient involvement will be achieved.

   It is also essential that NICE is given adequate resources to deliver this proposed system.

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?**
   Unsure.

   We do not believe that the 'appropriate modifications' to the TA process as set out in the proposals will achieve quicker or improved access for rare cancers such as sarcoma. More substantial modifications of NICE processes are needed to address the challenges of appraising treatments for rare cancers such as sarcoma. Unless these are made, the new system is unlikely to provide equity of access for patients to new treatments. Ideally, we would like a complete review of NICE processes in light of the new responsibility for appraising all cancer treatments.

   We welcome the introduction of real world evidence into the appraisal process along with the new category of 'conditional approval' which provides time for further data collection

to inform decision-making. This may go some way to address the imbalance for rare cancers such as sarcoma, but much more clarification is needed about how this will work in practice. The proposals lack any significant detail about the type of data that will be acceptable, for example Quality of Life, audit data etc, and the weight that will be given to this real-world evidence by the TA committee.

For cancers with small populations such as sarcoma, the 24 month timeframe for additional data collection is unlikely to be long enough to generate meaningful information and conclusions. Whilst the proposals imply that there could be flexibility around this, clarification is required. There is potential for linking extra data collection with clinical trial networks and academic units. However, there also needs to be flexibility around the type of data that will be accepted for rare cancers such as sarcoma. Clinical trial data is the gold standard, but has challenges for cancers with smaller patient populations, where trials may take a long time to recruit.

We would like clarity about the type of real world data that will be accepted by NICE and how patient organisations such as Sarcoma UK can contribute to this through their own research programmes and data collections.

The proposed new system has not addressed the NICE Quality Adjusted Life Year (QALY) thresholds. In the current NICE processes, many treatments for rare cancers such as sarcoma have difficulty meeting this threshold, which was one of the reasons for the establishment of the Cancer Drugs Fund.

The NICE appraisal system does not currently incorporate a sufficiently meaningful patient perspective. The patient role in Technology Appraisals needs to be reconsidered so that real consideration and weight is given to the patient perspective, as well as the involvement of a wider and larger group of skilled patients. The new PACE (Patient and Clinician Engagement) model used by the Scottish Medicines Consortium should be considered by NICE as a means to gather more detailed evidence from patients, patient representative groups and clinicians, as part of the case for/against funding a treatment. This requires a significant shift in NICE processes in relation to the type of data considered acceptable and methods of Public and Patient Involvement.

4. **Do you agree with the proposal that a new category of NIE 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 categories:**
    a. **Recommended for routine use;**
    b. **Recommended for use within the Cancer Drugs Fund**
    c. **Not recommended**

    Agree

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" will be removed from the criteria for the higher cost effectiveness threshold to apply?**
   Agree.

   However, we seek clarification about the proposed changes to the three-month threshold for the end-of-life criteria and the "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." Para 29

   We believe that there needs to be clear guidance to TA committees about the circumstances when a treatment can be considered under end-of-life rules even if it does not offer life extension of three months. This guidance should also direct the committee to consider life extension in relation to the average survival rate for that individual cancer (or sub-type) for which limited standard treatment options are available.

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

   We support this proposal as it should help bring about earlier access to new treatments. However, we would like further clarification about how this proposal will involve the patient voice. We are also concerned about how NICE will engage with patient organisations to facilitate their involvement in TA processes with very tight deadlines.

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 processes?**
   Unsure.

8. **Do you agree with the proposal that all drugs that receive a draft NICE recommendation for routine use, or for conditional use with the CDF, receive interim funding from the point of marketing authorisation until the final appraisal decision, normally within 90 days of marketing authorisation?**
   Unsure.

   This proposal retains the original aim of the CDF to provide faster access to new treatments and we are supportive of this. However, if the interim funding is from the CDF budget, we have concerns that this may reduce the amount available under the managed access fund.

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?

We support this as an alternative funding scenario for rare and less common cancers, although more clarity is needed around the potential impact on overall CDF funding (which requires detail of CDF budgets and how funds will be allocated.)

10. **Do you have any comment 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?**

    Around 10% of approvals through the Cancer Drugs Fund are for off-label treatments (Rare Cancer Foundation) and the Fund has been used successfully to increase access to new treatments off-label, which has greatly benefited patients with sarcoma. This is an area that needs to be addressed as a priority.

    We support the introduction of a separate system for off-label prescribing that would ensure access for patients with rare cancers such as sarcoma. We support a model which would establish a tumour-specific national clinical reference group with budget responsibility for off-label prescribing; monitoring usage; and ensuring evidence is collected. There is also potential to link with nationally approved treatment algorithms, linked to the SACT database.

    Any system must incorporate expert clinical and patient involvement from the specialist area into the decision making process, and collect robust evidence on the use of these treatments.

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?**
    Unsure.

    The proposal for budget management and investment control mechanisms are unclear and complex and it is difficult to comment on this proposal without having sight of the figures for the 'fixed' level of the CDF budget. However, we believe pricing and manufacturers' costs should be transparent and any financial control mechanisms should be applied fairly to ensure that patients with rare cancers such as sarcoma have equal access to new treatments.

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

    Effective collection of clinical outcomes data.

    A more robust consultation process on any future changes. This consultation process has been significantly flawed with a restricted timeframe for consultation and a highly

complex set of questions. The simplified version for the public was too late to enable proper engagement around these proposals.

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

Whilst we welcome a number of elements within the proposals, we believe that the proposals in their current form are unlikely to improve access to treatments for sarcoma patients.

The original CDF recognised that NICE processes were unsuited to appraising treatments for rare cancers such as sarcoma - cancers that affect small population numbers, where there is limited clinical trial data. This challenge has not been addressed in these new proposals. The adjustments proposed to the NICE TA processes are not sufficient to bring about an improved and more equitable system for rare cancers such as sarcoma. We would like to see a complete review of NICE appraisal processes and the QALY thresholds.

Sarcoma patients generally have poor survival rates, limited treatment options, and one of the poorest patient experience of all cancer types. Access to clinical trials is low. Patients are aware that new and potentially life enhancing treatments are on the horizon but they hold little hope that they will be able to access them in England. The proposed new system is unlikely to improve this situation.