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Rarer Cancers Forum

[redacted]
Macmillan Cancer Support

**Sent by email**

[redacted]
Appeals Committee Chair
National Institute for Health and Clinical Excellence

02 June 2009

Dear [redacted]

**Rarer Cancers Forum and Macmillan Cancer Support – response to initial appeal scrutiny**

*Bevacizumab (first line), sorafenib (first and second line), sunitinib (second line) and temsirolimus (first line) for the treatment of advanced and/or metastatic renal cell carcinoma*

**Appeal point 1 – The Appraisal Committee wrongly considered that the patient population for bevacizumab was not only patients with renal cell carcinoma, but other patients for cancers for which bevacizumab has marketing authorisations.**

We are pleased that the Appeal Committee Chair has upheld this appeal point. The supplementary guidance *Appraising life-extending, end of life treatments* is not clear about how the patient population is determined and therefore this is a matter of procedural unfairness. If the Appraisal Committee has interpreted the guidance correctly then we believe that, in this case, the decision is perverse, because, as outlined in our original appeal submission, bevacizumab has not been approved by NICE for use within the NHS for any of its other licence indications.

**We wish to clarify appeal points 2 and 4 as per the initial scrutiny letter and we will set out further points below**

**Appeal point 2 – Failure to appraise temsirolimous as an ultra orphan drug**

We are disappointed that you are not minded to uphold our appeal point on temsirolimous as an ultra-orphan drug. We are therefore providing further

evidence to show how the decision of not approving this treatment is perverse.

Point 6.1.4 of the *Guide to the Methods of Technology Appraisals* states that "Advice on social value judgements that should, generally, be considered by the Appraisal Committee are provided in the Institute's document 'Social value judgements: principles for the development of NICE guidance'". Point 4.4 in the second edition of *Social Value Judgements: principles for the development of NICE guidance,* states that "NICE does not expect to receive referrals from the Secretary of State for Health to evaluate 'ultra-orphan drugs' (drugs used to treat very rare diseases or conditions). This is because the Department of Health currently has other mechanisms to assess the availability of ultra-orphan drugs in the NHS." We therefore believe that it is perverse that temsirolimus was included in this appraisal at the scoping stage. It is clear that the Department of Health believes that ultra-orphan treatments should be considered in a different way to the standard NICE process, but this has not happened in this case leading to a perverse negative decision.

Point 6.1.3 in the *Guide to the Methods of Technology Appraisals* states that "when formulating its recommendations to the Institute, the Appraisal Committee has discretion to consider those factors it believes are most appropriate to each appraisal." The Appraisal Committee could therefore add additional weight to the health related quality of life improvements offered by this treatment and the tiny patient population, rather than the cost-effectiveness data.

The supplementary guidance *Appraising life-extending, end of life treatments* issued to the Institute in January 2009, and indeed the *Guide to the Methods of Technology Appraisals,* have no fixed threshold on cost-effectiveness tolerability. It is therefore possible for the Appraisal Committee to be flexible and allow higher ICERs for ultra-orphan drugs through this process. We would urge the Appeal Committee to ask the Appraisal Committee to reconsider the acceptability of the economic data for temsirolimous in light of the points above.

**Appeal point 4 – Unethical clinical trials**

We would like to clarify the point that we were making in our original appeal document in relation to unethical clinical trials.

The issue is with patients who receive a non-standard agent in clinical trial (e.g. another tyrosine kinase inhibitor). Once this patient's disease progresses and they then need a second line treatment it is currently not clear whether or not the patient could receive sunitinib. We believe that in these cases patients should be able to access sunitinib.

We hope that the further information provided in this letter will persuade you that these appeal points are valid and should be fully considered in the appeal process.

We look forward to your final decision.

Yours sincerely

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On behalf of the Rarer Cancers Forum and Macmillan Cancer Support