National Institute for
Health and Clinical Excellence

MidCity Place
71 High Holborn
London
WC1V 6NA

Tel: 0845 003 7780
Fax: 0845 003 7784

Email: nice@nice.org.uk
www.nice.org.uk

Sent by email

[redacted]
Rarer Cancers forum
Macmillan Cancer support

19 May 2009

Dear [redacted]

Final Appraisal Determination: 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

Thank you for lodging your appeal against the above Final Appraisal Determination.

Introduction

The Institute's appeal procedures provide for an initial scrutiny of points that an appellant wishes to raise, to confirm that they are at least arguably within the permitted grounds of appeal ("valid"). The permitted grounds of appeal are:

- Ground 1: The Institute has failed to act fairly and in accordance with its published procedures as set out in the Institute's Guide to the Technology Appraisal Process.
- Ground 2: The Institute has prepared guidance which is perverse in the light of the evidence submitted.
- Ground 3: The Institute has exceeded its powers.

This letter sets out my initial view of the points of appeal you have raised: principally whether they fall within any of the grounds of appeal, or whether further clarification is required of any point. Only if I am satisfied that your points contain the necessary information and arguably fall within any one of the grounds will your appeal be referred to the Appeal Panel.

You have the opportunity to comment on this letter in order to elaborate on or clarify any of the points raised before I make my final decision as to whether each appeal point should be referred on to the Appeal Panel.

I can confirm that there will be an oral hearing of your appeal.

Initial View

Ground one: Procedural Unfairness/Ground 2 perversity

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.

I agree that the possible contradiction in the guidance on appraising life extending end of life treatments should be considered by an appeal panel, and so this is a valid appeal point under ground 1.

If the policy was correctly understood and applied by the appraisal committee, then it would not be a valid ground of appeal to argue that that policy is itself perverse. That would be a matter that would have to be raised with the Institute corporately. If the policy was correctly understood but applied in a way that in this case had a perverse result, where there was some other approach also consistent with the policy that would not have had that result, then that could be a valid ground 2 appeal point.

2 Failure to appraisal temsirolimus as an ultra orphan drug.

As your letter notes, NICE does not have a special appraisal process for ultra orphan drugs. It cannot therefore be a valid ground 1 appeal point that NICE appraised temsirolimus under its standard procedure.

It is possible that there might be some aspect of ultra orphan status that it would be perverse not to reflect in guidance, but your letter does not as yet make a specific allegation in that regard. (I am afraid the one point it does make, that the budget impact would be very small, is not one NICE can take into account, as it relates to affordability). If you would like to consider whether there is a specific

point on perversity that you would like to make here I will evaluate it, but at present I do not think this is a valid ground 2 appeal point.

3. No standard second line treatment

Unless you can give more detail as to why the fact that sorafenib and sunitinib are new options was not considered in accordance with NICE's published procedures, or was considered in a way that was perverse, I am afraid I am not minded to agree that this is a valid appeal point.

4 Unethical clinical trials

I do not think I quite understood this point. I am not sure how the guidance would impact on the conduct of clinical trials, other than that if the products had been recommended it would be questionable whether any placebo controlled trial would have been ethical? As the products are not recommended I cannot immediately see how the guidance has an impact on whether or not any given trial design is or is not ethical?

I am not minded to agree this is a valid appeal point.

5 Equality

In order to be a valid appeal point, you will need to identify a group with some protected characteristic (for instance, gender, race, or disability) who will not be able to access treatment on the same terms as other patients. At present I cannot see that there is such a group?

I am not minded to agree this is a valid appeal point.

Conclusion

As I am minded to rule that at least one of your appeal points is valid, I will pass your appeal to the Appeal Panel for consideration.

If you wish to make any further comment on the points that I have indicated that I do not, at this preliminary stage, view as valid, please let me have these within ten working days from the date of this letter (Wednesday 3 June 2009). I will then reach a final decision on the validity of those points.

Yours sincerely

[redacted]

Appeals Committee Chair

National Institute for Health and Clinical Excellence