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 via email

[redacted]
Celgene ltd
Morgan House, Madeira Walk
Windsor
SL4 1EP

23 April 2010

Dear [redacted]

Final Appraisal Determination: Azacitidine for the treatment of myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia

Thank you for lodging Celgene's appeal against the above Final Appraisal Determination and for your letter of 16 April.

Introduction

As you have renumbered your initial scrutiny points, I will include in this letter the relevant comments from my initial scrutiny letter also renumbered, as well as my final view on the points I was initially minded to reject.

Ground one

1.1. Final scope

I was initially doubtful that there is an arguable procedure/fairness point here. Having considered your

letter of 16 April and as the issue of the comparator will need to be considered under ground 2 in any case, I now agree this point should be considered at the appeal hearing.

1.2 Ultra orphan indications

In my initial scrutiny letter I said:

I am afraid I cannot quite understand how it is that you say the SVJ document makes it clear that ultra-orphan drugs must be appraised in a different way or to different thresholds? It seems to say that it is not expected they will be appraised at all, and is silent on what if anything should be done differently if they are?

The citizen's council is an advisory body, and for its reports to become part of NICE's processes they must be adopted by NICE's board. I am not aware that the Board has adopted guidance on ultra orphan drugs, and if that is correct, it would not be open to the Committee to depart from normal processes and thresholds. I note the draft guidance to which you refer, but unless that has been adopted, I do not agree that a draft document from 2006 would be relevant to a decision taken in 2010. Lapatinib was rather a different case as the draft guidance was current, and indeed came into force before the appraisal was concluded. In any event you overstate the appeal panel's position. They remarked that "It might have been reasonable for the Institute not to apply the new policy to lapatinib at all, on the basis that the Final Appraisal Determination had been finalised before the policy was adopted." The actual basis on which the appeal was allowed was that, having decided to apply the policy, the manufacturer was not then allowed to make a submission on the effect of the policy in that case and that was unfair.

I am minded to conclude this is not a valid ground of appeal under either ground one or ground three

I have considered your additional points, but it is still my view that it is not arguable that there is a requirement of procedural fairness for NICE to have adopted a different approach to this technology than it does to other technologies. I note your “predetermination” point, but the comment quoted cannot amount to predetermination, at worst, it is an observation of the likely effect of applying the ordinary procedure. Further, if the document quoted was indeed submitted as a consultation response to the DoH, the appeal panel will have to assume that the position of ultra orphan drugs has been considered by the DoH and/or the Institute in light of the comments made, and the decision to refer this drug to the usual appraisal process was taken knowingly. Whether that was or was not a right decision to take is not an issue which an appeal panel of the Institute can comment on. It can only comment on whether the committee has acted fairly in addressing the decision it was asked take.

It therefore remains my view that this is not a valid ground of appeal.

Ground two

I have now agreed these are all valid points.

Ground 3

3.1 Change of scope

As noted above I have now agreed this point may go forward under ground one, and it follows it may go forward under ground three, although in practice I would doubt if it will add anything to consider it separately under this ground.

3.2 Ultra Orphan

For the reasons given above it remains my view this is not a valid argument under ground 3.

3.3 Human Rights

In my initial letter I said:

This is a valid appeal point. As it is almost entirely a legal point, and as neither the appeal panel nor the appraisal committee are legally qualified, I am concerned that merely referring the point as put to an appeal hearing may not generate the most robust scrutiny of the issue. I therefore suggest we proceed as follows. I will request the appraisal committee to make whatever observations they wish on the issue (if any) in writing some time in advance of the hearing. I will then ask the appeal panel's legal advisor to prepare a written note of provisional advice for the appeal panel. The appraisal committee's observations (if any) and the note of provisional advice will be shared with all appellants in advance of the hearing. In this way all sides will be aware in advance of the various positions being advanced and the hearing will, I hope, run more smoothly.

This remains my view.

Conclusion

The institute will now make arrangements for the appeal hearing. I agree to the presence of a stenographer employed by yourselves on the clear requirement that the transcript will be fully available and sent to the Institute and your co-appellants in a timely fashion

Yours sincerely

[redacted]

Appeal Committee Chair
National Institute for Health and Clinical Excellence