



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

██████████
MDS UK Patient Support Group
The MDS Foundation, Inc/MDS UK Patient Support Group
European Office
London, UK

31 March 2010

Dear ██████████

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

Thank you for lodging your combined 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 the appeal.

Initial View

By way of guidance for any future appeals, if you could number or mark your appeal points this is helpful to sure the appeal panel keeps track of all of them.

Ground 1

A QoL evidence was offered but ignored.

I agree this is a valid ground one appeal point

B There was a failure to consult in an open and transparent manner

I agree this is a valid ground one appeal point, but it contains elements of a ground two complaint (see below). it would be helpful to the panel if you take care to limit your comments under this specific heading to an unfair process only.

Ground 2

C Key evidence was ignored

I agree this is a valid ground two appeal point.

D Perverse to rely on BSC as the only comparator

I agree this is a valid ground two appeal point

E Perverse given the 9.5 month extension to life

I am not sure this is a valid point. Life extension would be captured in the calculation of an ICER and the application of the EoL criteria, and to then give it additional weight again would seem to be double counting. The relatively small financial burden is an affordability argument and not one which NICE has the remit to consider. And whatever the pros and cons may be of an argument that ultra orphan drugs should be given more weight, NICE's current process does not allow for this, and i do not feel it could be arguably perverse not to have done so.

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

Ground 3

F Breach of the Human Rights Act / unethical

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.

Conclusion

As I am minded to rule that at least some of your appeal points are 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, or that I have re-cast, please provide to me this within 10 working days from the date of this letter, no later than **Friday 16 April**. I will then reach a final decision on the validity of those points.

Yours sincerely


Appeals Committee Chair
National Institute for Health and Clinical Excellence

Cc 