



26th April 2010

By First Class Post and Email

████████████████████
National Institute for Health and Clinical Excellence
MidCity Place
71 High Holborn
London
WC1V 6NA

Dear ██████████,

Response to Final Scrutiny Letter

Thank you for your letter dated 23 April 2010. We are disappointed that you will not allow our ground for appeal that the Institute has acted perversely in not recommending the azacitidine given the significant life-extension offered by the drug.

Further, we have grave doubts over your comments about ultra-orphan medicines. You state that “the appeal panel can only ask itself whether the **committee** fairly appraised the drug referred to it in accordance with its published procedures. Whether the drug should have been referred, or whether the procedures should be different, are not matter’s on which the **committee** can have a view and so not matters which it can be challenged about on appeal”.

We have highlighted the references to “the committee” above because our appeal is against the Institute, *i.e.*, NICE, and not the appraisal committee. This is clear from NICE’s guidance to appellants, which states at paragraph 3.4.2:

The grounds of appeal are as follows.

- 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.
- 2 **The Institute** has prepared a FAD that is perverse in the light of the evidence submitted.
- 3 **The Institute** has exceeded its powers. (Emphasis added.)

We understand that several NICE representatives will be representing the Institute at the appeal hearing and we do not necessarily expect the appraisal committee to be challenged on this particular point. In relation to the published procedures, we would also refer you to

NICE's social value judgments, which make reference to ultra-orphan drugs. We continue to feel strongly that this is a valid point for appeal and that it is a matter for the appeal panel to decide.

We are very concerned that the rejection of this grounds based on your comments above is itself unfair and unlawful and we would strongly urge you to reconsider your position. If you will not, we would be grateful if we could address this refusal in our appeal. Based on the Institute's guidance for appellants, we consider that it would be a basis for an appeal under grounds 1 and 3.

We remain available for any further assistance that we can give you.

Yours sincerely

[REDACTED]

Chairman, MDS UK Patient Support Group

[REDACTED]

Deputy Chairman, MDS UK Patient Support Group