



14<sup>th</sup> 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 Scrutiny Letter**

Thank you for your letter dated 31 March 2010 responding to our grounds for appeal. We are grateful that you consider the majority of our arguments to be valid. Regarding your comments over end-of-life and perversity, we feel strongly that this point is at least arguable and should be put before the Appeal Panel under Ground 2 for the reasons below.

The FAD states at paragraph 4.13 that the “additional weight that would need to be assigned to the original QALY benefits in this patient group for the cost effectiveness of the drug to fall within the current threshold range would be too great”. We are not arguing that there has been some procedural flaw in the application of the end-of-life criteria as your scrutiny letter suggests. Rather, we are arguing that given the 9.5 month extension to life and the ultra-orphan nature of the drug, the decision not to recommend azacitidine is perverse, particularly when you consider that azacitidine offers a much greater extension to life than any other end-of-life treatment appraised by NICE to date as far as we are aware.

Further, we do agree with your comments on the ultra-orphan nature of the medicine in this context. It is not clear to us that NICE is barred from considering such drugs differently and/or placing greater weight on ICERs for such drugs. Indeed, the guidance and position papers available on NICE’s website on ultra-orphan drugs suggest to us that there is scope for NICE to deal with such drugs differently if in fact the Institute should deal with them at all.

At the very least, we believe our comments on ultra-orphan medicines should be put before the Appeal Panel in the context of the perversity argument above. Your comments regarding affordability are noted but this does not affect our perversity argument.

In summary, we feel strongly that our end-of-life argument under Ground 2 is arguable and important from a patient perspective and sincerely hope that it is put before the Appeal Panel along with our other arguments.

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