



**Royal College  
of Physicians**

Setting higher medical standards

11 St. Andrews Place  
Regent's Park, London NW1 4LE

Telephone +44(0) 20 7935 1174  
Textphone +44(0) 20 7486 5687  
Facsimile +44(0) 20 7487 5218

[www.rcplondon.ac.uk](http://www.rcplondon.ac.uk)

Dr Carole Longson  
Director, Centre for Health Technology Evaluation  
National Institute for Health and Clinical Excellence  
MidCity Place  
71 High Holborn  
London  
WC1V 6NA

**From The Registrar**

Telephone extension [REDACTED]  
Direct facsimile [REDACTED]  
[REDACTED]@rcplondon.ac.uk

25<sup>th</sup> November 2010

Dear Dr Longson

**Re: Single technology appraisal (STA) Azacitidine for the treatment of myelodysplastic syndrome, chronic myelomonocytic leukaemia, and acute myeloid leukaemia - Appraisal consultation document**

I write on behalf of the NCRI/RCP/RCR/ACP/JCCO with relation to this ACD consultation. We are grateful for the opportunity to respond and would like to make the following comments.

The recommendation to not support the use of azacitidine in the NHS in England is naturally disappointing. Whilst we accept that NICE is working within its mandate, it is clear that drugs to treat orphan diseases such as high-risk MDS may need to be considered separately from high cost high disease incidence agents.

**Has all of the relevant evidence been taken into account?**

- Yes. The evidence supplied by the manufacturer and by the UK MDS Support Group following the appeal has been considered by the DSU. There is no more evidence available to our knowledge.

**Are the summaries of clinical and cost-effectiveness reasonable interpretation of the evidence?**

- Yes. Whilst one could argue that there is a group of patients in whom a preference would be expressed by most Haematologists for therapy with low dose cytarabine rather than supportive care or intensive chemotherapy, this group cannot be precisely defined and as such will overlap with the other groups. In this regard, the criteria defined by the manufacturer following extensive consultation with the UK experts and review of the literature are reasonable but not precise. The Appraisal Committee continues to accept the clinical effectiveness of azacitidine. The data used to create the weighted average ICER are reasonable and based on the best estimate of management of MDS in the community hospitals in UK, namely the HMRN dataset.



**Are the provisional recommendations sound and a suitable basis for guidance to the NHS?**

- Within the mandate of NICE these recommendations are sound. However, they compare less favourably with international practice.

**Are there any aspects of the recommendations that need particular consideration?**

- No. This has been extensively discussed in the appeal process

Yours sincerely

[REDACTED]

[REDACTED]