16th March 2010

Dear [Name],

Re: Single technology appraisal (STA) - Azacitidine for the treatment of myelodysplastic syndrome, chronic myelomonocytic leukaemia, and acute myeloid leukaemia - Final appraisal determination (FAD)

I write on behalf of the NCRI Haematological Oncology Clinical Studies Group/RCP/RCR/ACP/JCCO with regard to this FAD consultation. We are pleased to be invited to comment and would like to submit an appeal as detailed below.

**Grounds for appeal**

2. The Institute has prepared guidance that is perverse in the light of the evidence submitted.

**Basis of appeal**

a) NICE has considered only Best Supportive Care as the comparator to azacitidine. The primary dataset on which the submission from Celgene and the ERG analysis is based is the AZA001 phase 3 trial in which 41% patients received chemotherapy and 62% patients Best Supportive Care. Within Celgene’s response to the interim recommendation is a table reflecting UK practice in this regard. Although there is considerable variability between the 11 units surveyed, 57% patients were said to be treated with chemotherapy (range 20-100%) compared with 43% treated with best Supportive care only (range 0-80%). Thus in no UK unit was Best Supportive Care the only modality of therapy used to treat the target MDS population. It appears perverse to use only Best Supportive Care in the health economic model and to ignore the widely used chemotherapy option.

b) NICE accepts that azacitidine therapy fits their End of Life criteria but fails to indicate the influence that correction for these criteria might have on the ICER and therefore the conclusions for cost effectiveness. This is again perverse in apparently ignoring the End of Life criteria.

**Supporting comments**

a) The decision to not recommend for use within the NHS a drug with proven efficacy to prolong survival will contribute to the poor survival of older cancer patients in the UK compared to other European countries. A major component of the Cancer Reform Strategy is to address this survival deficit in relation to our European colleagues. This NICE decision appears to directly oppose this policy.
b) MDS affects predominantly elderly people (median age = 75 years) and depriving such patients of life-prolonging treatment is inappropriate.

c) There is a risk that the UK will be perceived as no longer competitive in terms of a clinical research environment when compared to continental Europe and the US. Despite the best efforts of the NCRN and NIHR to reverse this decline, decisions such as this from NICE are likely to further discourage investment into the UK.

Please feel free to contact me if you require clarification of the above.

Yours sincerely

[signature]
Registrar