

NICE STA - Azacitidine for the treatment of myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia

Comments from the Royal College of Pathologists and the British Committee for Standards in Haematology on the second Appraisal Consultation Document (ACD)

I believe that the role of azacitidine in MDS and AML has been analysed in enormous detail during a NICE process that has now been running for twenty months. I suppose we should acknowledge that more than 1000 patients will have been diagnosed with a form of high risk MDS that might be suitable for azacitidine therapy during that time period. The recent ACD, following the successful appeal against the FAD, is comprehensive and confirms once again the clinical effectiveness yet relative expensive nature of the treatment in terms of the predicted incremental cost per QALY. I cannot argue with the findings of the ACD which is very thorough and I will not cover all the ground again. However, I feel the need to re-iterate most strongly my total support for the provision of azacitidine for appropriate MDS and AML patients in the UK. The recent Executive meeting of The UK MDS Forum on November 15th 2010 emphasised the utter confusion and inequality across the UK in terms of access to this drug. Some patients in England now have ready (though perhaps temporary) access to the drug via the new Interim Cancer Drug Fund. The lists produced by clinically-led panels for strategic health boards have, to date, ranked azacitidine very highly amongst drugs to be funded by this mechanism. This is to be welcomed and is a 'real world' judgement of this drugs worth and emphasises to the NICE appraisal committee the importance attached to this drug by clinicians and commissioners providing NHS care. However, some of the ten strategic health boards in England have yet to draft a list and hence azacitidine remains largely unavailable in these regions. In Scotland the drug is not available because of a negative SMC decision with no prospect of this being re-visited until the NICE process is completed. In Wales and Northern Ireland the drug is also currently unavailable. A positive NICE decision is clearly very important in providing a more level playing field for access to this drug across the UK.

I still believe that a negative decision represents indirect discrimination against elderly people. This is because the licensed indication for azacitidine is for patients 'ineligible for stem cell transplant.' Denying the licensed indication, therefore, has a disproportionately larger impact on elderly people than on younger people with the identical diagnosis, in terms of access to effective therapy, because elderly people cannot receive stem cell transplantation. From my experience of training on equality legislation, this scenario seems very similar to examples that are commonly cited as types of indirect discrimination. Furthermore, there are examples of some PCTs providing access to azacitidine as a 'bridge to transplant.' This represents a somewhat lateral interpretation of the licensed indication and compounds the inequality towards elderly patients in

whom this drug is frequently their only hope of effective therapy. I accept that a legal opinion has been sought on this, but I remain unconvinced that this would not be considered a strong example of indirect discrimination by many people presented with this scenario.

As stated in my original written evidence, I would emphasise once again the nihilism that currently exists for the treatment of these elderly people with MDS. The denial of the first and only widely applicable effective treatment for this group of patients would re-enforce this nihilism. It would also strongly re-enforce the recently publicised very negative perception that elderly patients with malignant diseases are poorly served in the UK. A negative decision from NICE will certainly add MDS to the list of diseases, predominantly affecting the elderly, with relatively poor survival compared to many of our European neighbours.

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