

Single technology appraisal (STA)

Azacitidine for the treatment of myelodysplastic syndrome, chronic myelomonocytic leukaemia, and acute myeloid leukaemia

Final appraisal determination

Appeal on behalf of the Royal College of Pathologists and the BSH

Grounds for appeal

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

Basis of Appeal

Whilst it is acknowledged that the majority of patients currently receive best supportive care as standard treatment, there is clearly a significant minority of patients who receive either intensive remission induction chemotherapy or low dose cytosine arabinoside. In the pivotal study (AZA 001) used as the basis for Celgene's submission and for the ERG analysis informing the single technology appraisal, 41% of patients were allocated to randomization to some form of chemotherapy. Also, evidence submitted by Celgene in their response to the interim guidance includes a survey of practice in eleven UK centres. These data state that between 20-100% of patients are offered treatment with chemotherapy and clearly, therefore, best supportive care is never the only modality of treatment offered in any of these centres.

It appears perverse in the light of this submitted evidence to only include best supportive care in the final economic model. Rather, the data should be modeled to take into account a realistic proportion of patients receiving chemotherapy.

The final appraisal determination acknowledges that high risk MDS and AML patients treated with azacitidine fulfill the end of life criteria. However, the report does not show the calculated effect on the ICER and this is also perverse.

Additional Comments

As we have previously stated high risk MDS is a disease of elderly people with a median age of approximately 74 years. Younger patients with high risk MDS and AML can be successfully treated without resort to azacitidine by allogeneic stem cell transplantation which is a treatment that is both readily available and highly expensive. However, this treatment is not available to the vast majority of older patients because of the associated morbidity and mortality risk. Therefore, denying this group of patients azacitidine, which is the only clinically effective therapy currently licensed for, and tolerated by, elderly patients appears to directly discriminate against elderly people in the provision of effective care. Furthermore, this decision not to recommend azacitidine for use within the NHS will clearly lead to the poorer survival of elderly patients suffering with these malignancies compared to mainland Europe. This is directly at odds with the Government's stated aim of improving cancer survival in elderly British patients through the Cancer Reform Strategy. The decision seems particularly perverse in the light of a statement made by ██████████ National Cancer Director, in response

to data showing relatively poor cancer survival amongst elderly patients in the UK: 'We need to ensure that cancer patients of all ages are diagnosed as early as possible and receive appropriate treatment. The findings have already been shared with the National Cancer Equality Initiative and we will be working with the NHS and other interested parties to tackle any age inequalities.'