

PRESS RELEASE

NICE appraisal of azacitidine for myelodysplastic syndromes

The National Institute for Health and Clinical Excellence (NICE) is currently appraising the use of azacitidine (Vidaza, Celgene) for myelodysplastic syndromes. In the final draft guidance, published today (4 March 2010), NICE has not recommended azacitidine as a treatment option for people who have the following conditions, and are not eligible for haematopoietic stem cell transplantation:

- intermediate-2 and high-risk myelodysplastic syndromes
- chronic myelomonocytic leukaemia
- acute myeloid leukaemia

In line with the NICE technology appraisals process this draft guidance is now with consultees, who have the opportunity to appeal against the proposed guidance. NICE has not yet issued final guidance to the NHS. Final guidance is expected to be published in May 2010.

Myelodysplastic syndromes (MDS) are a group of bone marrow disorders, where the marrow doesn't produce enough of one or more types of blood cells. The majority of patients with MDS receive best supportive care in current clinical practice.

Dr Carole Longson, Health Technology Evaluation Centre Director at NICE said: "Azacitidine is the first drug that has been developed specifically for treating MDS. It is not a cure, but could potentially prolong the life of people with these conditions by around nine months longer than standard treatment.

"We are disappointed not to be able to recommend this drug. The independent Appraisal Committee considered all published evidence on the effectiveness of azacitidine and the cost, including the proposed 'patient access scheme'. The

Appraisal Committee concluded that relative to the benefits, the price the NHS is being asked to pay for azacitidine, is still too high for it to be recommended as a cost effective use of NHS resources.

“The committee agreed that azacitidine did fit the criteria to be considered under the supplementary advice for end of life medicines; however, the magnitude of additional weight that would need to be assigned to the original QALY for the cost effectiveness of the drug to fall within the current threshold range would be too great, even when the patient access scheme was incorporated.”

Ends

For more information call the NICE press office on 0845 003 7782.

Notes to Editors

About the appraisal

1. The guidance is available at: <http://guidance.nice.org.uk/TA/Wave18/19>
2. According to the manufacturer's estimates, azacitidine costs approximately £45,000 per patient.
3. The manufacturer of azacitidine has agreed a patient access scheme with the Department of Health, in which azacitidine for the treatment of myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia will be available with a 7% reduction in the acquisition cost.
4. The most plausible ICER for azacitidine in the general patient population was approximately £63,000 per QALY gained
5. The Committee also felt that the evidence related to the potential effect azacitidine could have on patients' quality of life is weak and it has included a recommendation in the guidance for more research into this area, with the hope that this evidence base will improve.

About NICE

6. The National Institute for Health and Clinical Excellence (NICE) is the independent organisation responsible for providing national guidance on the promotion of good health and the prevention and treatment of ill health.
7. NICE produces guidance in three areas of health:
 - **public health** – guidance on the promotion of good health and the prevention of ill health for those working in the NHS, local authorities and the wider public and voluntary sector
 - **health technologies** – guidance on the use of new and existing medicines, treatments and procedures within the NHS
 - **clinical practice** – guidance on the appropriate treatment and care of people with specific diseases and conditions within the NHS.