



**National Institute for  
Health and Clinical Excellence**

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[www.nice.org.uk](http://www.nice.org.uk)

## **PRESS RELEASE**

### **NICE recommends new treatment for myelodysplastic syndromes**

NICE has today issued final guidance recommending azacitidine (Vidaza, Celgene) as a treatment option for people with myelodysplastic syndromes (MDS).

Azacitidine has been recommended in line with its licensed indications, which means that it is now a treatment option for patients that have one of the following conditions and who are not eligible for haematopoietic stem cell transplantation: intermediate-2 and high-risk myelodysplastic syndromes, chronic myelomonocytic leukaemia or acute myeloid leukaemia.

**Dr Carole Longson, Health Technology Evaluation Centre Director at NICE**

**said:** “We are pleased to be able to recommend this new treatment for MDS – it is not a cure, but it has the potential to extend life expectancy for these patients. During consultation on the draft recommendations, the manufacture of azacitidine offered to provide the drug at a reduced price. Azacitidine is an expensive drug, and this discount enabled us to recommend it as a cost effective use of resources on the NHS.”

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, and some patients receive low-dose or standard-dose chemotherapy. There are approximately 700 patients in England and Wales with the type of MDS for which azacitidine is licensed.

**Ends**

For more information call the NICE press office on 0845 003 7782 and out of hours on 07775 583 813.

## Notes to Editors

### About the appraisal

1. The guidance will be available from 23 March at: [www.nice.org.uk/guidance/TA218](http://www.nice.org.uk/guidance/TA218)
2. Azacitidine is the first drug that has been developed specifically for treating myelodysplastic syndromes. It is not a cure, but it does have the potential to extend patients' lives. The clinical trial reported an average life extension of nine months.
3. The Committee agreed that azacitidine met the criteria for being a life-extending, end-of-life treatment and considered that, on balance, the additional weight that would need to be assigned to the QALY benefits in this patient group for the cost effectiveness of azacitidine to fall within the current threshold range was acceptable.
4. The committee agreed that the most plausible ICER for azacitidine in the overall patient population was approximately £47,200 per QALY gained. This included the patient access scheme.
5. The Department of Health and the manufacturer have agreed that azacitidine will be available to the NHS with a patient access scheme in which a discount is applied to all invoices. The level of the discount is confidential. It is the responsibility of the manufacturer to communicate the level of discount to the relevant NHS organisations.

### About NICE

4. The National Institute for Health and Clinical Excellence (NICE) is the independent organisation responsible for providing national guidance and standards on the promotion of good health and the prevention and treatment of ill health
5. 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, medical technologies (including devices and diagnostics) and procedures within the NHS
  - **clinical practice** – guidance on the appropriate treatment and care of people with specific diseases and conditions within the NHS.
6. NICE produces standards for patient care:
  - **quality standards** – these reflect the very best in high quality patient care, to help healthcare practitioners and commissioners of care deliver excellent services
  - **Quality and Outcomes Framework** – NICE develops the clinical and health improvement indicators in the QOF, the Department of Health scheme which rewards GPs for how well they care for patients
7. NICE provides advice and support on putting NICE guidance and standards into practice through its **implementation programme**, and it collates and accredits high quality health guidance, research and information to help health professionals deliver the best patient care through **NHS Evidence**.