European Medicines Agency
Pre-Authorisation Evaluation of Medicines for Human Use

London, 23 October 2008
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COMMITTEE FOR MEDICINAL PRODUCTS FOR HUMAN USE
SUMMARY OF POSITIVE OPINION*
for
VIDAZA

International Nonproprietary Name (INN): ***azacitidine***

On 23 October 2008 the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion,** recommending to grant a marketing authorisation for the medicinal product Vidaza, 25mg/ml, powder for suspension for injection intended for the treatment of myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML) in adults who are not eligible for haematopoietic stem cell transplantation. Vidaza was designated as an orphan medicinal product for MDS on 6 February 2002 and for AML on 29 November 2007. The applicant for this medicinal product is Celgene Europe Limited.

The active substance of Vidaza is azacitidine, a pyrimidine analogues medicinal product (L01BC07) which is believed to exert its antineoplastic effects by multiple mechanisms including cytotoxicity on cells in the bone marrow and hypomethylation of DNA.

The benefits with Vidaza are its clinical efficacy in terms of survival benefit observed in a randomised clinical trial comparing azacitidine plus best supportive care with conventional care regimen. The most common side effects reported with azacitidine treatment were haematological reactions including thrombocytopenia, neutropenia and leukopenia, gastrointestinal events including nausea, vomiting or injection site reactions.

A pharmacovigilance plan for Vidaza, as for all medicinal products, will be implemented as part of the marketing authorisation.

The approved indication for Vidaza is:

For the treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with:

• intermediate-2 and high-risk myelodysplastic syndromes (MDS) according to the International Prognostic Scoring System (IPSS),

• chronic myelomonocytic leukaemia (CMML) with 10-29% marrow blasts without myeloproliferative disorder

• acute myeloid leukaemia (AML) with 20-30% blasts

It is proposed that Vidaza is prescribed by physicians experienced in the use of chemotherapeutic agents for the treatment of MDS.

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* Summaries of positive opinion are published without prejudice to the Commission Decision, which will normally be issued within 67 days from adoption of the Opinion.

** Applicants may request a re-examination of any CHMP opinion, provided they notify the EMEA in writing of their intention to request a re-examination within 15 days of receipt of the opinion.

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Detailed recommendations for the use of this product will be described in the Summary of Product Characteristics (SPC) which will be published in the European Public Assessment Report (EPAR) and will be available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers that there is a favourable benefit to risk balance for Vidaza and therefore recommends the granting of the marketing authorisation.