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EPAR summary for the public

Vidaza

azacitidine

This is a summary of the European public assessment report (EPAR) for Vidaza. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Vidaza.

What is Vidaza?

Vidaza is a medicine that contains the active substance azacitidine. It is available as a powder to be made up into a suspension for injection.

What is Vidaza used for?

Vidaza is used for the treatment of adults with the following diseases, if they cannot have haematopoietic stem cell transplantation (when the patient receives stem cells to restore the bone marrow's ability to produce healthy blood cells):

- myelodysplastic syndromes, a group of conditions where too few blood cells are produced by the bone marrow. In some cases, myelodysplastic syndromes can lead to acute myeloid leukaemia (AML, a cancer affecting white blood cells called myeloid cells). Vidaza is used in patients with an intermediate to high risk of progressing to AML or death;
- chronic myelomonocytic leukaemia (CMML, a cancer affecting white blood cells called monocytes). Vidaza is used when the bone marrow consists of 10 to 29% abnormal cells and the bone marrow is not producing large numbers of white blood cells;
- AML that has developed from a myelodysplastic syndrome and the bone marrow consists of 20 to 30% abnormal cells;
- AML, when the bone marrow has more than 30% abnormal cells.

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Because the number of patients with these diseases is low, the diseases are considered ‘rare’, and Vidaza was designated an ‘orphan medicine’ (a medicine used in rare diseases) on 6 February 2002 for myelodysplastic syndromes and on 29 November 2007 for AML. At the time of orphan medicine designation, CMML was classified as a type of myelodysplastic syndrome.

The medicine can only be obtained with a prescription.

How is Vidaza used?

Vidaza treatment should be started and monitored under the supervision of a doctor experienced in the use of cancer medicines. Patients should receive medicines to prevent nausea (feeling sick) and vomiting before giving Vidaza.

The recommended dose of Vidaza is 75 mg per square metre body surface area (calculated using the patient’s height and weight). It is given as an injection under the skin of the upper arm, thigh or abdomen (tummy) every day for one week, followed by three weeks with no treatment. This four-week period is one ‘cycle’. Treatment continues for at least six cycles and then for as long as it benefits the patient. The liver, kidneys and blood should be checked before each cycle. If the blood counts fall too low or if the patient develops kidney problems, the next treatment cycle should be delayed or a lower dose should be used.

See the summary of product characteristics (also part of the EPAR) for full details.

How does Vidaza work?

The active substance in Vidaza, azacitidine, belongs to the group ‘anti-metabolites’. Azacitidine is an analogue of cytidine, which means that it is incorporated into the genetic material of cells (RNA and DNA). It is thought to work by altering the way the cell turns genes on and off and also by interfering with the production of new RNA and DNA. These actions are thought to correct the problems with the maturation and growth of young blood cells in the bone marrow that cause myelodysplastic disorders, and to kill cancerous cells in leukaemia.

How has Vidaza been studied?

Vidaza has been studied in two main studies. The first study involved 358 adults with intermediate to high-risk myelodysplastic syndromes, CMML or AML, who were unlikely to go on to have a stem cell transplant. The patients’ bone marrow contained up to 30% abnormal cells. The second study involved 488 patients with AML who were 65 years or older and could not have haematopoietic stem cell transplantation. Their bone marrow contained more than 30% abnormal cells. Both studies compared Vidaza with conventional care (treatment chosen for each patient based on local practice and the patient’s condition). The main measure of effectiveness was how long the patients survived.

What benefit has Vidaza shown during the studies?

Vidaza was more effective than conventional care in extending survival. In the first study, patients receiving Vidaza survived for an average of 24.5 months, compared with 15.0 months in patients receiving conventional care. The effect of Vidaza was similar in all three diseases.

In the second study in AML patients with more than 30% abnormal cells, patients receiving Vidaza survived for an average of 10.4 months, compared with 6.5 months in patients receiving conventional care.

What is the risk associated with Vidaza?

The most common side effects of Vidaza in more than 60% of patients with myelodysplastic syndromes, CMML or AML (20 to 30% abnormal cells) are blood reactions including thrombocytopenia (low platelet counts), neutropenia (low levels of neutrophils, a type of white blood cell) and leucopenia (low white blood cell counts), side effects affecting the stomach and gut including nausea and vomiting, and injection site reactions. Side effects were similar in AML patients with more than 30% abnormal cells. For the full list of all side effects reported with Vidaza, see the package leaflet.

Vidaza must not be used in patients with advanced liver cancer or in women who are breastfeeding. For the full list of restrictions, see the package leaflet.

Why has Vidaza been approved?

The CHMP decided that Vidaza's benefits are greater than its risks and recommended that it be given marketing authorisation.

What measures are being taken to ensure the safe and effective use of Vidaza?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Vidaza have been included in the summary of product characteristics and the package leaflet.

Other information about Vidaza

The European Commission granted a marketing authorisation valid throughout the European Union for Vidaza on 17 December 2008.

The full EPAR for Vidaza can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Vidaza, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summaries of the opinion of the Committee for Orphan Medicinal Products for Vidaza can be found on the Agency's website: here (myelodysplastic syndromes) and here (AML).

This summary was last updated in 07-2016.