



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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF decitabine for the treatment of myelodysplastic syndromes

On 14 February 2003, orphan designation (EU/3/03/135) was granted by the European Commission to Eurogen Pharmaceuticals Ltd, U.K., for decitabine for the treatment of myelodysplastic syndromes. The sponsorship was transferred to MGI Pharma Ltd, United Kingdom, in March 2006 and subsequently to Janssen-Cilag International NV, Belgium, in March 2007.

What are the myelodysplastic syndromes ?

Myelodysplastic syndromes are diseases in which the bone marrow does not function normally and not enough normal blood cells are made. The bone marrow is the spongy tissue inside the large bones in the body. The bone marrow makes red blood cells (which carry oxygen and other materials to all tissues of the body), white blood cells (which fight infection), and platelets (which make the blood clot). Myelodysplastic syndromes are life-threatening.

What are the methods of treatment available?

There was no satisfactory treatment authorised in the European Union for this medical condition at the time of submission of the application for orphan drug designation. Treatments that have sometimes been used for myelodysplastic syndromes include symptomatic supportive care, the short-term use of growth factors such as erythropoietin (a substance which causes the marrow to make red blood cells), chemotherapy with possible adverse effects, and bone-marrow transplant applicable to a minority of patients.

What is the estimated number of patients affected by the condition*?

According to the information provided by the sponsor, myelodysplastic syndromes were considered to affect between 38,000 and 117,000 persons in the European Union at the time the designation application was made.

How is this medicinal product expected to act?

Decitabine (5-aza-2'-deoxycytidine) is a chemical substance, which is related to cytidine. Cytidine is part of in the fundamental genetic material of cells (DNA and RNA). Decitabine inhibits the synthesis of DNA and RNA and inhibits the growth of tumour cells. Decitabine also induces cell differentiation of blood cells.

What is the stage of development of this medicinal product?

At the time of submission of the application for orphan designation, clinical trials in patients with myelodysplastic syndromes were ongoing.

In the United States of America, orphan drug status was granted on 8 March 1999 for treatment of myelodysplastic syndromes.

Decitabine was not granted marketing authorisation in any country inside or outside the European Union at the time of submission of the application.

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According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 January 2003 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.