



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

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in September 2008 on request of the Sponsor.

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of doxorubicin carbon/iron magnetically targeted microparticles for the treatment of hepatocellular carcinoma

On 11 September 2002, orphan designation (EU/3/02/112) was granted by the European Commission to Interface International Consultancy Limited, United Kingdom, for Doxorubicin carbon/iron magnetically targeted microparticles for the treatment of hepatocellular carcinoma.

What is hepatocellular carcinoma?

Tumours that begin in the liver are known as liver tumours. Liver tumours which have potential to grow rapidly and infiltrate surrounding healthy tissues are called hepatocellular carcinomas. Hepatocellular carcinoma is a life-threatening condition.

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

At the time of designation, hepatocellular carcinoma affected approximately 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 38,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

No medicinal products for the treatment of the condition had been authorised in the European Union at the time of submission of the application for orphan drug designation.

How is this medicine expected to work?

Doxorubicin is a widely used drug to treat cancers and also used in hepatocellular carcinoma. Doxorubicin interferes with the production of genetic material and can kill rapidly dividing cells such as cancer cells. When doxorubicin is administered with carbon/iron particles, the application of an external magnet can keep the drug at the tumour site. This may increase the presence of the drug in the tumour and may enhance its activity.

What is the stage of development of this medicine?

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

*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.

Doxorubicin carbon/iron magnetically targeted microparticles has been designated as an orphan medicinal product in the United States for treatment of hepatocellular carcinoma and has not been marketed anywhere worldwide for hepatocellular carcinoma, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 18 July 2002 a positive opinion recommending the grant of the above mentioned designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the Community) or insufficient returns on investment.

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

For more information:

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