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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF

nemorubicin hydrochloride for the treatment of hepatocellular carcinoma

On 27 July 2005, orphan designation (EU/3/05/300) was granted by the European Commission to Nerviano Medical Sciences Srl, Italy, for nemorubicin hydrochloride for the treatment of hepatocellular carcinoma.

What is hepatocellular carcinoma?

Tumours that begin in the liver are known as liver tumours. Liver tumours that have the potential to infiltrate healthy tissues and spread to other parts of the body are called hepatocellular carcinomas. The most common factors known to be associated with this disease are the viral infections causing liver inflammations (the so-called hepatitis B and hepatitis C) and an alcohol-induced liver disease (the so-called liver cirrhosis). Hepatocellular carcinoma is a life-threatening condition.

What are the methods of treatment available?

The choice of the treatment of hepatocellular cancer depends on several factors, mainly the stage of the disease. Treatments may include surgery, radiation therapy (using high-dose x-rays or other high-energy rays to kill cancer cells), chemotherapy (using drugs to kill cancer cells) or immunotherapy (treatment by stimulation of the body's own defense system). At the time of submission of the application for orphan drug designation, several products were authorised for the condition in some countries of the Community.

Nemorubicin hydrochloride might be of potential significant benefit for the treatment of hepatocellular carcinoma, particularly in combination with currently authorised medicinal products, because it might improve the long-term outcome of the patients. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, hepatocellular carcinoma was considered to affect about 46,000 persons in the European Union.

How is this medicinal product expected to act?

Nemorubicin hydrochloride is derived from doxorubicin by replacing a chemical group of its structure. Doxorubicin is a widely used drug to treat cancers and is also used in hepatocellular carcinoma. It is expected that nemorubicin hydrochloride, as doxorubicin, will also interfere with the production of genetic material in the cancer cells and will thereby, rapidly kill dividing cells such as cancer cells.

What is the stage of development of this medicinal product?

The effects of nemorubicin hydrochloride were evaluated in experimental models.

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

Nemorubicin hydrochloride was not authorised anywhere worldwide for hepatocellular carcinoma or designated as orphan medicinal product elsewhere for this condition, 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 15 June 2005 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: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Nemorubicin hydrochloride	Treatment of hepatocellular carcinoma
Czech	Nemorubicín hydrochlorid	Léčba hepatocelulárního karcinomu
Danish	Nemorubicinhydrochlorid	Behandling af hepatocellulært carcinom
Dutch	Nemorubicinel hydrochloride	Behandeling van hepatocellulair carcinoom
Estonian	Nemorubitsiin-hüdrokloriid	Hepatosellulaarse kartsinoomi ravi
Finnish	Nemorubisiinihydrokloridi	Hepatosellulaarisen karsinooman hoito
French	Nemorubicine hydrochloride	Traitement du carcinome hépatocellulaire
German	Nemorubizinhydrochlorid	Behandlung des Leberzellkarzinoms
Greek	Νεμορουβικίνη (υδροχλωρική)	Θεραπεία του ηπατοκυτταρικού καρκινώματος
Hungarian	Nemorubicin hidroklorid	Hepatocelluláris carcinoma kezelése
Italian	Nemorubicina cloridrato	Trattamento del carcinoma epatozellulare
Latvian	Nemorubicīna hidrohlorīds	Hepatocelulāras karcinomas ārstēšana
Lithuanian	Nemorubicino hidrochloridas	Hepatoceliulinės karcinomos gydymas
Polish	Nemorubicyny chlorek	Leczenie raka wątrobowokomórkowego
Portuguese	Cloridrato de nemorrubicina	Tratamento do carcinoma hepatocelular
Slovak	Hydrochlorid nemorubicínu	Liečba hepatocelulárneho karcinómu
Slovenian	Nemorubicinijev hidroklorid	Zdravljenje hepatocelularnega karcinoma
Spanish	Clorhidrato de nemorrubicina	Tratamiento del carcinoma hepatocelular
Swedish	Nemorubicin hydroklorid	Behandling av hepatocellulärt carcinom
Norwegian	Nemorubicinhydroklorid	Behandling av hepatocellulært karsinom
Icelandic	Nemorúbicín hýdróklóríð	Meðferð við lifrarfrumukrabbameini