



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Mertansine functionalised gold nanoconjugate for the treatment of hepatocellular carcinoma

On 22 February 2018, orphan designation (EU/3/18/1981) was granted by the European Commission to Midatech Pharma Plc, United Kingdom, for mertansine functionalised gold nanoconjugate for the treatment of hepatocellular carcinoma.

What is hepatocellular carcinoma?

Hepatocellular carcinoma is a primary cancer of the liver (a cancer that starts in the liver, rather than one that has spread to the liver from elsewhere in the body). It is more common in men than in women, and occurs mostly in people who have liver scarring (cirrhosis), or after hepatitis B or C infection. Features of the disease include yellow skin, pain and swelling in the belly, easy bruising, weight loss, weakness, loss of appetite and nausea (feeling sick).

Hepatocellular carcinoma is a long-term debilitating and life threatening condition, with patients surviving on average for a few months or years after diagnosis.

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

At the time of designation, hepatocellular carcinoma affected approximately 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 78,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, some patients with early stage hepatocellular carcinoma were treated with surgery to remove part of the liver, liver transplantation or radiofrequency ablation (directing heat and electricity through a needle to destroy cancer cells). Chemotherapy (medicines to treat cancer) was used if surgery was not possible or the disease had spread to other parts of the body (metastatic disease). Nexavar (sorafenib) was authorised in the EU for use in hepatocellular carcinoma.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients, with early laboratory studies showing that it blocks tumour growth more efficiently than the currently authorised product. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Mertansine functionalised gold nanoconjugate is expected to work by targeting cancer cells and disrupting cell growth. By not allowing the cancer cells to divide and multiply, it is expected to slow down the growth and spread of the cancer.

A component of this medicine, mertansine, is a cytotoxic (cell killing) agent that had been tried as a cancer medicine in the past. This current product contains some additional components to help it target cancer cells more efficiently.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with hepatocellular carcinoma had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for hepatocellular carcinoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 January 2018 recommending the granting of this 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 EU) 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

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Mertansine functionalised gold nanoconjugate	Treatment of hepatocellular carcinoma
Bulgarian	Метранзин функционализиран златен нано-конюгат	Лечение на хепатоцелуларен карцином
Croatian	Mertanzin funkcionalizirani nanokonjugat zlata	Liječenje hepatocelularnog karcinoma
Czech	Funkční konjugát zlatých nanočástic s mertansinem	Léčba hepatocelulárního karcinomu
Danish	Mertansin funktionelt guld nano konjugat	Behandling af hepatocellulært karcinom
Dutch	Met mertansine gefunctionaliseerd goud-nanoconjugaat	Behandeling van hepatocellulair carcinoom
Estonian	Mertansiin-funktsionaliseeritud kuld-nanokonjugaat	Hepatotsellulaarse kartsinoomi ravi
Finnish	Mertansiinillä funktionalisoitu kultananokonjugaatti	Hepatosellulaarisen karsinooman hoito
French	Nano-conjugué fonctionnalisé avec des particules d'or de mertansine	Traitement du cancer hépatocellulaire
German	Mertansin funktionalisiertes Gold-Nanopartikelkonjugat	Behandlung des Leberzellkarzinoms
Greek	Ενεργοποιημένο με μερτανσίνη σύζευγμα νανοσωματιδίων χρυσού	Θεραπεία του ηπατοκυτταρικού καρκινώματος
Hungarian	Mertanszin funkcionalizált arany nanokonjugát	Hepatocelluláris carcinoma kezelése
Italian	nano coniugati d'oro funzionalizzati con mertansine	Trattamento del carcinoma epatocellulare
Latvian	Mertanzīna funkcionalizēts zelta nanokonjugāts	Hepatocellulāras karcinomas ārstēšana
Lithuanian	Mertansino funkcionalizuotas aukso nanokonjugatas	Hepatoceliulinės karcinomos gydymas
Maltese	Mertansina nanokonjugat tad-deheb funzjonalizzat	Kura tal-karċinoma epatoċellulari
Polish	Mertanzyna nanokoniugat mertanzyny z funkcjonalizowanym złotem	Leczenie raka wątrobowokomórkowego
Portuguese	Nanoconjugados de ouro funcionalizados com mertasina	Tratamento do carcinoma hepatocelular
Romanian	Mertansină nanoconjugat de aur funcționalizat	Tratamentul carcinomului hepatocelular
Slovak	Mertansine funkcionalizovaný nanokonjugát zlata	Liečba hepatocelulárneho karcinómu
Slovenian	Funkcionalizirani konjugat nanodelcev zlata z mertanzinom	Zdravljenje hepatocelularnega karcinoma

¹ At the time of designation

Language	Active ingredient	Indication
Spanish	Nanoconjugado de oro funcionalizado mertansina	Tratamiento del carcinoma hepatocelular
Swedish	Mertansin funktionaliserat guldnanokonjugat	Behandling av hepatocellulärt karcinom
Norwegian	Mertansin funksjonalisert gullnanokonjugat	Behandling av hepatocellulært karsinom
Icelandic	Mertansín-virkjuð nanógullsamtinging	Meðferð við lifrarfrumukrabbameini