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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Doxorubicin polyisohexylcyanoacrylate nanoparticles for treatment of hepatocellular carcinoma

First publication	1 July 2005
Rev.1: sponsor's change of address	19 August 2009
Rev.2: sponsor's name change	4 March 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 21 October 2004, orphan designation (EU/3/04/229) was granted by the European Commission to BioAlliance Pharma, France, for doxorubicin polyisohexylcyanoacrylate nanoparticles for the treatment of hepatocellular carcinoma.

In October 2014, BioAlliance Pharma changed name to Onxeo.

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 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 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 was equivalent to a total of around 46,000 people*, and is below the ceiling

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 464,200,000 (Eurostat 2004).

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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?

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 drug to treat tumours. Doxorubicin interferes with the production of genetic material and can kill rapidly dividing cells such as tumour cells. In the designated product, doxorubicin is entrapped in nanoparticles of polyisohexylcyanoacrylate. Once administered to the patient, the nanoparticles are adsorbed to the surface of tumour cells and doxorubicin entrapped in these particles is subsequently released on the cancer cell. The nanoparticles degrade and release also polyisohexylcyanoacrylate, which may contribute to delivering doxorubicin into the cancer cells. This may increase the presence of the drug in the tumour cells and may enhance its activity.

What is the stage of development of this medicine?

The effects of doxorubicin polyisohexylcyanoacrylate nanoparticles 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.

Doxorubicin polyisohexylcyanoacrylate nanoparticles was not marketed anywhere worldwide for hepatocellular carcinoma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 September 2004 recommending the granting of this designation.

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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:

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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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Doxorubicin polyisohexylcyanoacrylate nanoparticles	Treatment of the hepatocellular carcinoma
Czech	Polyisohexylcyanoacrylátové nanočástice doxorubicínu	Léčba hepatocelulárního karcinomu
Danish	Doxorubicin polyisohexylcyanoacrylat nanopartikel	Behandling af hepatocellulært carcinom
Dutch	Doxorubicine polyisohexylcyanoacrylate nanopartikels	Behandeling van hepatocellulair carcinoom
Estonian	Doksorubitsiini polüisohexüültsüanoakrülaat nanopartiklid	Hepatotsellulaarse kartsinoomi ravi
Finnish	Doksorubisiini polyisoheksyylisyanoakrylaatti nanopartikkeli	Hepatosellulaarisen karsinooman hoito
French	Nanoparticules de polyisohexylcyanoacrylate de Doxorubicine	Traitement du carcinome hépatocellulaire
German	Doxorubizin polyisohexylcyanoacrylat Nanopartikel	Behandlung des Leberzellkarzinoms
Greek	Νανοσωματίδια δοξορουβικίνης πολυ-ισοεξυλοκυανοακρυλικού	Θεραπεία του ηπατοκυταρρικού καρκινώματος
Hungarian	Doxorubicin polyisohexylcyanoacrylat nanoméretű részecskék	Hepatocelluláris carcinoma kezelése
Italian	Nanoparticelle di poliisoesilcianoacrilato di doxorubicina	Trattamento del carcinoma epatocellulare
Latvian	Doksorubicīna poliizohexilcianoakrilāta nanodaļiņas	Hepatocellulāras karcinomas ārstēšana
Lithuanian	Doksorubicino polyizoheksilcianoakrilato nanodalelės	Hepatoceliulinės karcinomos gydymas
Maltese	Doxorubicin polyisohexylcyanoacrylate nanoparticles	Treatment of the hepatocellular carcinoma
Polish	Doksorubicyny poliizoheksylocyjanoakrylan nanocząsteczkowy	Leczenie raka wątrobowokomórkowego
Portuguese	Nanoparticulas de poliisohexilcianoacrilato de Doxorrubicina	Tratamento do carcinoma hepatocelular
Slovak	Nanočastice polyizohexylcyanoakrylát doxorubicínu	Liečba hepatocelulárneho karcinómu
Slovenian	Doksorubicin poliizoheksilcianoakrilat nanodelci	Zdravljenje hepatocelularnega karcinoma
Spanish	Doxorrubicina en nanopartículas contenidas en poliisohexilcianoacrilato	Tratamiento de carcinoma hepatocelular
Swedish	Doxorubicin polyisohexylcyanoakrylat nanopartikel	Behandling av hepatocellulärt karcinom

[1] At the time of designation

Language	Active Ingredient	Indication
Norwegian	Doksorubicinpolyisoheksylcyanoakrylat nanopartikler	Behandling av hepatocellulært karsinom
Icelandic	Doxórúbícín polyisohexylcýanóakrýlat nanóagnir	Meðferð við lifrarfrumukrabbameini