8 March 2017
EMA/15467/2017
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Antroquinonol for the treatment of pancreatic cancer

On 12 January 2017, orphan designation (EU/3/16/1812) was granted by the European Commission to Biological Consulting Europe Ltd, United Kingdom, for antroquinonol for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: to produce a fluid that helps with the digestion of food, and to produce hormones such as insulin. Due to the absence of symptoms in the early stages of pancreatic cancer, most patients are diagnosed with it when the cancer has spread nearby or to other parts of the body.

Pancreatic cancer is a very severe and life-threatening disease that is associated with shortened life expectancy.

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

At the time of designation, pancreatic cancer affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 82,000 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, several medicines were authorised in the EU for treating pancreatic cancer. The choice of treatment depended on several factors, including how far the disease had advanced. Treatments included surgery and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that antroquinonol might be of significant benefit for patients with pancreatic cancer because laboratory studies show that it may reduce tumour growth when used in combination with currently authorised cancer medicines (gemcitabine and

*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 513,700,000 (Eurostat 2016).

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paclitaxel). 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?

Antroquinonol is expected to work by blocking 'the Ras signalling pathway'. This is a mechanism within cells that helps them to grow and survive. However, in cancer cells it works abnormally, leading to the growth of the cancer. By blocking the Ras pathway, antroquinonol is expected to kill cancer cells and slow down the growth of the cancer.

What is the stage of development of this medicine?

The effects of antroquinonol have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with antroquinonol in patients with solid cancers were ongoing.

At the time of submission, antroquinonol was not authorised anywhere in the EU for pancreatic cancer. Orphan designation of antroquinonol had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 December 2016 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:

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Antroquinonol	Treatment of pancreatic cancer
Bulgarian	Антроквинонол	Лечение на рак на панкреаса
Croatian	Antrokvinonol	Liječenje raka gušterače
Czech	Antroquinonol	Léčba karcinomu pankreatu
Danish	Antroquinonol	Behandling af pancreascancer
Dutch	Antroquinonol	Behandeling van pancreaskanker
Estonian	Antrokvinonool	Pankreasevähi ravi
Finnish	Antrokinonoli	Haimasyövän hoito
French	Antroquinonol	Traitement du cancer pancréatique
German	Antroquinonol	Behandlung des Pankreaskarzinoms
Greek	Αντροκινονόλη	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Antrokinonol	Hasnyálmirigyrák kezelése
Italian	Antroquinonol	Trattamento del cancro pancreatico
Latvian	Antrokvinonols	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Antrokvinonolis	Kasos vėžio gydymas
Maltese	Antroquinonol	Kura tal-kanċer tal-frixa
Polish	Antrochinonol	Leczenie raka trzustki
Portuguese	Antroquinonol	Tratamento do carcinoma do pâncreas
Romanian	Antrochinonol	Tratamentul cancerului pancreatic
Slovak	Antroquinonol	Liečba rakoviny pankreasu
Slovenian	Antrokvinonol	Zdravljenje raka trebušne slinavke
Spanish	Antroquinonol	Tratamiento del cáncer de páncreas
Swedish	Antrokinonol	Behandling av pankreascancer
Norwegian	Antrokinonol	Behandling av pankreascancer
Icelandic	Antrókínólól	Meðferð briskrabbameins

[1] At the time of designation