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EMA/COMP/93499/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cysteamine bitartrate for the treatment of pancreatic cancer

On 26 March 2014, orphan designation (EU/3/14/1252) was granted by the European Commission to Raptor Pharmaceuticals Europe B.V., The Netherlands, for cysteamine bitartrate 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 juice 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, the majority of patients are diagnosed when the cancer has spread locally 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 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 77,000 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, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that cysteamine bitartrate might be of significant benefit for patients with pancreatic cancer because it works in a different way to existing

^{*}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 511,100,000 (Eurostat 2014).

treatments and experimental studies suggest that it can reduce the spread of the cancer. 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?

The active substance in this medicine, cysteamine bitartrate, is thought to block the action of certain enzymes called matrix metalloproteinases. Tumours such as pancreatic cancer produce high levels of these enzymes, which break down the substances that cement normal cells together in the tissues, enabling the cancer cells to spread more easily between them. By blocking the action of the enzymes, the medicine is expected to reduce the spread of cancer cells.

What is the stage of development of this medicine?

The effects of cysteamine bitartrate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with cysteamine bitartrate in patients with pancreatic cancer had been started.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2014 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cysteamine bitartrate	Treatment of pancreatic cancer
Bulgarian	Цистеамин битартрат	Лечение на рак на панкреаса
Czech	Cystamin bitartat	Léčba karcinomu pankreatu
Croatian	Cisteaminhidrogentartarat	Liječenje raka gušterače
Danish	Cysteaminbitartrat	Behandling af pancreascancer
Dutch	Cysteaminebitartraat	Behandeling van pancreaskanker
Estonian	Tsüsteamiinbitartraat	Pankreasevähi ravi
Finnish	Kysteamiinibitartraatti	Haimasyövän hoito
French	Bitartrate de cystéamine	Traitement du cancer pancréatique
German	Cysteamin-Bitartrat	Behandlung des Pankreaskarzinoms
Greek	Διτρυγική κυστεαμίνη	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Ciszteamin bitartarát	Hasnyálmirigyrák kezelése
Italian	Cisteamina bitartrato	Trattamento del cancro pancreatico
Latvian	Cisteamīna bitartrāts	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Cisteamino bitartratas	Kasos vėžio gydymas
Maltese	Cysteamine bitartrate	Kura tal-kanċer tal-frixa
Polish	Cysteaminowy dwuwodnian	Leczenie raka trzustki
Portuguese	Bitartarato de cisteamina	Tratamento do carcinoma do pâncreas
Romanian	Bitartrat de cisteamină	Tratamentul cancerului pancreatic
Slovak	Cysteaminbitartarát	Liečba rakoviny pankreasu
Slovenian	Cisteaminijev bitartrat	Zdravljenje raka trebušne slinavke
Spanish	Bitartrato de cisteamina	Tratamiento del cáncer de páncreas
Swedish	Cysteaminbitartrat	Behandling av pankreascancer
Norwegian	Cysteaminbitartrat	Behandling av pankreascancer
Icelandic	Cýsteamín bítartrat	Meðferð briskrabbameins

¹ At the time of designation