23 February 2015
EMA/COMP/733378/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pegylated recombinant human hyaluronidase PH20 for the treatment of pancreatic cancer

On 16 December 2014, orphan designation (EU/3/14/1394) was granted by the European Commission to Pharm. Research Associates (UK) Limited, United Kingdom, for pegylated recombinant human hyaluronidase PH20 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, 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 1.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 92,000 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).

*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).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with pancreatic cancer because early results suggest that response rates and survival times may be increased in patients with pancreatic cancer that has spread to other parts of the body who are given this medicine in addition to conventional treatment. 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 medicine contains a version of a natural enzyme, hyaluronidase, which breaks down a compound, hyaluronan. Hyaluronan is found in large amounts in many pancreatic cancers and helps the cancer to grow and to resist the effects of cancer medicines. By breaking down the excess hyaluronan, the medicine is expected to make the cancer easier to treat with other authorised therapies.

In this medicine, hyaluronidase has been 'pegylated' (combined with a chemical called polyethylene glycol). This decreases the rate at which the substance is removed from the body and allows the medicine to be given less often.

What is the stage of development of this medicine?

The effects of pegylated recombinant human hyaluronidase PH20 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with pegylated recombinant human hyaluronidase PH20 in patients with pancreatic cancer were ongoing.

At the time of submission, pegylated recombinant human hyaluronidase PH20 was not authorised anywhere in the EU for pancreatic cancer. Orphan designation of the medicine 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 13 November 2014 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	Pegylated recombinant human hyaluronidase PH20	Treatment of pancreatic cancer
Bulgarian	Пегилирана рекомбинантна човешка хиалуронидаза PH20	Лечение на рак на панкреаса
Croatian	Pegilirana rekombinantna ljudska hijaluronidaza PH20	Liječenje raka gušterače
Czech	Pegylovaná rekombinantní lidská hyaluronidáza PH20	Léčba karcinomu pankreatu
Danish	Pegyleret rekombinant humant hyaluronidase PH20	Behandling af pancreascancer
Dutch	Gepegyleerd recombinant humaan hyaluronidase PH20	Behandeling van pancreaskanker
Estonian	Pegüleeritud rekombinantne inimese hüaluronidaas PH20	Pankreasevähi ravi
Finnish	Pegyloitu rekombinantti ihmisen hyaluronidaasi PH20	Haimasyövän hoito
French	Forme pégylée de la hyaluronidase recombinante humaine PH20	Traitement du cancer pancréatique
German	Pegylierte rekombinante humane Hyaluronidase PH20	Behandlung des Pankreaskarzinoms
Greek	Πεγκυλιωμένη ανασυνδυασμένη ανθρώπινη υαλουρονιδάση PH20	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Pegilált rekombináns humán hialuronidáz PH20	Hasnyálmirigyrák kezelése
Italian	Ialuronidasi ricombinante umana PH20 pegilata	Trattamento del cancro pancreatico
Latvian	Pegilēta rekombinanta cilvēku hialuronidāze PH20	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Pegiliuota rekombinantinė žmogaus hialuronidazė PH20	Kasos vėžio gydymas
Maltese	Hyaluronidase PH20 uman rikombinanti pegilat	Kura tal-kanċer tal-frixa
Polish	Pegylowana rekombinowana ludzka hialuronidaza PH20	Leczenie raka trzustki
Portuguese	Hialuronidase PH20 humana recombinante peguilada	Tratamento do carcinoma do pâncreas
Romanian	Forma pegilată a hialuronidazei PH20 recombinante umane	Tratamentul cancerului pancreatic
Slovak	Pegylovaná rekombinantná ľudská hyaluronidáza PH20	Liečba rakoviny pankreasu
Slovenian	Pegilirana rekombinantna humana hialuronidaza PH20	Zdravljenje raka trebušne slinavke
Spanish	Forma pegilada de la hialuronidasa recombinante humana PH20	Tratamiento del cáncer de páncreas

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Pegylerat rekombinant humant hyaluronidas PH20	Behandling av pankreascancer
Norwegian	Pegylert rekombinant human hyaluronidase PH20	Behandling av pankreascancer
Icelandic	Pegýlerað raðbrigða manna hýalúróníðasi PH20	Meðferð briskrabbameins