



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human elafin for the treatment of oesophagus carcinoma

On 28 January 2010, orphan designation (EU/3/09/710) was granted by the European Commission to Proteo Biotech AG, Germany, for recombinant human elafin for the treatment of oesophagus carcinoma.

What is oesophagus carcinoma?

Oesophagus carcinoma is cancer of the oesophagus (the tube that connects the mouth to the stomach). It usually starts in the cells lining the oesophagus, and spreads easily to other parts of the body.

Oesophageal carcinoma is a life-threatening disease because it is associated with poor overall survival and because of serious complications such as inflammation that develop after surgery to remove the cancer.

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

At the time of designation, oesophageal carcinoma affected approximately 0.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 15,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

The choice of treatment for oesophageal carcinoma depends on where in the oesophagus the cancer is located, and how advanced it is. At the time of orphan designation, the main treatment was surgery, sometimes preceded by radiotherapy (treatment with radiation) or chemotherapy (medicines to treat cancer) to make the cancer shrink and easier to remove. Other medicines were also used after surgery to treat complications.

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 504,800,000 (Eurostat 2009).



The sponsor has provided sufficient information to show that recombinant human elafin might be of significant benefit for patients with oesophageal carcinoma because it may improve the treatment of the complications of oesophagus carcinoma following surgery. 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?

Surgery for oesophagus carcinoma is associated with more inflammatory complications than other types of surgery. Recombinant human elafin is expected to help to control the inflammatory response after surgery.

The product is a copy of the natural substance elafin. In the body, elafin works by blocking some type of 'proteases' present in cells of the immune system that are involved in the production of inflammatory substances, such as interleukins. By blocking proteases, recombinant human elafin is expected to reduce the production of inflammatory substances and therefore relieve the inflammatory complications of oesophagus carcinoma, especially those affecting the lungs.

What is the stage of development of this medicine?

The effects of recombinant human elafin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the designated product in patients with oesophagus carcinoma were ongoing.

At the time of submission, recombinant human elafin was not authorised anywhere in the EU for oesophagus 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 5 November 2009 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 Community) 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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Translations of the active ingredient and indication in all official EU languages, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant human elafin	Treatment of oesophagus carcinoma
Bulgarian	Рекомбинантен човешки елафин	Лечение на карцином на хранопровода
Czech	Rekombinantní lidský elafin	Léčba karcinomu jícnu
Danish	Rekombinant humant elafin	Behandling af esophaguskarcinom
Dutch	Recombinant humaan elafin	Behandeling van slokdarmcarcinoom
Estonian	Inimese rekombinantne elafin	Söögitoru kartsinoomi ravi
Finnish	Rekombinantti ihmisen elafiini	Ruokatorven syövän hoito
French	Elafine humaine recombinante	Traitement du cancer de l'oesophage
German	Rekombinantes humanes Elafin	Behandlung des Ösophaguskarzinoms
Greek	Ανασυνδυασμένη ανθρώπινη ελαφίνη	θεραπεία οισοφαγικού καρκινώματος
Hungarian	Rekombinációs humán elafin	Nyelőcsőrák kezelése
Italian	Elafina umana ricombinante	Trattamento chirurgico del carcinoma esofageo
Latvian	Rekombinants cilvēka elafīns	Barības vada karcinomas ārstēšana
Lithuanian	Rekombinantinis žmogaus elafinas	Stemplės karcinomos gydymas
Maltese	Elafin rikombinanti umana	Kura tal-karcinoma tal-esofagu
Polish	Rekombinowana ludzka elafina	Leczenie raka przełyku
Portuguese	Elafina humana recombinante	Tratamento do carcinoma do esófago
Romanian	Elafin umană recombinantă	Tratamentul cancerului esofagian
Slovak	Rekombinantný ľudský elafin	Liečba karcinómu pažeráka
Slovenian	Rekombinantni humani elafin	Zdravljenje raka na požiralniku
Spanish	Elafina humana recombinante	Tratamiento del carcinoma de esófago
Swedish	Rekombinant human elafin	Behandling av cancer i matstrupe
Norwegian	Rekombinant human elafin	Behandling av øsofagus cancer
Icelandic	Raðbrigða manna elafín	Meðferð á krabbameini í vélinda