



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Genetically modified human adenovirus encoding human PH20 hyaluronidase for the treatment of pancreatic cancer

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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 June 2011, orphan designation (EU/3/11/880) was granted by the European Commission to VCN Biosciences S.L., Spain, for genetically modified human adenovirus encoding human PH20 hyaluronidase 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 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 66,000 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).

*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. At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).



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 advanced the disease is. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with pancreatic cancer because it works in a different way to existing treatments and early studies in experimental models show that it might be used in combination with other treatments to improve the outcome of patients with this condition. 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?

This medicine is made up of an 'oncolytic' virus, a virus that has been genetically modified so that it is able to target, replicate itself in and destroy tumour cells while sparing normal cells. When inside a tumour cell, the virus is expected to take over the cell's replication apparatus and use it to make more copies of itself. This is expected to kill the cell, leaving the virus to spread to neighbouring tumour cells.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of genetically modified human adenovirus encoding human PH20 hyaluronidase in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with pancreatic cancer had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for pancreatic cancer 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 7 April 2011 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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08174 - Sant Cugat del Vallès
Barcelona
Spain
E-mail: info@vcnbiosciences.com

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	Genetically modified human adenovirus encoding human PH20 hyaluronidase	Treatment of pancreatic cancer
Bulgarian	Генетичномодифициран човешки аденовирус, кодиращ човешка PH20 хиалуронидаза.	Лечение на рак на панкреаса
Czech	Geneticky modifikovaný lidský adenovirus obsahující gen humánní hyaluronidasy PH20	Léčba karcinomu pankreatu
Danish	Genetisk modificeret humant adenovirus som koder for human PH20 hyaluronidase hyaluronidase	Behandling af pancreascancer
Dutch	Genetisch gemodificeerd human adenovirus welke codeert voor humaan PH20 hyaluronidase	Behandeling van pancreaskanker
Estonian	Geneetiliselt modifitseeritud inimese adenoviirus, mis kodeerib inimese PH20 hüaluronidaasi	Pankreasevähi ravi
Finnish	Geneettisesti muunneltu ihmisen adenovirus, joka koodaa ihmisen PH20 hyaluronidaasia	Haimasyövän hoito
French	Adénovirus humain génétiquement modifié codant pour la hyaluronidase PH20 humaine	Traitement du cancer pancréatique
German	Genetisch modifiziertes humanes Adenovirus Typ 5, welches das humane PH20 Hyaluronidase-Gen enthält	Behandlung des Pankreaskarzinoms
Greek	Γενετικά τροποποιημένος ανθρώπινος αδενοϊός που φέρει τον κώδικα για την ανθρώπινη υαλουρονιδάση PH20	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Humán PH20 hialuronidázt kódoló genetikailag módosított human adenovírus	Hasnyálmirigyrák kezelése
Italian	Adenovirus umano geneticamente modificato che codifica la ialuronidasi umana PH20	Trattamento del cancro pancreatico
Latvian	Ģenētiski modificēts cilvēka adenovīruss, kas kodēts ar PH20 hialuronidāzi	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Genetiškai modifikuotas žmogaus adenovirusas, koduojantis žmogaus PH20 hialuronidazę	Kasos vėžio gydymas
Maltese	Adenovirus uman modifikat ġenetikament li jikkodifika PH20 hyaluronidase uman	Kura tal-kanċer tal-frixa
Polish	Genetycznie zmodyfikowany ludzki adenowirus zawierający gen ludzkiej hialuronidazy PH20	Leczenie raka trzustki
Portuguese	Adenovirus humano geneticamente modificado que codifica a hialuronidase humana PH20	Tratamento do carcinoma do pâncreas
Romanian	Adenovirus uman modificat genetic ce codifică hialuronidaza PH20 umană	Tratamentul cancerului pancreatic
Slovak	Geneticky modifikovaný ľudský adenovírus kódujúci ľudskú hialuronidázu PH20	Liečba rakoviny pankreasu

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Genetsko modificiran humani adenovirus, kodiran za humano hialuronidazo PH20	Zdravljenje raka trebušne slinavke
Spanish	Adenovirus humano genéticamente modificado que codifica para la hialuronidasa humana PH20	Tratamiento del cáncer de páncreas
Swedish	Genetiskt modifierat mänskligt adenovirus som kodar för mänskligt PH20 hyaluronidas	Behandling av pankreascancer
Norwegian	Genmodifisert humant adenovirus kodende for human PH20 hyaluronidase	Behandling av pankreascancer
Icelandic	Erfðabreytt manna adenoveira sem tjáir manna PH20 hyalúrónidasa	Meðferð briskrabbameins