



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

2 March 2015  
EMA/COMP/797466/2014  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Herpes simplex type 1 virus containing cellular *B-myb* gene as tumour-specific promoter for the treatment of pancreatic cancer

On 15 January 2015, orphan designation (EU/3/14/1412) was granted by the European Commission to Karcinolys S.A.S., France, for herpes simplex type 1 virus containing cellular *B-myb* gene as tumour-specific promoter 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 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 people<sup>\*</sup>, 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).

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<sup>\*</sup>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).



The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with pancreatic cancer because early studies in experimental models showed an anti-tumour effect, which was increased when the medicine was used together with the chemotherapy medicine gemcitabine. 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 consists of an 'oncolytic' virus (a virus that kills cancer cells) that contains a part of a gene (called *B-myb*). This is expected to allow the virus to multiply and kill only cells that have high levels of the gene *B-myb* (such as pancreatic cancer cells), while sparing normal cells.

The virus in the medicine (a herpes virus) has been modified so that it does not cause disease in humans.

### **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 the medicine in experimental models was ongoing.

At the time of submission of the application for orphan designation, 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 11 December 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:

Karcinolys S.A.S.  
16, rue de Pervenches  
92500 Rueil-Malmaison  
France  
Tel. +33 6 45 62 30 43  
E-mail: [jean-luc.bejot@karcinolys.com](mailto:jean-luc.bejot@karcinolys.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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Herpes simplex type 1 virus containing cellular <i>B-myb</i> gene as tumour-specific promoter	Treatment of pancreatic cancer
Bulgarian	Херпес симплекс тип 1 вирус съдържащ клетъчен <i>B-myb</i> ген като тумор-специфичен промотер	Лечение на рак на панкреаса
Croatian	Virus Herpesa simpleksa tipa 1 koji sadrži stanični gen <i>B-myb</i> kao tumor-specifični promotor	Liječenje raka gušterače
Czech	Herpes simplex virus typu 1 obsahující <i>B-myb</i> gen jako specifický tumorózní promotor	Léčba karcinomu pankreatu
Danish	Herpes simplex type 1-virus indeholdende cellulære <i>B-myb</i> -genet som tumorspecifik promotor	Behandling af pancreascancer
Dutch	Herpes simplex type 1 virus welke cellulair <i>B-myb</i> gen als tumor specifieke promotor bevat	Behandeling van pancreaskanker
Estonian	Herpes simplex tüüp 1 viirus, mis sisaldab rakulist <i>B-myb</i> geeni kui kasvaja-spetsiifilist promooterit	Pankreasevähi ravi
Finnish	Herpes simplex tyypin 1 virus, joka sisältää sellulaarisen <i>B-myb</i> geenin kasvainspesifisenä promoottorina	Haimasyövän hoito
French	Virus de l'Herpès simplex de type 1 contenant un gène des cellules <i>B-myb</i> , comme promoteur spécifique de tumeur	Traitement du cancer pancréatique
German	Herpes simplex Typ 1 Virus, der das zelluläre <i>B-myb</i> Gen als Tumor-spezifischen Promotor enthält	Behandlung des Pankreaskarzinoms
Greek	Ίός του απλού έρπητα τύπου 1 που περιέχει ένα κυτταρικό γονίδιο <i>B-myb</i> ως ειδικό υποκινητή του όγκου.	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Celluláris <i>B-myb</i> gént, mint tumor-specifikus promotert tartalmazó 1-es típusú Herpes simplex vírus	Hasnyálmirigyrák kezelése
Italian	Virus herpes simplex tipo 1 contenente il gene cellulare <i>B-myb</i> come promotore tumore-specifico	Trattamento del cancro pancreatico
Latvian	<i>Herpes simplex</i> 1. tipa vīruss, kas satur celulāro <i>B-myb</i> gēnu kā audzēja specifisku promoteru	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	<i>Herpes simplex</i> 1 tipo virusas, pernešantis ląstelės <i>B-myb</i> geną kaip augliui specifinį promotorių	Kasos vėžio gydymas
Maltese	Virus Herpes simplex tat-tip 1 li fih il-gene <i>B-myb</i> ċellulari bħala promotur speċifiku għal tumur	Kura tal-kanċer tal-frixa
Polish	Wirus <i>Herpes simplex</i> typu 1 zawierający gen komórkowy <i>B-myb</i> jako swoisty promotor nowotworu	Leczenie raka trzustki

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Portuguese	Virus herpes simplex tipo 1 contendo o gene <i>B-myb</i> celular como promotor específico de tumores	Tratamento do carcinoma do pâncreas
Romanian	Virus herpes simplex tip 1 care contine gena celulara <i>B-myb</i> ca promotor tumoral specific	Tratamentul cancerului pancreatic
Slovak	Vírus Herpes simplex typ 1 obsahujúci bunkový <i>B-myb</i> gén ako tumor špecifický promótor	Liečba rakoviny pankreasu
Slovenian	Herpes simpleks virus tipa 1, ki vsebuje celični <i>B-myb</i> gen kot specifični tumorski promoter	Zdravljenje raka trebušne slinavke
Spanish	Virus de herpes simplex tipo 1 conteniendo el gene <i>B-myb</i> celular como promotora específico de tumores	Tratamiento del cáncer de páncreas
Swedish	Herpes simplex typ 1 virus innehållande cellulär <i>B-myb</i> gen som tumörspecifik promotor	Behandling av pankreascancer
Norwegian	Herpes simplex type 1 virus som inneholder cellulært <i>B-myb</i> gen som tumorspesifikk promotor	Behandling av pankreascancer
Icelandic	Herpes simplex gerð 1 veiru sem inniheldur frumu <i>B-myb</i> gen sem æxlis-sértækur hvati	Meðferð briskrabbameins