



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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EMA/COMP/434969/2014  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

[5-Amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone for the treatment of pancreatic cancer

On 22 August 2014, orphan designation (EU/3/14/1323) was granted by the European Commission to Synovo GmbH, Germany, for [5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone 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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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 the medicine might be of significant benefit for patients with pancreatic cancer because it works in a different way to existing treatments and studies in experimental models indicate that it may reduce the spread of the cancer and improve survival. 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 expected to work in patients with pancreatic cancer by blocking the action of proteins called p38 MAP kinases. In pancreatic cancer, p38 MAP kinases play an important role in regulating the way that cells of the immune system (the body's natural defences) respond to various chemical messengers from the cancer. By blocking p38 MAP kinases, the medicine is expected to improve the ability of the immune system to recognise and destroy cancer cells, thereby slowing the progression of the disease.

### **What is the stage of development of this medicine?**

The effects of the medicine have been evaluated in experimental models.

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 10 July 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Treatment of pancreatic cancer
Bulgarian	[5-амино-1-(4-флуоро-фенил)-1H-пиразол-4-ил]-[3-(2,3-дихидрокси-пропокси)-фенил]-метанон	Лечение на рак на панкреаса
Croatian	[5-amino-1-(4-fluoro-fenil)-1H-pirazol-4-il]-[3-(2,3-dihidroksi-propoksi)-fenil]-metanon	Liječenje raka gušterače
Czech	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Léčba karcinomu pankreatu
Danish	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanon	Behandling af pancreascancer
Dutch	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Behandeling van pancreaskanker
Estonian	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Pankreasevähi ravi
Finnish	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Haimasyövän hoito
French	[5-amino-1-(4-fluoro-phényl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phényl]-méthanone	Traitement du cancer pancréatique
German	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Behandlung des Pankreaskarzinoms
Greek	[5-αμινο-1-(4-φθορο-φαινυλο)-1H-πυραζολο-4-υλο]-[3-(2,3-διυδροξυ-προποξυ)-φαινυλο]-μεθανονη	Θεραπεία καρκίνου του παγκρέατος
Hungarian	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Hasnyálmirigyrák kezelése
Italian	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Tattamento del cancro pancreatico
Latvian	[5-amino-1-(4-fluoro-fenil)-1H-pirazol-4-il]-[3-(2,3-dihidroksi-propoksi)-fenil]-metanons	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	[5-amino-1-(4-fluoro-fenil)-1H-pirazol-4-il]-[3-(2,3-dihidroksi-propoksi)-fenil]-metanonas	Kasos vėžio gydymas
Maltese	[5-amino-1-(4-fluoro-phenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-phenyl]-methanone	Kura tal-kanċer tal-frixa
Polish	[5-amino-1-(4-fluoro-fenyl)-1H-pirazol-4-yl]-[3-(2,3-dihydroksy-propoksy)-fenyl]-metanone	Leczenie raka trzustki
Portuguese	[5-amino-1-(4-fluoro-fenil)-1H-pirazol-4-il]-[3-(2,3-dihidroxi-propoxi)-fenil]-metanona	Tratamento do carcinoma do pâncreas
Romanian	[5-amino-1-(4-fluoro-fenil)-1H-pirazol-4-il]-[3-(2,3-dihidroxi-propoxi)-fenil]-metanonă	Tratamentul cancerului pancreatic

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

Language	Active ingredient	Indication
Slovak	[5-amino-1-(4-fluoro-fenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-fenyl]-metanón	Liečba rakoviny pankreasu
Slovenian	[5-amino-1-(4-fluoro-fenil)-1H-pirazol-4-il]-[3-(2,3-dihidroxi-propoxi)-fenil]-metanon	Zdravljenje raka trebušne slinavke
Spanish	[5-amino-1-(4-fluoro-fenil)-1H-pirazol-4-il]-[3-(2,3-dihidroxi-propoxi)-fenil]-metanona	Tratamiento del cáncer de páncreas
Swedish	[5-amino-1-(4-fluoro-fenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroxy-propoxy)-fenyl]-metanon	Behandling av pankreascancer
Norwegian	[5-amino-1-(4-fluoro-fenyl)-1H-pyrazol-4-yl]-[3-(2,3-dihydroksy-propoksy)-fenyl]-metanon	Behandling av pankreascancer
Icelandic	[5-aminó-1-(4-flúóró-phenyl)-1H-pýrazól-4-ýl]-[3-(2,3-díhýdroxý-própoxý)-phenýl]-methanón	Meðferð briskrabbameins