



## Committee for Orphan Medicinal Products

### Public summary of positive opinion for orphan designation of N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride for the treatment of pancreatic cancer

On 9 November 2009, orphan designation (EU/3/09/685) was granted by the European Commission to Merck KGaA, Germany, for N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride 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: producing a juice that helps with the digestion of food, and producing hormones such as insulin. Due to the absence of symptoms in the early stages of the disease, 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 leads to poor long-term survival.

#### 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 is equivalent to a total of around 66,000 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?

At the time of submission of the application for orphan drug designation, several medicines were authorised for pancreatic cancer in the EU. The choice of treatment for pancreatic cancer 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 early studies in experimental models indicate that it might improve the treatment of this condition when used in combination with existing treatments. 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?

N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride is expected to work by blocking an enzyme called MEK1/2, which is involved in stimulating cells to grow and divide. MEK1/2 is over-activated in cancer cells, which makes these cells divide uncontrollably. By blocking this enzyme, the medicine is expected to control cell division and slow down the rate of growth of the 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 27), Norway, Iceland and Liechtenstein. This represents a population of 504,800,000 (Eurostat 2009).

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

The effects of N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the designated product in patients with pancreatic cancer had been started.

At the time of submission, this medicine was not authorised anywhere in the EU for pancreatic cancer or designated as 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 2 September 2009 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 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:**

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Withdrawn

**Translations of the active ingredient and indication in all official EU languages,  
Norwegian and Icelandic**

Language	Active ingredient	Indication
English	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl)amino]isonicotinamide hydrochloride	Treatment of pancreatic cancer
Bulgarian	N-[(2S)-2,3-дихидроксипропил]-3-[(2-флуоро-4-йодфенил)амино] изоникотинамид хидрохлорид	Лечение на рак на панкреаса
Czech	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluor-4-jodofenyl)amino] isonikotinamid hydrochlorid	Léčba karcinomu pankreatu
Danish	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluor-4-iodophenyl) amino] isonicotinamidhydrochlorid	Behandling af pancreascancer
Dutch	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluor-4-joodfenyl) amino] isonicotinamide hydrochloride	Behandeling van pancreaskanker
Estonian	N-[(2S)-2,3-dihüdrosüpropüül]-3-[(2-fluoro-4-jodofenüül) amino] isonikotiinamiidvesinikkloriid	Pankreasevähi ravi
Finnish	N-[(2S)-2,3-dihydroksipropyyl]-3-[(2-fluoro-4-jodifenyyl) amino] isonikotiinamidihydrokloridi	Haimasyövän hoito
French	chlorhydrate de N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide	Traitement du cancer pancréatique
German	N-[(2S)-2,3-Dihydroxypropyl]-3-[(2-fluor-4-iodophenyl)amino]isonicotinamidhydrochlorid	Behandlung des Pankreaskarzinoms
Greek	N-[(2S)-2,3-διυδροξυπροπυλ]-3-[(2-φθόρο-4-ιωδοφαινυλ)άμινο] ισονικωτιναμιδιο υδροχλωρικο	Θεραπεία καρκίνου του παγκρέατος
Hungarian	N-[(2S)-2,3-dihidroxipropil]-3-[(2-fluoro-4-jodofenil) amino] izonikotinamid-hidroklorid	Hasnyálmirigyrák kezelése
Italian	N-[(2S)-2,3-diidrossipropil]-3-[(2-fluoro-4-iodofenil) amino] isonicotinamide cloridrato	Trattamento del cancro pancreatico
Latvian	N-[(2S)-2,3-dihidroksipropil]-3-[(2-fluoro-4-jodofenil) amino] izonikotīnamīda hidrohlorīds	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	N-[(2S)-2,3-dihidroksipropil]-3-[(2-fluoro-4-jodofenil) amino] izonikotinamido hidrochloridas	Kasos vėžio gydymas
Maltese	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride	Kura tal-kanċer tal-frixa
Polish	N-[(2S)-2,3-dihydroksypropylo]-3-[(2-fluoro-4-jodofenylo)amino] izonikotynamidu chlorowodorek	Leczenie raka trzustki
Portuguese	Cloridrato de N-[(2S)-2,3-dihidroxipropil]-3-[(2-fluoro-4-iodofenil) amino] isonicotinamida	Tratamento do carcinoma do pâncreas
Romanian	Clorhidrat de N-[(2S)-2,3-dihidroxipropil]-3-[(2-fluoro-4-iodofenil) amino] izonicotinamidă.	Tratamentul cancerului pancreatic
Slovak	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodofenyl) amino] isonikotinamid hydrochlorid	Liečba rakoviny pankreasu
Slovenian	N-[(2S)-2,3-dihidroksipropil]-3-[(2-fluoro-4-jodofenil) amino] izonikotinamidijev klorid	Zdravljenje raka trebušne slinavke
Spanish	Clorhidrato de N-[(2S)-2,3-dihidroxipropil]-3-[(2-fluoro-4-jodofenil) amino] isonicotinamida	Tratamiento del cáncer de páncreas
Swedish	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-jodofenyl) amino] isonikotinamid hydroklorid	Behandling av pancreascancer

Norwegian	N-[(2S)-2,3-dihydroksypropyl]-3-[(2-fluor-4-jodfenyl) amino] isonikotinamidhydroklorid	Behandling av pankreascancer
Icelandic	N-[(2S)-2,3-tvíhýdroxyprópýl]-3-[(2-flúró-4-jodófenýl) amínó] ísónikótínamíð hýdróklóríð	Meðferð briskrabbameins

Withdrawn