



## **Committee for Orphan Medicinal Products**

### **Public summary of positive opinion for orphan designation of becatecarin for the treatment of cancers of the biliary tree**

On 25 July 2006, orphan designation (EU/3/06/388) was granted by the European Commission to Helsinn Birex Pharmaceuticals Ltd, Ireland, for becatecarin for the treatment of cancers of the biliary tree.

#### **What is cancer of the biliary tree?**

The biliary tree (or biliary tract) is the path through which bile is secreted by the liver cells (hepatocytes) on its way to the duodenum, or small intestine. Between meals, bile is stored in the gallbladder and after meals it is discharged into the duodenum where it aids the digestion of lipids. Cholangiocarcinoma (intrahepatic and extrahepatic) and gallbladder carcinoma are the most frequent malignant tumours of the biliary tree. They are characterised by various clinical features such as abnormal liver function tests, abdominal pain, yellowish discoloration of the skin, and weight loss. They are often diagnosed when the disease has reached a late stage, worsening the prognosis for the patient. Cancer of the biliary tract is a life-threatening disease.

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

At the time of designation cancers of the biliary tree affected approximately 0.8 in 10,000 people in the European Union (EU)\*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 37,000 people.

#### **What treatments are available?**

The only possibly curative treatment for patients with these tumours is surgical excision at an early stage. However, anatomic tumour location, poor physical condition or the presence of metastatic disease frequently precludes surgery as an option. Most cancers of the biliary tree are treated like other cancers of the gastrointestinal tract (the stomach and the intestines), with chemotherapy (using drugs to kill cancer cells).

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that becatecarin might be of potential significant benefit for the treatment of cancers of the biliary tree, mainly because it has a new mechanism of action and may be used in patients that are not able to be treated surgically. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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\*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

**How is this medicine expected to work?**

Topoisomerase enzymes are proteins that catalyze the breaking and rejoining of the genetic material (DNA) when a cell is dividing. Becatecarin is an agent that belongs to a group of so called topoisomerase inhibitors. Topoisomerase inhibitors interfere with the action of topoisomerase enzymes. The mechanism of action of becatecarin is not exactly known, but it is thought that by inhibiting (blocking) the function of topoisomerase enzymes, it will destroy cancer cells and slow down the growth of the tumour.

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

The effects of becatecarin were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with cancers of the biliary tree were ongoing.

Becatecarin was not authorised anywhere worldwide for the treatment of cancers of the biliary tree, at the time of submission. Orphan designation of becatecarin was granted in the United States in March 2004 for treatment of bile duct tumours.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 15 June 2006 a positive opinion recommending the grant of the above-mentioned 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

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

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
English	Becatecarin	Treatment of cancers of the biliary tree
Czech	Becatecarin	Léčba rakoviny žlučových cest
Danish	Becatecarin	Behandling af cancer i galde systemet
Dutch	Becatecarin	Behandeling van kanker van de galwegen
Estonian	Becatecarin	Sapiteede vähkkasvajate ravi
Finnish	Bekatekariini	Sappiteiden syövän hoito
French	Bécatécarine	Traitement des cancers des voies biliaires
German	Becatecarin	Behandlung von Karzinomen des Gallengangssystems
Greek	Βεκατεκαρίνη	Θεραπευτική αγωγή για καρκίνους του χοληφόρου δέντρου
Hungarian	Becatecarin	Epeúti tumorok kezelése
Italian	Becatecarina	Trattamento dei tumori dell'albero biliare
Latvian	Bekatekarīns	Žultsceļu vēža ārstēšana
Lithuanian	Bekatekarinas	Tulžies latakų vėžio gydymas
Polish	Bekatekarin	Leczenie raka dróg żółciowych
Portuguese	Becatecarina	Tratamento do cancro da árvore biliar
Slovak	Bekatekarín	Liečba rakoviny žľočových ciest
Slovenian	Bekatekarin	Zdravljenje raka žolčnih vodov in žolčnika
Spanish	Becatecarina	Tratamiento del cáncer del árbol biliar
Swedish	bekatekarin	Behandling av cancer i gallgångarna
Norwegian	Bekatecarin	Behandling av kreft i gallegangene
Icelandic	Becatecarín	Meðferð gallvegakrabbameins