



EMA/COMP/369/2004 Rev.2  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Porfimer sodium (for use with photodynamic therapy) for the treatment of cholangiocarcinoma

First publication	22 November 2004
Rev.1: transfer of sponsorship	20 September 2011
Rev.2: withdrawal from the Community Register	3 April 2013
<b>Disclaimer</b> 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.	

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2013 on request of the Sponsor.***

On 29 July 2004, orphan designation (EU/3/04/215) was granted by the European Commission to Axcan Pharma International BV, the Netherlands, for porfimer sodium (for use with photodynamic therapy) for the treatment of cholangiocarcinoma.

The sponsorship was transferred to Pinnacle Biologics B.V., The Netherlands, in August 2011.

### What is cholangiocarcinoma?

Bile, which is secreted by the liver and stored in the gall-bladder, is discharged through the bile ducts (a passage with well-defined walls) into the intestine a few centimetres below the stomach, where it contributes to the digestion of fats. Tumours that begin in the bile ducts are known as cholangiocarcinomas. Cholangiocarcinoma mainly affects older people and is a life-threatening condition.



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

At the time of designation, cholangiocarcinoma affected not more than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 46,000 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).

## **What treatments are available?**

Surgery may be undertaken to remove the cancer, or surgical devices may be inserted to relieve the pressure in the bile ducts caused by the tumour, which is obstructing the bile ducts. At the time of submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for treatment of the condition.

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

Porfimer sodium is an agent that increases the sensitivity of tissues to the effects of light. Porfimer is used before the administration of laser light to cancerous tumours to decrease tumour size. Porfimer sodium will only be able to change the sensitivity of tissues if there is light and oxygen present. This might then result in the destruction of the cancer cells, probably due to generation of substances called free radicals and through interruption of the blood supply to the tumour.

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

The effects of porfimer sodium have been evaluated in experimental models.

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

Porfimer sodium was not marketed anywhere worldwide for cholangiocarcinoma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

Porfimer sodium also has an orphan designation and is authorised in the European Union for treatment of high-grade dysplasia in Barrett's oesophagus.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 June 2004 recommending the granting of this designation.

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\*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 25), Norway, Iceland and Lichtenstein. At the time of designation, this represented a population of 464,200,000 (Eurostat 2004).

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	Porfimer sodium (for use with photodynamic therapy)	Treatment of cholangiocarcinoma
Bulgarian	Натриев порфимер (за използване с фотодинамична терапия)	Лечение на холангиокарцином.
Czech	Porfimer sodný( pro fotodynamickou terapii)	Léčba cholangiokarcinomu
Danish	Porfimer natrium (til anvendelse med fotodynamisk terapi)	Behandling af cholangiocarcinom
Dutch	Porfimeer-natrium (voor gebruik bij photodynamische therapie)	Behandeling van cholangiocarcinoma
Estonian	Porfimeernaatrium (fotodünaamilise raviga kasutamiseks)	Kolangiokartsinoomi ravi
Finnish	Porfimerinatrium (käytettäväksi fotodynaamisen hoidon yhteydessä)	Kolangiokarsinooman hoito
French	Porfimère sodique (pour utilisation associée au traitement photodynamique)	Traitement du cholangiocarcinome
German	Porfimer-Natrium (für die Anwendung mit photodynamischer Therapie)	Behandlung von Gallengangskarzinom
Greek	Porfimer sodium (για χρήση με φωτοδυναμική θεραπεία)	Αγωγή για χολαγγειοκαρκίνωμα
Hungarian	Porfimerum nátrium (fotodinámiás kezelés céljára)	Cholangiocarcinoma kezelésé
Italian	Porfimer sodico (per uso associato a terapia fotodinamica)	Trattamento del colangiocarcinoma
Latvian	Nātrija porfimērs (lietošanai ar fotodinamikas terapiju)	Žultsvada karcinomas ārstēšana
Lithuanian	Porfimerio natrio druska (vartojimui su fotodinamine terapija)	Cholangiokarcinomos gydymas
Maltese	Porfimer sodium (għall-użu ma' terapija fotodinamika)	Kura tal-kolanġokarċinoma
Polish	Porfimer sodowy (do stosowania z leczeniem fotodynamicznym)	Leczenie raka dróg żółciowych
Portuguese	Porfímero sódico (para uso com terapia fotodinâmica)	Tratamento do colangiocarcinoma
Romanian	Porfimer sodic (pentru uz împreună cu terapia fotodinamică)	Tratamentul colangiocarcinomului
Slovak	Porfimer sodný (pre použitie s fotodynamickou terapiou)	Liečba cholangiokarcinómu
Slovenian	Porfimer-natrij (za uporabo pri fotodinamičnem zdravljenju)	Zdravljenje holangiokarcinoma
Spanish	Porfímero sódico (para el uso con terapia fotodinámica)	Tratamiento del colangiocarcinoma

<sup>1</sup> At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Swedish	Porfimeronatrium (för användning med fotodynamisk terapi)	Behandling av kolangiokarcinom