EMA/COMP/69/2002 Rev.5
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Porfimer sodium (for use with photodynamic therapy) for the treatment of high-grade dysplasia in Barrett's oesophagus

First publication	6 January 2003
Rev.1: administrative update	23 January 2003
Rev.2: information about Marketing Authorisation	29 November 2005
Rev.3: text correction	28 February 2007
Rev.4: transfer of sponsorship	20 September 2011
Rev.5: withdrawal from the Community Register	5 June 2013
Disclaimer 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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2013 on request of the Sponsor.

On 6 March 2002, orphan designation (EU/3/02/086) 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 high-grade dysplasia in Barrett's oesophagus.

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

What is high-grade dysplasia in Barrett's oesophagus?

Barrett's oesophagus (BO) is a condition in which the normal lining of the lower part of the gullet, is replaced over time by another type of lining, normally present in the stomach. BO is clearly recognisable at endoscopy. Usually BO develops during the process of healing after a chronic injury to the gullet, such as caused by reflux of gastric juice from the stomach to the gullet. Continued reflux causes initially mild (low-grade), and later, severe (high-grade) dysplasia. Dysplasia may lead to cancer of the gullet (oesophageal carcinoma) and as such is a life-threatening condition.

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

At the time of designation, high-grade dysplasia in Barrett's oesophagus affected approximately 3.6 in 10,000 people in the European Union (EU). This is equivalent to a total of around 137,000 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?

High-grade dysplasia requires careful endoscopic follow-up with multiple biopsies. As an alternative approach, surgery of the BO segment can be used. 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 a photosensitizing agent used in photodynamic therapy. The actions of porfimer sodium are light and oxygen dependent. The induced cell toxicity may be due to free radical generation and through interruption of the blood supply to the tumour.

What is the stage of development of this medicine?

The effects of the medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with high-grade dysplasia in BO were ongoing.

Porfimer sodium had not been marketed anywhere worldwide for high grade dysplasia in BO or designated as an orphan medicinal product elsewhere for this condition, at the time of submission.

Update: Porfimer sodium (for use with photodynamic therapy) (PhotoBarr) was authorised in the EU since 25 March 2004. Photodynamic therapy (PDT) with PhotoBarr was indicated for ablation of high-grade dysplasia (HGD) in patients with Barrett's Oesophagus (BO).

The marketing authorisation holder (MAH) responsible for PhotoBarr requested the withdrawal of the marketing authorisation in 2011.On 20 April 2012 the European Commission issued a decision to withdraw the marketing authorisation for PhotoBarr.

More information can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 December 2001 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.
At the time of designation, this represented a population of 380,600,000 (Eurostat 2002).

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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Porfimer sodium (for use with photodynamic therapy)	Treatment of high-grade dysplasia in Barrett's Esophagus
Bulgarian	Натриев порфимер (за използване с фотодинамична терапия)	Лечение на високостепенна дисплазия при хранопровод на Барет
Czech	Porfimer sodný(pro fotodynamickou terapii)	Léčba vysoce rizikové dysplazie v Barrettově jícnu
Danish	Porfimer natrium (for anvendning med fotodynamisk terapi)	Behandling af dysplasi af høj grad ved Barrets esophagus
Dutch	Profimeer-natrium (voor gebruik bij photodynamische therapie)	Behandeling van hoge graad dysplasie bij Barrett's slokdarm
Estonian	Porfimeernaatrium (fotodünaamilise raviga kasutamiseks)	Barrett'i söögitoru kõrge raskusastme düsplaasia ravi
Finnish	Porfimerinatrium (käytettäväksi fotodynaamisen hoidon yhteydessä)	Korkea–asteisen dysplasian hoito Barretin esofagus potilailla
French	Porfimère sodique (pour utilisation associée au traitement photodynamique)	Traitement des dysplasies de haut grade dans les syndromes de Barrett
German	Porfimer-natrium (für die Anwendung mit photodynamischer Therapie)	Behandlung der hochgradigen Dysplasie des Barret's Ösophagus
Greek	Porfimer sodium (για χρήση με φωτοδυναμική θεραπεία)	Θεραπεία υψηλού βαθμού δυσπλασίας οισοφάγου του συνδρόμου Barrett
Hungarian	Porfimerum nátrium (fotodinámiás kezelés céljára)	Barrett esophagushoz társuló magasfokú diszplázia kezelése
Italian	Porfimer sodico (per uso associato a terapia fotodinamica)	Trattamento della displasia ad alto grado nell'esofago di Barret
Latvian	Nātrija porfimērs (lietošanai ar fotodinamikas terapiju)	Augstas pakāpes displāzijas Bareta barības vada ārstēšana
Lithuanian	Porfimero natrio druska (vartojimui su fotodinamine terapija)	Aukštos rizikos displazijos, esant Bareto stemplei, gydymas
Maltese	Porfimer sodium (għall-użu ma' terapija fotodinamika)	Kura tad-displasja ta' grad għoli fl-esofagu ta' Barrett
Polish	Porfimer sodowy (do stosowania z leczeniem fotodynamicznym)	Leczenie dysplazji dużego stopnia w przełyku Barretta
Portuguese	Porfímero sódico (para utilização associada à terapia fotodinâmica)	Tratamento de displasia de alta graduação no síndroma de Barrett
Romanian	Porfimer sodic (pentru uz împreună cu terapia fotodinamică)	Tratamentul displaziei de grad înalt la pacienţii cu esofag Barett
Slovak	Porfimer sodný (pre použitie s fotodynamickou terapiou)	Liečba dysplázie ťažkého stupňa pri Barrettovom pažeráku
Slovenian	Porfimer-natrij (za uporabo pri fotodinamičnem zdravljenju)	Zdravljenje visokostopenjske displazije Barretovega požiralnika

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
Spanish	Porfímero sódico (para el uso asociado a la terapia fotodinámica)	Tratamiento de la displasia de alto grado en el esofágo de Barrett
Swedish	Porfimernatrium (för användning med fotodynamisk terapi)	Behandling av höggradig dysplasi vid Barretts esofagus.