5 May 2015
EMA/COMP/126073/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin for the treatment of biliary tract cancer

On 19 March 2015, orphan designation (EU/3/15/1470) was granted by the European Commission to Luzitin S.A., Portugal, for 5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl) bacteriochlorin for the treatment of biliary tract cancer.

What is biliary tract cancer?

Biliary tract cancer is cancer of the bile ducts and gallbladder. These are parts of the digestive system that transport and store bile, a fluid which is produced by the liver and released into the intestines after a meal to help digest fats. The cancer is characterised by various clinical features such as abnormal liver function tests, pain in the belly, yellowish discoloration of the skin, and weight loss.

Biliary tract cancer is a long-term debilitating and life-threatening disease which is often diagnosed when the disease has reached a late stage, worsening the prognosis for the patient.

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

At the time of designation, biliary tract cancer affected approximately 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 77,000 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?

At the time of designation, choice of treatment for biliary tract cancer depended mainly on how advanced the disease was. Some patients with early disease could undergo surgery to remove the cancer. Other treatments included chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with biliary tract cancer because it works in a different way to existing treatments

*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 512,900,000 (Eurostat 2015).

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by specifically targeting the cancer. In addition, the medicine may be used in combination with existing treatments to improve the outcome of patients with the condition. These assumptions 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 a photosensitising agent (a substance that changes when exposed to light). When injected into the patient, the medicine is absorbed into cells throughout the body. When laser light of a specific wavelength is shone at the cancer area, the photosensitising agent is activated and reacts with oxygen in the cells to create toxic molecules containing oxygen called 'reactive oxygen species' which are expected to kill the cancer cells.

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 biliary tract cancer had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for biliary tract 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 12 February 2015 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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin	Treatment of biliary tract cancer
Bulgarian	5,10,15,20-тетракис(2,6-дифлуоро-3-N-метилсулфамоилфенил)бактериохлорин	Лечение на рак на жлъчните пътища
Croatian	5,10,15,20-tetrakis(2,6-difluoro-3-N-metilsulfamoilfenil)bakterioklorin	Liječenje raka bilijarnog trakta
Czech	5,10,15,20-tetrakis(2,6-difluor-3-N-metylsulfamoylfenyl)bakteriochlorin	Léčba karcinomu žlučových cest
Danish	5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin	Behandling af galdegangscancer
Dutch	5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylfenyl)bacteriochlorine	Behandeling van galweg kanker
Estonian	5,10,15,20-tetrakis(2,6-difluoro-3-N-metüülsulfamoüülphenüül)-bakteriokloriin	Sapiteede kasvaja ravi
Finnish	5,10,15,20-tetrakis(2,6-difluori-3-N-metyylisulfamoyylifenyyli)bakteriokloriini	Sappiteiden syövän hoito
French	Bactériochlorine 5,10,15,20-tétrakis(2,6-difluoro-3-N-méthylsulfamoylphényl)	Traitement du cancer des voies biliaires
German	5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin	Behandlung von Tumoren der Gallenwege
Greek	5,10,15,20-τετράκις(2,6-διφθορο-3-N-μεθυλοσουλφαμοϋλοφαινυλο) βακτηριοχλωρίνη	Θεραπεία του καρκίνου της χοληφόρου οδού
Hungarian	5,10,15,20-tetrakisz(2,6-difluoro-3-N-metilsulfamoilfenil)bakterioklorin	Epeúti rák kezelése
Italian	5,10,15,20-tetra(2,6-difluoro-3-N-metilsolfamoilfenile)batterioclorina	Trattamento del carcinoma delle viebiliari
Latvian	5,10,15,20-tetrakis(2,6-diflor-3-N-metilsulfamoilfenil)bakteriohlorīns	Žultsvadu sistēmas vēža ārstēšana
Lithuanian	5,10,15,20-tetrakis(2,6-difluoro-3-N-metilsulfamoilfenil)bakteriochlorinas	Tulžies latakų vėžio gydymas
Maltese	5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin	Kura tal-kanċer tal-apparat tal-bili

[1] At the time of designation

Language	Active ingredient	Indication
Polish	5,10,15,20-tetrakis(2,6-difluoro-3-N-metylosulfamoilofenylo)bakteriochloryna	Leczenie raka dróg żółciowych
Portuguese	5,10,15,20-tetraquis(2,6-difluoro-3-N-metilsulfamoilfenil)bacterioclorina	Tratamento da neoplasia das vias biliares
Romanian	5,10,15,20-tetrakis(2,6-difluoro-3-N-metilsulfamoilfenil)bacterioclorină	Tratamentul cancerului de căi biliare
Slovak	5,10,15,20-tetrakis(2,6-difluóro-3-N-metylsulfamoylfenyl)bakteriochlorín	Liečba karcinómu žlčových ciest
Slovenian	5,10,15,20-tetrakis(2,6-difluoro-3-N-metilsulfamoilfenil)bakterioklorin	Zdravljenje raka žolčnih vodov
Spanish	5,10,15,20-tetrakis(2,6-difluoro-3-N-metilsulfamoilfenilo)bacterioclorina	Tratamiento del cáncer del árbol biliar
Swedish	5,10,15,20-tetrakis(2,6-difluoro-3-N-metylsulfamoylfenyl)bakterioklorin	Behandling av gallvägscancer
Norwegian	5,10,15,20-tetrakis(2,6-difluoro-3-N-metylsulfamoylfenyl)bakterioklorin	Behandling av gallegangskreft
Icelandic	5,10,15,20-tetrakís(2,6-díflúor-3-N-metýlsúlfamoýlfenýl)bakteríoklórín	Meðferð við krabbameini í gallvegum