2 March 2011
EMA/COMP/16280/2011
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Paclitaxel (aqueous gel) for the treatment of oesophagus carcinoma

On 23 February 2011, orphan designation (EU/3/10/846) was granted by the European Commission to BTG plc, United Kingdom, for paclitaxel (aqueous gel) for the treatment of oesophagus carcinoma.

What is oesophagus carcinoma?

Oesophagus carcinoma is cancer of the oesophagus (the tube that connects the mouth to the stomach). It usually starts in the cells lining the oesophagus, and spreads easily to other parts of the body. Oesophagus carcinoma is often detected late because in the early stage of the disease patients do not have significant symptoms. The cancer is usually diagnosed in people aged over 60 years, and men are about twice as likely to develop the disease as women.

Oesophagus carcinoma is a life-threatening disease because of its serious complications such as dysphagia (difficulty swallowing) that lead to poor overall survival.

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

At the time of designation, oesophagus carcinoma affected approximately 0.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 25,000 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?

The choice of treatment for oesophagus carcinoma depends on where in the oesophagus the cancer is located, and how advanced it is. At the time of orphan designation, the main treatment was surgery, sometimes preceded by radiotherapy (treatment with radiation) or chemotherapy (medicines to treat cancer) to make the cancer shrink and easier to remove. Other medicines were also used after surgery to treat complications.

*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 506,500,000 (Eurostat 2010).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

The sponsor has provided sufficient information to show that paclitaxel (aqueous gel) might be of significant benefit for patients with oesophagus carcinoma because the medicine is expected to be given locally, causing fewer side effects than systemic treatments, such as standard chemotherapy, which reach cells throughout the body. 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?

Paclitaxel has been available as an anticancer medicine since 1993. It belongs to the group of anticancer medicines known as the 'taxanes'. Paclitaxel blocks the ability of cancer cells to break down their internal 'skeleton' that allows them to divide and multiply. With their skeleton still in place, the cells cannot divide and they eventually die.

Paclitaxel (aqueous gel) is a depot formulation of paclitaxel that is expected to be given by injection directly into the tumour. This is a type of formulation where the medicine is prepared so that it is slowly released. The paclitaxel is expected to mainly remain within the tumour without spreading throughout the body, thereby causing fewer side effects.

What is the stage of development of this medicine?

The effects of paclitaxel (aqueous gel) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with oesophagus carcinoma was ongoing.

At the time of submission, paclitaxel (aqueous gel) was not authorised anywhere in the EU for oesophagus carcinoma. Orphan designation of paclitaxel (aqueous gel) had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 December 2010 recommending the granting of this designation.

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:

BTG plc
5 Fleet Place
London EC4M 7RD
United Kingdom
Telephone: + 44 20 7575 0000
Telefax: + 44 20 7575 0010
E-mail: info@btgplc.com

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	Paclitaxel (aqueous gel)	Treatment of oesophagus carcinoma
Bulgarian	Паклитаксел (воден гел)	Лечение на карцином на хранопровода
Czech	Paclitaxel (vodný gel)	Léčba karcinomu jícnu
Danish	Paclitaxel (vandig gel)	Behandling af esophaguskarcinom
Dutch	Paclitaxel (waterige gel)	Behandeling van slokdarmcarcinoom
Estonian	Paclitaxel (veepõhine geel)	Söögitoru kartsinoomi ravi
Finnish	Paklitakseeli (vesipitoinen geeli)	Ruokatorven syövän hoito
French	Paclitaxel (gel aqueux)	Traitement du cancer de l'oesophage
German	Paclitaxel (wässriges Gel)	Behandlung des Ösophaguskarzinoms
Greek	Paclitaxel (υδατική γέλη)	θεραπεία οισοφαγικού καρκινώματος
Hungarian	Paclitaxel (vizes gél)	Nyelőcsőrák kezelése
Italian	Paclitaxel (gel acquoso)	Trattamento chirurgico del carcinoma esofageo
Latvian	Paclitaksels (ūdens gēls)	Barības vada karcinomas ārstēšana
Lithuanian	Paklitakselis (vandeninis gelis)	Stemplės karcinomos gydymas
Maltese	Paclitaxel (ġell bl-ilma)	Kura tal-karċinoma tal-esofagu
Polish	Paklitaksel (wodny żel)	Leczenie raka przełyku
Portuguese	Paclitaxel (gel aquoso)	Tratamento do carcinoma do esófago
Romanian	Paclitaxel (gel apos)	Tratamentul cancerului esofagian
Slovak	Paklitaxel (vodný gél)	Liečba karcinómu pažeráka
Slovenian	Paklitaksel (vodni gel)	Zdravljenje raka na požiralniku
Spanish	Paclitaxel (gel acuoso)	Tratamiento del carcinoma de esófago
Swedish	Paklitaxel (vattenhaltig gel)	Behandling av cancer i matstrupe
Norwegian	Paklitaksel (vandig gel)	Behandling av øsofaguscancer
Icelandic	Paklítaxel (vatnslausnarhlaup)	Meðferð á krabbameini í vélinda

[1] At the time of designation