



21 May 2015
EMA/COMP/252166/2014 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Paclitaxel-succinate-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe for the treatment of glioma

First publication	2 July 2014
Rev.1: transfer of sponsorship	21 May 2015
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.	

On 4 June 2014, orphan designation (EU/03/14/1274) was granted by the European Commission to CLL Pharma, France, for paclitaxel-succinate-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe for the treatment of glioma.

The sponsorship was transferred to Orphit, France, in April 2015.

What is glioma?

Glioma is a type of brain tumour that affects the 'glial' cells (the cells that surround and support the nerve cells). Patients with glioma can have severe symptoms, but the types of symptoms experienced depend on where the tumour develops in the brain.

Symptoms can include headaches, nausea (feeling sick), loss of appetite, vomiting, and changes in personality, mood, mental capacity and concentration. About a fifth of patients with glioma have seizures (fits) for months or years before the disease is diagnosed.

Glioma is a long-term debilitating and life-threatening disease because of the severe damage to the brain, and is associated with poor long-term survival.



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

At the time of designation, glioma affected approximately 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 113,000 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, several medicines were authorised for the treatment of glioma in the EU. Treatments for glioma included surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines to treat cancer) to improve survival. Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure within the skull and medicines to prevent seizures.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with glioma because data from experimental models showed improved survival in comparison with published results with an authorised medicine. 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?

This medicine contains paclitaxel, a cancer medicine that has been available since the 1990s. Paclitaxel belongs to a group of medicines known as 'taxanes'. Paclitaxel blocks the ability of cancer cells to break down their internal 'skeleton', which allows them to divide and multiply. With their skeleton still in place, the cells cannot divide and they eventually die. Paclitaxel is a very insoluble substance, which means that it cannot easily cross the blood-brain barrier in order to reach the regions in the brain affected by glioma.

In this medicine, paclitaxel has been attached to a peptide (a short chain of amino acids, the building blocks of proteins). This peptide is thought to increase the solubility of paclitaxel and allow it to better cross the blood-brain barrier, thereby improving its delivery to the glioma 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 glioma had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for glioma 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 9 April 2014 recommending the granting of this designation.

*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. At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

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:

Orphit
1093 Chemin des Plateaux
Fleuris Résidences Panoramiques Villa B
06700 Saint-Laurent-Du-Var
France
Tel. +33 6 27 27 15 77
E-mail: steph.laruelle@free.fr

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Paclitaxel-succinate-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Treatment of glioma
Bulgarian	Паклитаксел-сукцинат-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Лечение на глиома
Croatian	Paklitakselsukcinat-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Liječenje glioma
Czech	paclitaxel-sukcinát-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Léčba gliomů
Danish	Paclitaxel-succinat-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Behandling af gliom
Dutch	Paclitaxel-succinaat-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Fen	Behandeling van glioma
Estonian	Paklitakseel-suktsinaat Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Glioomi ravi
Finnish	Paklitakseli-sukkinaatti-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Gliooman hoito
French	Succinate de Paclitaxel-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Traitement des gliomes
German	Paclitaxel-Succinat (Bernsteinsäure)-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Behandlung von Gliomen
Greek	Σουξινική πακλιταξέλη-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Θεραπεία του γλοιώματος
Hungarian	Paclitaxel-szukcinát-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Glioma kezelése
Italian	Paclitaxel-succinato-Arg-Arg-Leu-Ser-Tir-Ser-Arg-Arg-Arg-Fen	Trattamento del glioma
Latvian	Paklitaksela-sukcināts-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Gliomas ārstēšana
Lithuanian	Paklitaksel-sukcinato-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Gliomos gydymas
Maltese	Paclitaxel-succinate-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Kura tal-glioma
Polish	Paklitaksel-bursztynian-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Leczenie glejaka
Portuguese	Paclitaxel-succinato-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Tratamento do glioma
Romanian	Paclitaxel-succinat-Arg-Arg-Leu-Ser-Tir-Ser-Arg-Arg-Arg-Fe	Tratamentul gliomului
Slovak	Paklitaxel-sukcinát-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Liečba gliómu

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Paclitaxel-sukcinat-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Zdravljenje glioma
Spanish	Paclitaxel-succinato-Arg-Arg-Leu-Ser-Tir-Ser-Arg-Arg-Arg-Fe	Tratamiento del glioma
Swedish	Paklitaxel-succinat-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Behandling av gliom
Norwegian	Paklitakselsuksinat-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe	Behandling av gliom
Icelandic	Paclítaxel-súkkínat-Arg-Arg-Leu-Ser-Týr-Ser-Arg-Arg-Arg-Phe	Meðferð á glíóma