



14 October 2013  
EMA/COMP/46/2002 Rev.2  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Carmustine (solution for intratumoural injection) for the treatment of glioma

First publication	6 January 2003
Rev.1: withdrawal from the Community Register	3 May 2012
Rev.2: administrative update	14 October 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***Please note that this product was withdrawn from the Community register of designated orphan medicinal products in April 2012 on request of the sponsor.***

On 5 March 2002, orphan designation (EU/3/02/085) was granted by the European Commission to Icon Clinical Research UK Ltd, United Kingdom, for carmustine (solution for intratumoural injection) for the treatment of glioma.

### What are gliomas?

Tumours that begin in brain tissue are known as primary brain tumours. Primary brain tumours are classified by the type of tissue from which they originate. The most common brain tumours are gliomas, which begin in the glial (supportive) tissue.

Due to their localisation, gliomas represent a potentially debilitating and life-threatening condition. Patients affected by gliomas can suffer from neurological complications, depending on the site of intra-cerebral development of the tumour.



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

At the time of designation, gliomas affected approximately 0.85 in 10,000 people in the European Union (EU). This was equivalent to a total of around 32,000 people<sup>\*</sup>, 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?**

Treatment for gliomas depends on a number of factors and may include surgery, radiotherapy or chemotherapy as well as symptomatic treatments, such as corticosteroids to control the effects of raised intracranial pressure, and anticonvulsants to help control seizures, as required. Methods of treatment of the condition had been authorised at the time of submission of the application for orphan designation. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that carmustine (solution for intratumoural injection) might be of potential significant benefit for the treatment of gliomas, particularly in terms of its new route of administration.

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

Carmustine is an anti-neoplastic medicinal product. Administration of this product blocks cell multiplication and consequently inhibits tumour growth. The product is administered directly into the brain tumour.

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

At the time of submission of the application for orphan designation, clinical trials in patients with inoperable recurrent malignant gliomas were ongoing.

Carmustine solution for intratumoural injection had not been marketed anywhere worldwide for gliomas, at the time of submission.

Carmustine solution for intratumoural injection has been granted orphan drug designation by the United States Food and Drug Administration for the treatment of intracranial malignancies on 3 July 2000.

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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<sup>\*</sup>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:

Icon Clinical Research UK Ltd  
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Telefax: +44 (0)1962 826001  
E-mail: [carmustine@iconuk.com](mailto:carmustine@iconuk.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<sup>1</sup>, Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Carmustine (solution for intratumoral injection)	Treatment of glioma
Danish	Carmustin (intratumoral injektionsvæske)	Behandling af gliom
Dutch	Carmustine (oplossing voor intratumorale injectie)	Behandeling van glioma
Finnish	Karmustiini (liuos kasvaimensisäistä injektiota varten)	Gliooman hoito
French	Carmustine (solution pour injection intratumorale)	Traitemenit des gliomes
German	Carmustin (Lösung für intratumorale Injektion)	Behandlung des Glioms
Greek	Καρμουστίνη (διάλυμα για ενδονεοπλασματική ένεση)	Θεραπεία γλοιώματος
Italian	Carmustina (soluzione iniettabile per via intratumorale)	Trattamento dei gliomi
Portuguese	Carmustina (solução para injecção intratumoral)	Tratamento de gliomas
Spanish	Carmustina (solución para inyección intratumoral)	Tratamiento de glioma
Swedish	Carmustin (lösning för intratumoral injektion)	Behandling av gliom

<sup>1</sup> At the time of designation