



**COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

**PUBLIC SUMMARY OF  
POSITIVE OPINION FOR ORPHAN DESIGNATION  
OF  
fampridine  
for the treatment of Guillain-Barré syndrome**

On 10 July 2007, orphan designation (EU/3/07/458) was granted by the European Commission to Dr Ulrich Granzer, Germany, for fampridine for the treatment of Guillain-Barré syndrome.

**What is Guillain-Barré syndrome?**

Guillain-Barré syndrome is an inflammation of the peripheral nervous system (i.e., nerves outside the brain or the spinal cord), caused by activation of the immune system and production of specific antibodies. It is characterized by destruction or loss of the myelin sheath that surrounds most nerve cells. Numbness in the toes is often the first neurological symptom, followed within hours or days by weakness of the legs or limbs. The condition is chronically debilitating and life-threatening.

**What are the methods of treatment available?**

Plasmapheresis (taking the blood out of the patient, filtering it and re-injecting it into the veins) is used to eliminate the antibodies that cause the disease. Several drugs were authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation. Fampridine may be of potential significant benefit in patients affected by Guillain-Barré syndrome. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, Guillain-Barré syndrome was considered to affect between 9,200 and 18,000 persons in the European Union.

**How is this medicinal product expected to act?**

The action of fampridine is believed to be through an improvement of the function of the affected nerve cells. The drug blocks specific proteins that transport potassium from outside to inside the cell which would hypothetically improve the conduction of the electrical impulse in the damaged nerve cells.

**What is the stage of development of this medicinal product?**

The effects of fampridine were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with Guillain-Barré syndrome were ongoing.

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\* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Fampridine was not authorised anywhere worldwide for the treatment of Guillain-Barré syndrome or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 31 May 2007 a positive opinion recommending the grant of the above-mentioned designation.

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Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

**For more information:**

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**Translations of the active ingredient and indication in all EU languages  
and Norwegian and Icelandic**

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
English	Fampridine	Treatment of Guillain-Barré syndrome
Bulgarian	Фампридин	Лечение на синдрома на Гилен-Баре
Czech	Fampridin	Léčba Guillainova-Barrého syndromu
Danish	Fampridin	Behandling af Guillain-Barré syndrom
Dutch	Fampridine	Behandeling van het Guillain-Barré syndroom
Estonian	Fampridiin	Guillaini-Barré sündroomi ravi
Finnish	Fampridiini	Guillain-Barrén oireyhtymän hoito
French	Fampridine	Traitement du syndrome de Guillain-Barré
German	Fampridin	Behandlung des Guillain-Barré-Syndroms
Greek	Φαμπριδίνη	Αγωγή για το σύνδρομο Guillain-Barré
Hungarian	Fampridine	Guillain-Barré szindróma kezelése
Italian	Fampridina	Trattamento della sindrome di Guillain-Barré
Latvian	Fampridīns	Gijēna Barē (Guillain-Barré) sindroma ārstēšana
Lithuanian	Fampridinas	Guillain-Barre sindromo gydymui
Maltese	Fampridine	Kura tas-sindrome Guillain-Barré
Polish	Fampridin	Leczenie zespołu Guillain-Barré
Portuguese	Fampridina	Tratamento do síndrome de Guillain-Barré
Romanian	Fampridină	Tratamentul sindromului Guillain-Barré-Strohl
Slovak	Fampridín	Liečba syndrómu Guillain-Barré
Slovenian	Fampridin	Zdravljenje Guillain-Barréjevega sindroma
Spanish	Fampridina	Tratamiento del síndrome Guillain-Barré
Swedish	Fampridin	Behandling av Guillain-Barrés syndrom
Norwegian	Fampridin	Behandling av Guillain-Barrés syndrom
Icelandic	Famprídín	Meðferð við Guillain-Barré heilkenni