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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fingolimod for the treatment of chronic inflammatory demyelinating polyneuropathy

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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 2 February 2010, orphan designation (EU/3/09/718) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for fingolimod for the treatment of chronic inflammatory demyelinating polyneuropathy.

What is chronic inflammatory demyelinating polyneuropathy?

Chronic inflammatory demyelinating polyneuropathy (CIDP) is a progressive disease of the peripheral nerves (nerves that branch out from the brain and spinal cord). In this disease, inflammation destroys the protective sheath around the nerves. It is believed to be caused by the patient's immune system attacking the nerves. This damage is mainly due to immune system cells called T cells and antibodies (proteins in the blood that normally help to fight infections). Patients with CIDP have numbness and unusual sensations in the hands and feet, pain and muscle weakness that gradually gets worse.

CIDP is a long-term debilitating and life-threatening disease because it may result in an inability to walk without help in a majority of patients and, less commonly, in damage to the lungs that does not allow the patient to breathe normally.

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What is the estimated number of patients?

At the time of designation, CIDP affected less than 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 40,000 people*, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, one medicine was authorised in Germany for the treatment of CIDP. In addition, several medicines were used in the EU to reduce the activity of the immune system, such as corticosteroids. In some cases, treatment consisted of 'plasma exchange', a procedure used to remove antibodies that are attacking the nerves by replacing the patient's plasma (the liquid part of the blood) with plasma from a donor.

The sponsor has provided sufficient information to show that fingolimod might be of significant benefit for patients with CIDP because early studies in experimental models indicate that it might improve the treatment of patients with this condition. In addition, fingolimod is expected to be available as capsules, whereas some of the existing treatments need to be given by infusion (drip into a vein). 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?

Fingolimod is expected to work by attaching to a receptor called sphingosine-1-phosphate receptor, which is involved in the movement of immune cells, including T cells, around the body. By attaching to this receptor, fingolimod is expected to stop T cells from moving out of the lymph nodes and into the blood. This is expected to reduce the number of T cells circulating in the blood and limit their ability to damage the nerves.

What is the stage of development of this medicine?

The effects of fingolimod have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the designated product in patients with CIDP had been started.

At the time of submission, fingolimod was not authorised anywhere in the EU for CIDP 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 3 December 2009 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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

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 Community) 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, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Fingolimod	Treatment of chronic inflammatory demyelinating polyneuropathy
Bulgarian	Финголимод	Лечение на хронична възпалителна демиелинизираща полиневропатия
Czech	Fingolimodum	Léčba chronické zánětlivé demyelinizační polyneuropatie
Danish	Fingolimod	Behandling af kronisk inflammatorisk demyeliniserende polyneuropati
Dutch	Fingolimod	Behandeling van chronische inflammatoire demyeliniserende polyneuropathie
Estonian	Fingolimood	Kroonilise põletikulise demüeliniseeriva polüneuropaatia ravi
Finnish	Fingolimodi	Kroonisen tulehduksellisen demyelinoivan polyneuropatian hoito
French	Fingolimod	Traitement de la polyneuropathie démyélinisante inflammatoire chronique
German	Fingolimod	Behandlung der chronisch entzündlichen demyelinisierenden Polyneuropathie
Greek	Φιγγολιμόδη	Θεραπεία της χρόνιας φλεγμονώδους απομυελινωτικής πολυνευροπάθειας
Hungarian	Fingolimod	Krónikus gyulladásos demyelinizációval járó polineuropátia kezelésére
Italian	Fingolimod	Trattamento della polineuropatia demielinizzante infiammatoria cronica
Latvian	Fingolimods	Hroniskas iekaisīgas demielinizējošas polineiropātijas ārstēšanai
Lithuanian	Fingolimodas	Lėtinės uždegiminės demielinizuojančios polineuropatijos gydymas
Maltese	Fingolimod	Kura tal-polinewropatija demajlinanti infjammatorja kronika
Polish	Fingolimod	Leczenie przewlekłej zapalnej polineuropatii demielinizacyjnej
Portuguese	Fingolimod	Tratamento da polineuropatia desmielinizante inflamatória crónica
Romanian	Fingolimod	Tratamentul polineuropatiei demielinizante inflamatorii cronice
Slovak	Fingolimod	Liečba chronickej zápalovej demyelinizačnej polyneuropatie
Slovenian	Fingolimod	Zdravljenje kronične vnetne demielinizacijske polinevropatije
Spanish	Fingolimod	Tratamiento de la polineuropatía desmielinizante inflamatoria crónica
Swedish	Fingolimod	Behandling av kronisk inflammatorisk demyeliniserande polyneuropati
Norwegian	Fingolimod	Behandling av kronisk inflammatorisk demyeliniserende polynevropati
Icelandic	Fingólimod	Meðferð á langvinnum afmýlingar bólgufjöltaugakvilla