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EMA/COMP/660610/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Siponimod for the treatment of polymyositis

On 19 November 2014, orphan designation (EU/3/14/1370) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for siponimod for the treatment of polymyositis.

What is polymyositis?

Polymyositis is an inflammatory disease of the muscles, characterised by muscle weakness, difficultly moving and tiredness. In addition to skeletal muscles (the muscles used for movement), the muscles of the oesophagus (food pipe), the respiratory system and the heart are sometimes affected, leading to difficulties in eating and breathing.

Polymyositis is an auto-immune disease. This means that it is caused by the body's immune system attacking its own tissues. The damage and inflammation are mainly due to immune system cells called T cells and macrophages. The reason why the immune system acts in this way is not known.

Polymyositis is a life-threatening and long-term debilitating condition particularly due to muscle weakness, lung and heart problems.

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

At the time of designation, polymyositis affected approximately 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 20,000 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, prednisolone was authorised for the condition in some EU countries.

The sponsor has provided sufficient information to show that siponimod might be of significant benefit for patients with polymyositis because early studies have shown improvement in muscle function in

*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. This represents a population of 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

patients for whom other treatments have stopped working. 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?

Siponimod is expected to work by attaching to some receptors called sphingosine-1-phosphate receptors (i.e. S1P1 and S1P5), which are involved in the movement of immune cells around the body. By attaching to these receptors, siponimod is expected to reduce the movement of these cells from the lymph nodes into the muscles where they can attack the tissues. This is expected to ease the symptoms of the disease.

What is the stage of development of this medicine?

The effects of siponimod have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with siponimod in patients with polymyositis were ongoing.

At the time of submission, siponimod was not authorised anywhere in the EU for polymyositis. Orphan designation of siponimod had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 October 2014 recommending the granting of this designation.

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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:

Novartis Europharm Limited
Frimley Business Park
Camberley GU16 7SR
United Kingdom
Tel. +41 613 2411 11
E-mail: orphan.enquiries@novartis.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	Siponimod	Treatment of polymyositis
Bulgarian	Сипонимод	Лечение на полимиозит
Croatian	Siponimod	Liječenje polimiozitisa
Czech	Siponimod	Léčba polymyositidy
Danish	Siponimod	Behandling af polymyositis
Dutch	Siponimod	Behandeling van polymyositis
Estonian	Siponimood	Polümüosiidi ravi
Finnish	Siponimodi	Polymyosiitin hoito
French	Siponimod	Traitement des polymyosites
German	Siponimod	Behandlung von Polymyositis
Greek	Σιπονιμόδη	Θεραπεία της πολυμυοσίτιδος
Hungarian	Sziponimod	Polymyositis kezelése
Italian	Siponimod	Trattamento delle polimiositi
Latvian	Siponimods	Polimiozīta ārstēšana
Lithuanian	Siponimodas	Polimiozito gydymas
Maltese	Siponimod	Kura tal-polimijosite
Polish	Syponimod	Leczenie zapalenia wielomięśniowego
Portuguese	Siponimod	Tratamento da polimiosite
Romanian	Siponimod	Tratamentul polimiozitei
Slovak	Siponimod	Liečba polymyozitídy
Slovenian	siponimod	zdravljenje polimiozitisa
Spanish	Siponimod	Tratamiento de la polimiositis
Swedish	Siponimod	Behandling av polymyosit
Norwegian	Siponimod	Behandling av polymyositt
Icelandic	Sípoónimód	Meðferð við fjölvöðvabólgu

[1] At the time of designation