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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Apremilast for the treatment of Behçet's disease

On 5 August 2013, orphan designation (EU/3/13/1180) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for apremilast for the treatment of Behçet's disease.

What is Behçet's disease?

Behçet's disease is an autoimmune disease in which the immune system attacks its own blood vessels. The exact cause of the disease is unknown. As a result of the damage to the blood vessels, patients develop symptoms such as painful sores in the mouth and on the genitals, inflammation inside the eye and skin problems. The inflammation inside the eye can lead to blurred vision and blindness.

Behçet's disease is a long-term debilitating disease because the symptoms such as the sores in the mouth and on the genitals and inflammation of the blood vessels throughout the body can last several weeks and cause permanent tissue damage.

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

At the time of designation, Behçet's disease affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 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, treatment for Behçet's disease focussed on relieving the symptoms of the disease, reducing discomfort and preventing serious complications. Commonly used treatments included medicines that reduce inflammation, such as steroids. Colchicine was also approved for Behçet's disease in some EU Member States.

The sponsor has provided sufficient information to show that apremilast might be of significant benefit for patients with Behçet's disease because early studies in patients showed that the medicine was

^{*}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. This represents a population of 509,000,000 (Eurostat 2013).

effective at reducing the number of mouth sores. 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?

Apremilast is expected to work by blocking the production of various messenger molecules, including interleukins and tumour necrosis factors. These messengers are involved in causing inflammation and are found at high levels in patients with Behçet's disease. By blocking their production, apremilast reduces the symptoms of Behçet's disease, such as the sores in the mouth.

What is the stage of development of this medicine?

The effects of apremilast have been evaluated in experimental models.

At the time of submission of the application for orphan designation, one clinical trial with apremilast in patients with Behçet's disease had been conducted.

At the time of submission, apremilast was not authorised anywhere in the EU for Behçet's disease. Orphan designation of apremilast 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 11 July 2013 recommending the granting of this designation.

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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Apremilast	Treatment of Behçet's disease
Bulgarian	Апремиласт	Лечение на болест на Бехчет
Croatian	Apremilast	Liječenje Behçetove bolesti
Czech	Apremilast	Léčba Behcetovy nemoci
Danish	Apremilast	Behandling af Behçets sygdom
Dutch	Apremilast	Behandeling van de ziekte van Behçet
Estonian	Apremilast	Behçeti tõve ravi
Finnish	Apremilasti	Behçetin taudin hoito
French	Aprémilast	Traitement de la maladie de Behçet
German	Apremilast	Behandlung des Morbus Behçet
Greek	Απρεμιλάστη	Θεραπεία της νόσου Αδαμαντιάδη-Behçet
Hungarian	Apremilast	Behçet-kór kezelése
Italian	Apremilast	Trattamento della malattia di Behçet
Latvian	Apremilasts	Behčeta slimības ārstēšana
Lithuanian	Apremilastas	Bechčeto (<i>Behçet</i>) ligos gydymas
Maltese	Apremilast	Kura tal-marda ta' Behçet
Polish	Apremilast	Leczenie choroby Behçeta
Portuguese	Apremilaste	Tratamento da doença de Behçet
Romanian	Apremilast	Tratamentul bolii Behçet
Slovak	Apremilast	Liečba Behçetovej choroby
Slovenian	Apremilast	Zdravljenje Behçetove bolezni
Spanish	Apremilast	Tratamiento de la enfermedad de Behçet
Swedish	Apremilast	Behandling av Behçets sjukdom
Norwegian	Apremilast	Behandling av Behçets sykdom
Icelandic	Apremilast	Meðferð við Behcet sjúkdómi

¹ At the time of designation