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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant humanised anti-human interleukin-1 beta monoclonal antibody for the treatment of Behçet's disease

On 1 October 2010, orphan designation (EU/3/10/796) was granted by the European Commission to XOMA Ireland Ltd, Ireland, for recombinant humanised anti-human interleukin-1 beta monoclonal antibody for the treatment of Behçet's disease.

The sponsorship was transferred to Les Laboratoires Servier, France, in December 2011.

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, pain, and redness.

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 is equivalent to a total of fewer than 51,000 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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, there were no treatments that could cure Behçet's disease. Treatment focussed on relieving the symptoms of the disease, reducing discomfort and preventing serious

[*]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 506,500,000 (Eurostat 2010).

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complications. Commonly used treatments included medicines that reduce inflammation, such as steroids. Colchicine was approved for Behçet's disease in one Member State.

The sponsor has provided sufficient information to show that recombinant humanised anti-human interleukin-1 beta monoclonal antibody might be of significant benefit for patients with Behçet's disease because it works in a different way to existing treatments, and because early studies indicate that it might improve the ocular (eye) symptoms of the disease. 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?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a chemical messenger in the blood called interleukin-1 beta. Interleukin-1 beta is involved in the process of inflammation and is thought to play a role in Behçet's disease development. By attaching to interleukin-1 beta, this medicine is expected to block its activity, helping to relieve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of recombinant humanised anti-human interleukin-1 beta monoclonal antibody have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with Behçet's disease were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for Behçet's disease 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 8 July 2010 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:

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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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant humanised anti-human interleukin-1 beta monoclonal antibody	Treatment of Behçet's Disease
Bulgarian	Рекомбинантно хуманизирано анти-човешко интерлевкин-1 бета моноклонално антитяло	Лечение на Болест на Бехчет
Czech	Rekombinantní humanizovaná monoklonální protilátka proti anti humánnímu interleukinu-1 beta	Léčba Behcetova choroba
Danish	Rekombinant humaniseret anti-human interleukin-1 monoklonalt antistof	Behandling af Behcets sygdom
Dutch	Recombinant gehumaniseerd antihumaan interleukine-1 beta monoklonaal antilichaam	Behandeling van Ziekte van Behçet
Estonian	Rekombinantne humaniseeritud inimese interleukin-1 beeta vastane monoklonaalne antikeha	Behceti tõve ravi
Finnish	Beetainterleukiini-1:n rekombinantti, humanisoitu, anti-humaani, monoklonaalinen vasta-aine	Behcetin taudin hoito
French	Anticorps monoclonal recombinant humanisé anti-interleukine-1 bêta humaine	Traitement de la maladie de Behçet
German	Rekombinanter humanisierter monoklonaler Antikörper gegen humanes Interleukin-1 beta	Behandlung der Behçet Krankheit
Greek	Ανασυνδυασμένο, ανθρωποποιημένο, αντι-ανθρώπειο μονοκλωνικό αντίσωμα ιντερλευκίνης-1 βήτα	Θεραπευτική αγωγή της νόσου Behçet
Hungarian	Rekombináns humanizált anti-humán interleukin-1 beta monoclonális antitest	Behcet kór kezelése
Italian	Anticorpo monoclonale ricombinante umanizzato diretto contro l'interleuchina-1 beta umana	Trattamento della malattia di Behçet
Latvian	Rekombinanta humanizēta monoklonālā antiviela pret cilvēka interleikīnu-1 beta	Behčeta slimības ārstēšana
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnas prieš žmogaus interleukiną-1 beta	Behčeto ligos gydymas
Maltese	Antikorp monoklonali umanizzat rikombinanti għall-interleukin-1 beta anti-uman	Kura tal-marda ta' Behçet
Polish	Rekombinowane, humanizowane przeciwciało monoklonalne przeciw ludzkiej interleukinie-1 beta	Leczenie choroby Behçeta
Portuguese	Anticorpo monoclonal humanizado, recombinante, anti- interleucina-1 beta humana	Tratamento do doença de Behçet
Romanian	Anticorp monoclonal recombinant umanizat anti-interleukina-1 beta umană	Tratamentul bolii Behçet

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Rekombinantná humanizovaná monoklonálna protilátka proti humánnemu interleukínu-1 beta	Liečba Behcetovej choroby
Slovenian	Rekombinantna humanizirana protičloveška interlevkin 1 beta monoklonalna protitelesa	Zdravljenje Behçet-ove bolezni
Spanish	Anticuerpo monoclonal humanizado recombinante contra la interleukina 1-beta humana	Tratamiento de la enfermedad de Behçet
Swedish	Rekombinant humaniserad anti-human interleukin-1 beta monoklonal antikropp	Behandling av Behcets sjukdom
Norwegian	Rekombinant humanisert anti-humant interleukin-1 beta monoklonalt antistoff	Behandling av Behçets sykdom
Icelandic	Raðbrigða manna and-mennskt interleukin-1-beta einstofna mótefni	Meðferð við Behcets sjúkdómi