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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human residue 41 glutamic acid to glutamine variant of interferon-alfa-2b for the treatment of Behçet's disease

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in March 2011 on request of the sponsor.

On 19 January 2009, orphan designation (EU/3/08/606) was granted by the European Commission to Creabilis Therapeutics S.p.A., Italy, for recombinant human residue 41 glutamic acid to glutamine variant of interferon-alfa-2b for the treatment of Behçet's disease.

What is Behçet's disease?

Behçet's disease is an autoimmune disease (a disease in which the immune system, the body's natural defence, attacks parts of the body). In Behçet's disease, the immune system attacks blood vessels throughout the body. The exact cause of the disease is unknown. As a result of the damage to the blood vessels, almost all patients develop signs such as painful sores in the mouth called aphthous ulcers. They can also develop sores on the genitals, inflammation inside the eye and skin problems. The inflammation inside the eye can affect the uvea (the middle part of the eye), the iris or the retina (the light-sensitive surface at the back of the eye) and can lead to blurred vision, pain, and redness. Behçet's disease is a debilitating and long-lasting disease because of the sores in the mouth and on the genitals and inflammation of the blood vessels throughout the body.

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 below the threshold for orphan designation which is 5 in 10,000 people, and is equivalent to a total of around 50,000 people. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

[*]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 27), Norway, Iceland and Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

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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 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 human residue 41 glutamic acid to glutamine variant of interferon-alfa-2b might be of significant benefit for the patients because it may improve the treatment of ocular symptoms. 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?

Recombinant human residue 41 glutamic acid to glutamine variant of interferon-alfa-2b is a modified type of a type of interferon. Interferons are natural substances produced by the body to help it fight against attacks such as infections caused by viruses. Several types of interferon are used as medicines to treat diseases such as cancer and hepatitis C. Interferons are also used in some diseases to reduce the activity of the immune system, including Behçet's disease although they are not authorised for use in this disease.

This medicine is a modified form of interferon-alfa-2b, in which one of the amino acids that make up the protein has been exchanged for another. This modification is expected to make the medicine more resistant to breakdown in the body, so that lower doses can be given to the patient.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, one clinical trial had been performed in healthy volunteers.

At the time of submission, recombinant human residue 41 glutamic acid to glutamine variant of interferon-alfa-2b was not authorised anywhere in the world 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 5 November 2008 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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Recombinant human residue 41 glutamic acid to glutamine variant of Interferon-alfa-2b	Treatment of Behçet's disease
Bulgarian	Рекомбинантен човешки остатък 41 глутаминова киселина от глутаминова разновидност на Interferon-alfa-2b	Лечение на Болест на Бехчет
Czech	Lidský Rekombinant rezidua 41 kyselina glutamová na glutaminovou variantu interferonu-alfa-2b	Léčba Behcetova choroba
Danish	Rekombinant human residue 41-glutaminsyre til glutaminvariant af interferon alfa-2b	Behandling af Behcets sygdom
Dutch	Humaan recombinant variant van interferon-alfa-2b, waarbij glutaminezuurresidu op positie 41 is vervangen door glutamine	Behandeling van Ziekte van Behçet
Estonian	Interferoon alfa-2B glutamiini variandi vastane inimese rekombinantne glutamiinhappe 41 jääk	Behçeti tõve ravi
Finnish	Rekombinantti humaani glutamiinihappo-41-tähteen glutamiinivariantti interferoni-alfa-2b:stä	Behcetin taudin hoito
French	Variant recombinant humain de l'interféron-alpha-2b substituant le résidu 41d'acide glutamique par la glutamine	Traitement de la maladie de Behçet
German	Rekombinante Variante von Interferon-alpha-2b , wobei der Restbestand von menschlichem Gluatamat an Position 41 von Glutamin ersetzt wird.	Behandlung der Behçet Krankheit
Greek	Ανασυνδυασμένη παραλλαγή της ανθρώπινης ιντερφερόνης-άλφα-2b που προέρχεται από αντικατάσταση του καταλοίπου γλουταμινικού οξέος στη θέση 41 από γλουταμίνη	Θεραπευτική αγωγή Νόσος Behçet
Hungarian	Rekombináns humán, a 41-es pozícióban lévő glutaminsavat glutaminra cserélt interferon-alfa-2b variáns	Behcet kór kezelése
Italian	Variante di interferone-alfa-2b umano ricombinante con mutazione al residuo 41 da acido glutammico a glutammina	Trattamento della malattia di Behçet
Latvian	Rekombinants interferona alfa 2b variants, kur cilvēka glutamīnskābes atlikums 41. pozīcijā aizstāts ar glutamīnu	Behčeta slimības ārstēšana
Lithuanian	Rekombinantinė žmogaus 41 glutamo rūgštis prieš –interferono-alfa-2b glutamino variantą	Behčeto ligos gydymas
Maltese	Varjant ta' l-interferon-alfa-2b uman rikombinanti b'residwu ta' *glutamic acid* fil-pożizzjoni 41 mibdul ma' *glutamine*	Kura tal-marda ta' Behçet

[1] At the time of designation

Language	Active Ingredient	Indication
Polish	Rekombinowana odmiana ludzkiego interferonu ze zmianą kwasu glutaminowego na glutaminę w pozycji 41	Leczenie choroby Behçeta
Portuguese	Variante do interferão-alfa-2b recombinante humano, no qual o resíduo de ácido glutâmico em posição 41 foi substituído pela glutamina	Tratamento do doença de Behçet
Romanian	Variantă a interferonului alfa 2b recombinant uman la care restul de acid glutamic din poziţia 41 este înlocuit cu glutamină	Tratamentul bolii Behçet
Slovak	Rekombinant ľudských zostatkov 41 kyseliny glutámovej na glutamínový variant interferónu-alfa-2b	Liečba Behcetovej choroby
Slovenian	Rekombinantni humani interferon-alfa-2b, ki ima kot aminokislinsko skupino 41 namesto glutaminske kisline glutamin	Zdravljenje Behçet-ove bolezni
Spanish	Variante del Interferón alfa-2b humano recombinante en el que el residuo de ácido glutámico en posición 41 ha sido sustituído por glutamina	Tratamiento de la enfermedad de Behçet
Swedish	Rekombinant human residuum 41 glutaminsyra till glutamin variant av interferon-alfa-2b	Behandling av Behcets sjukdom
Norwegian	Rekombinant human reside-41-glutaminsyre til glutaminvariant av interferon-alfa-2b	Behandling av Behçets sykdom
Icelandic	Raðbrigða afbrigði af interferóni-alfa-2b úr mönnum þar sem glútamínsýru hefur verið skipt út fyrir glútamín við leif 41	Meðferð við Behcets sjúkdómi