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EMA/COMP/647258/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised IgG1 monoclonal antibody against human eotaxin-2 for the treatment of systemic sclerosis

On 19 November 2014, orphan designation (EU/3/14/1371) was granted by the European Commission to CBR Biotech Strategies GmbH, Germany, for humanised IgG1 monoclonal antibody against human eotaxin-2 for the treatment of systemic sclerosis.

What is systemic sclerosis?

Systemic sclerosis is a complex disease in which the immune system (the body's natural defences) is overactive, causing inflammation and excess production of various proteins, particularly collagen. The reason why the immune system is overactive is not known. Collagen is an important component of connective tissue (the tissue that supports the skin and internal organs).

The overproduction of collagen leads to the abnormal growth of connective tissue, causing the skin to become thick and hard. It can also damage the blood vessel walls of the internal organs, such as the heart, lungs and kidneys. This makes it more difficult for the blood to move through the vessels, causing tissue damage, circulation problems and high blood pressure.

Systemic sclerosis is a long-lasting, debilitating disease and may be life threatening because of its possible effects on the gut, heart, lungs and kidneys.

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

At the time of designation, systemic sclerosis affected approximately 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 64,000 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).

*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).

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What treatments are available?

At the time of designation, there were no treatments for systemic sclerosis that could stop the build-up of collagen. Treatments authorised in the EU were aimed at relieving the symptoms of the disease and limiting the damage it causes. Several medicines were used to reduce inflammation and circulation problems. Bosentan was authorised in the EU specifically to treat patients with systemic sclerosis in whom poor blood circulation caused by the disease has led to the development of 'digital ulcers' (sores on the fingers and toes).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with systemic sclerosis because studies in experimental models show that the medicine may reduce the build-up of collagen. 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?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a protein called eotaxin-2. Eotaxin-2 is involved in the inflammation process and in the build-up of collagen seen in systemic sclerosis. By attaching and blocking eotaxin-2, this medicine is expected to reduce inflammation and the accumulation of collagen, thereby relieving the symptoms of systemic sclerosis.

What is the stage of development of this medicine?

The effects of humanised IgG1 monoclonal antibody against human eotaxin-2 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with systemic sclerosis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for systemic sclerosis 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 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

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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	Humanised IgG1 monoclonal antibody against human eotaxin-2	Treatment of systemic sclerosis
Bulgarian	Хуманизирано IgG1 моноклонално антитяло срещу човешки еотаксин-2	Лечение на системна склероза
Croatian	Humanizirano IgG1 monoklonsko protutijelo protiv ljudskog eotaksina-2	Liječenje sistemske skleroze
Czech	Humanizovaná IgG1 monoklonální protilátka ki lidskému eotaxinu-2	Léčba systémové sklerodermie
Danish	Humaniseret IgG1 monoklonalt antistof mod humant eotaxin-2	Behandling af systemisk sklerose
Dutch	Gehumaniseerd IgG1 monoklonaal antilichaam tegen humaan eotaxine-2	Behandeling van systeem sclerose
Estonian	Humaniseeritud IgG1 monoklonaalne antikeha inimese eotaksiini 2 vastu	Süsteemse sklerodermia ravi
Finnish	Humanisoitua IgG1 monoklonaalinen vasta-aine ihmisen eotaksiini 2:a vastaan	Systeemisen skleroosin hoito
French	Anticorps monoclonal humanisé IgG1 dirigé contre l'éotaxine-2 humaine	Traitement de la sclérose systémique
German	Humanisierter monoklonaler IgG1-Antikörper gegen humanes Eotaxin-2	Behandlung der systemischen Sklerose
Greek	Ανθρωποποιημένο IgG1 μονοκλωνικό αντίσωμα έναντι της ανθρώπινης εοταξίνης-2	Θεραπεία της συστηματικής σκλήρυνσης
Hungarian	Humán eotaxin 2 ellenes humanizált IgG1 monoklonális antitest	Szisztémás scleroderma kezelése
Italian	Anticorpo monoclonale IgG1 umanizzato diretto contro l'eotassina-2 umana	Trattamento della sclerosi sistemica
Latvian	Humanizēta IgG1 monoklonāla antiviela pret cilvēka eotaksīnu-2	Sistēmiskas sklerozes ārstēšana
Lithuanian	Žmogaus IgG1 monokloninis antikūnas prieš žmogaus eotaksiną-2	Sisteminės sklerozės gydymas
Maltese	Antikorp monoklonali IgG1 umanizzat kontra eotaxin-2 uman	Kura tas-sklerosi sistemika
Polish	Humanizowane przeciwciało monoklonalne IgG1 przeciw ludzkiej eotaksynie-2	Leczenie twardziny narządowej
Portuguese	Anticorpo monoclonal humanizado IgG1 contra eotaxina-2	Tratamento da esclerose sistémica
Romanian	Anticorp monoclonal umanizat IgG1 împotriva eotaxinei-2 umane	Tratamentul sclerozei sistemice
Slovak	Humanizovaná IgG1 monoklonálna protilátka proti ľudskému eotaxínu-2	Liečba systémovej sklerózy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humanizirano monoklonsko protitelo IgG1 proti humanemu eotaksinu-2	Zdravljenje sistemske skleroze
Spanish	Anticuerpo monoclonal IgG1 humanizado contra eotaxina-2 humana	Tratamiento de la esclerosis sistémica
Swedish	Humaniserad IgG1 monoklonal antikropp mot humant eotaxin-2	Behandling av systemisk skleros
Norwegian	Humanisert IgG1 monoklonalt antistoff mot human eotaksin-2	Behandling av systemisk sklerose
Icelandic	Mannaðlagað IgG1 einstofna mótefni gegn manna eotaxíni-2	Meðferð við dreifðum herslismeinum