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Public summary of opinion on orphan designation

Humanised Fc-engineered monoclonal antibody against CD19 for the treatment of IgG4-related disease

On 17 January 2018, orphan designation (EU/3/17/1962) was granted by the European Commission to MWB Consulting Ltd, United Kingdom, for humanised Fc-engineered monoclonal antibody against CD19 (also known as XmAb5871) for the treatment of IgG4-related disease.

What is IgG4-related disease?

IgG4-related disease is a group of disorders caused by cells of the immune system (the body's natural defences) entering the patient's own tissues and leading to inflammation, swelling and scarring (fibrosis).

The disease comes and goes, and it can affect the pancreas, liver, kidneys, lungs, tear and salivary glands, eyes or any other organ, causing a wide range of symptoms. Patients with the disease often have high levels of an antibody called IgG4 in their blood, and IgG4-producing blood cells have also entered their tissues.

IgG4-related disease is debilitating because of symptoms of organ swelling such as weight loss, fevers, tiredness and pain. It is life threatening because affected organs become damaged and may stop working.

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

At the time of designation, IgG4-related disease affected less than 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 165,000 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, IgG4-related disease was mainly treated with steroid medicines called glucocorticoids which reduce inflammation.

*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 515,700,000 (Eurostat 2017).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with IgG4-related disease because data show that the medicine may help patients when glucocorticoids do not work well enough. 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?

The medicine is a monoclonal antibody (a type of protein) designed to recognise and attach to B cells, which are white blood cells that make antibodies, including IgG4. When the medicine attaches to B cells, it is expected to reduce their activity and so improve symptoms of the disease.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with IgG4-related disease were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for IgG4-related disease. Orphan designation of the medicine had been granted in the United States for IgG4-related disease.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 December 2017 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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 Fc-engineered monoclonal antibody against CD19	Treatment of IgG4-related disease
Bulgarian	Хуманизирано Fc разработено моноклонално антитяло срещу CD19	Лечение на IgG4-свързано заболяване
Croatian	Humanizirano Fc monoklonsko protutijelo protiv CD19	Liječenje bolesti povezane s IgG4
Czech	Humanizovaná upravená monoklonální protilátka Fc proti CD19	Léčba IgG4 odvozených nemocí
Danish	Humaniseret Fc-fremstillet monoklonalt antistof mod CD19	Behandling af IgG4-relateret sygdom
Dutch	Gehumaniseerd Fc ontworpen monoklonaal antilichaam tegen CD19	Behandeling van IgG4-gerelateerde aandoening
Estonian	CD19-vastane Fc-modifikatsiooniga humaniseeritud monoklonaalne antikeha	IgG4 seotud haiguste ravi
Finnish	Humanisoitu monoklonaalinen CD19-vasta-aine muunnellulla Fc-osalla	IgG4:ään liittyvän sairauden hoito
French	Anticorps monoclonal humanisé Fc anti-CD19 produit par génie génétique	Traitement des maladies associéee aux IgG4
German	Humanisierter Fc-veränderter monoklonaler Antikörper gegen CD19	Behandlung der IgG4-assoziierten Erkrankung
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα έναντι του CD19, με τροποιημένο τμήμα Fc	Θεραπεία της IgG4-σχετιζόμενης νόσου
Hungarian	Humanizált, Fc-optimalizált, CD19 elleni monoklonális antitest	IgG4-el kapcsolatos betegség kezelése
Italian	Anticorpo monoclonale umanizzato ingegnerizzato Fc, diretto contro CD19	Trattamento della malattia IgG4-correlata
Latvian	Humanizēta Fc inženierēta monoklonāla antiviela pret CD19	Ar IgG4 saistītās slimības ārstēšana
Lithuanian	Sukonstruotas humanizuotas Fc monokloninis antikūnas prieš CD19	Su IgG4 susijusios ligos gydymas
Maltese	Antikorp monoklonali Fc umanizzat maħdum kontra CD19	Kura ta' mard relatat mal-IgG4
Polish	Humanizowane przeciwciało monoklonalne anty-CD19 ze zmodyfikowaną domeną Fc	Leczenie choroby związanej z IgG4
Portuguese	Anticorpo monoclonal Fc humanizado anti CD19	Tratamento da doença associada a IgG4
Romanian	Anticorp monoclonal umanizat Fc anti-CD19 produs prin inginerie genetică	Tratamentul bolii asociate cu IgG4
Slovak	Humanizovaná Fc upravená monoklonálna protilátka proti CD19	Liečba IgG4 súvisiacej choroby

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humanizirano monoklonalno protitelo proti receptorjem CD19 Fc	Zdravljenje z IgG4 povezanih bolezni
Spanish	Anticuerpo monoclonal Fc humanizado contra CD19	Tratmiento de la enfermedad relacionada con IgG4
Swedish	Humaniserad Fc-framställd monoklonal antikropp riktad mot CD19	Behandling av IgG4-associerad sjukdom
Norwegian	Humanisert Fc konstruert monoklonalt antistoff mot CD19	Behandling av IgG4-relatert sykdom
Icelandic	Mannaaðlagað Fc-útbúið einstofna mótefni gegn CD19	Meðferð við IgG4-tengdum sjúkdómi