31 March 2016
EMA/COMP/68353/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised IgG4 monoclonal antibody against total complement component 1, subcomponent s for the treatment of autoimmune haemolytic anaemia

On 17 February 2016, orphan designation (EU/3/16/1609) was granted by the European Commission to Assign Group Development UK Ltd, United Kingdom, for humanised IgG4 monoclonal antibody against total complement component 1, subcomponent s for the treatment of autoimmune haemolytic anaemia.

What is autoimmune haemolytic anaemia?

Autoimmune haemolytic anaemia is a condition that occurs when the body recognises red blood cells as foreign and attacks them, eventually destroying them. This leads to low red blood cell counts and low levels of haemoglobin, which in turn causes symptoms such as tiredness and inability to exercise.

Autoimmune haemolytic anaemia is a long-term debilitating condition which may cause blood clots in the veins or arteries and infections, and may require transfusions of red blood cells.

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

At the time of designation, autoimmune haemolytic anaemia affected less than 2.3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 118,000 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, corticosteroids (medicines that suppress the activity of the immune system) were authorised in the EU for the treatment of autoimmune haemolytic anaemia. Red blood cell transfusion and splenectomy (removing the spleen) was also performed in some patients.

[*]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 513,700,000 (Eurostat 2016).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with autoimmune haemolytic anaemia. This is because early studies in patients show that the medicine prevents breaking of the red blood cells and improves haemoglobin levels, which may have benefits in the short and long term when compared with existing methods. 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 has been designed to attach to a protein called 'complement component 1, subcomponent s' (C1s), which is part of the body's defence system called 'complement'. In autoimmune haemolytic anaemia, patients' red blood cells often have proteins on their surface that allow complement to attack the cells. This results in complement destroying the red blood cells. By blocking C1s the medicine is expected to prevent the complement system from attacking the cells, thus reducing their destruction and improving the 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 autoimmune haemolytic anaemia were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for autoimmune haemolytic anaemia 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 21 January 2016 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:

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 IgG4 monoclonal antibody against total complement component 1, subcomponent s	Treatment of autoimmune haemolytic anaemia
Bulgarian	Хуманизирано IgG4 моноклонално антитяло срещу 1 компонентата на комплемента, субкомпонент s	Лечение на автоимунна хемолитична анемия
Croatian	Humanizirano IgG4 monoklonsko protutijelo na komponentu ukupnog komplementa 1, podkomponenta s	Liječenje autoimune hemolitičke anemije
Czech	Humanizovaná monoklonální protilátka IgG4 vůči celkové složce komplementu 1, podjednotce s	Léčba autoimunitní hemolytické anémie
Danish	Humaniseret monoklonalt IgG4-antistof mod total komplement komponent 1, subkomponent s	Behandling af autoimmun hæmolytisk anæmi
Dutch	Gehumaniseerd IgG4 monoklonaal antilichaam tegen totaal complement component 1, subcomponent s	Behandeling van auto-immune hemolytische anemie
Estonian	Kogu komplemendi komponendi 1 s-alamkomponendi vastane inimese monoklonaalne IgG4 antikeha	Autoimmuunse hemolüütilise aneemia ravi
Finnish	Humanisoitu monoklonaalinen IgG4-luokan vasta-aine kokonais komplementin komponenttia 1, osatekijää s vastaan	Autoimmuunin hemolyyttisen anemian hoito
French	Anticorps monoclonal humanisé de classe IgG4 dirigé contre le sous-composant s du composant total 1 du complément	Traitement de l'anémie hémolytique auto-immune
German	Humanisierter monoklonaler IgG4-Antikörper gegen die Gesamtkomplementkomponente 1, Untereinheit s	Behandlung der autoimmunhämolytischen Anämie
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα IgG4 εναντίον της υποσυνιστώσας s της συνιστώσας 1 του ολικού συμπληρώματος	Θεραπεία της αυτοάνοσης αιμολυτικής αναιμίας
Hungarian	Humanizált IgG4 monoklonális antitest a komplement teljes 1. komponensének s alkomponense ellen	Autoimmun hemolitikus anémia kezelésére
Italian	Anticorpo monoclonale umanizzato IgG4 contro il componente del complemento 1 totale, sottocomponente s	Trattamento dell'anemia emolitica autoimmune
Latvian	Cilvēciskotas IgG4 monoklonālās antivielas pret kopējās komplementa sistēmas 1. komponenta s apakškomponentu	Autoimūnas hemolītiskās anēmijas ārstēšanai
Lithuanian	Žmogaus IgG4 monokloninis antikūnas prieš visą komplemento komponentą 1, subkomponentą s	Autoimuninės hemolizinės anemijos gydymas

[1] At the time of designation

Language	Active ingredient	Indication
Maltese	Antikorp monoklonali IgG4 umanizzat direct kontra s-subkomponent s tal-komponent 1 ta' komplement totali	Kura tal-anemija emolitika awtoimmuna
Polish	Humanizowane przeciwciało monoklonalne IgG4 skierowane przeciwko całkowitej składowej dopełniacza 1, podjednostce s	Leczenie autoimmunologicznej niedokrwistości hemolitycznej
Portuguese	Anticorpo monoclonal de tipo IgG4 humanizado contra o componente 1 total do complemento, subcomponente s	Tratamento da anemia hemolítica auto-imune
Romanian	Anticorp monoclonal IgG4 umanizat împotriva componentei 1 a complementului total, subcomponenta s	Tratamentul anemiei hemolitice autoimune
Slovak	Humanizovaná monoklonálna IgG4 protilátka proti celej zložke komplementu 1, subkomponent s	Liečba autoimúnnej hemolytickej anémie
Slovenian	Humanizirano monoklonsko protitelo IgG4 proti kompletnemu komplementu 1, podenota s	Zdravljenje avtoimune hemolitične anemije
Spanish	Anticuerpo monoclonal humanizado IgG4 dirigido contra el subcomponente s del componente 1 del complemento	Tratamiento de la anemia hemolítica autoinmune
Swedish	Humaniserad monoklonal IgG4-antikropp mot total komplementkomponent 1, s-subkomponent	Behandling av autoimmun hemolytisk anemi
Norwegian	Humanisert monoklonalt IgG4-antistoff mot total komplementkomponent 1, underkomponent s	Behandling av autoimmun hemolytisk anemi
Icelandic	Mannaðlagað einstofna IgG4 mótefni gegn heildar komplementþætti 1, undirþætti s	Meðferð við sjálfsónæmis blóðlýsublóðleysi