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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human minibody against complement component C5 for the the treatment of atypical haemolytic uraemic syndrome (aHUS) associated with an inherited abnormality of the complement system

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Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 22 September 2008, orphan designation (EU/3/08/571) was granted by the European Commission to ADIENNE S.r.l., Italy, for recombinant human minibody against complement component C5 for the treatment of atypical haemolytic uraemic syndrome (aHUS) associated with an inherited abnormality of the complement system.

In January 2014, ADIENNE S.r.l. changed name to ADIENNE S.r.l.S.U.

What is atypical haemolytic uraemic syndrome (aHUS) associated with an inherited abnormality of the complement system?

Haemolytic uraemic syndrome (HUS) is a disorder characterised by haemolysis (destruction of red blood cells), thrombocytopenia (a decrease in the number of platelets, components that help the blood to clot), problems with blood clotting and kidney failure. In contrast to most types of HUS, which occur in children after an infection with gut bacteria, atypical HUS (aHUS) affects both children and adults, and is not caused by an infection. More than half of the cases of aHUS are thought to be caused by inherited (inborn) abnormalities of the complement system, a group of proteins in the blood that help the immune system (the body's natural defence system) to fight infections. In patients with aHUS, the complement system is overactive and damages the cells lining the blood vessels.

aHUS can be long term, or can keep coming back (relapsing). The condition is long lasting and life-threatening because of the risk of kidney failure.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

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

At the time of designation aHUS associated with an inherited abnormality of the complement system affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for orphan drug designation, only supportive treatments for aHUS associated with an inherited abnormality of the complement system were available. Transfusions of red blood cells and platelets were given as needed. Dialysis (a blood clearance technique) may be needed if the disease progresses to kidney failure. Some patients may receive infusion of plasma or a therapy called plasma exchange although its role in this condition is not completely clear. Some patients may need a kidney transplant.

How is this medicine expected to work?

Recombinant human minibody against complement component C5 is an antibody that has been designed to attach itself to a protein called component C5 of the complement system. In aHUS, the complement component C5 plays an important role for the activation of the complement system, ultimately leading to the damage of the cells lining the blood vessels. By binding to the complement component C5, the antibody prevents the activation of the complement system. This is expected to reduce the damage to the cells lining the blood vessels and the symptoms of the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of recombinant human minibody against complement component C5 was ongoing in experimental models. No clinical trials in patients with aHUS associated with an inherited abnormality of the complement system had been started.

At the time of submission, recombinant human minibody against complement component C5 was not authorised anywhere in the world for the treatment of aHUS associated with an inherited abnormality of the complement system, or designated as 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 July 2008 recommending the granting of this designation.

*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.
At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

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:

ADIENNE S.r.l.S.U.
Via Galileo Galilei, 19
20867 Caponago (MB)
Italy
Tel.: +39 02 4070 0445
Telefax: +39 02 9574 5179
E-mail: adienne@adienne.com

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 minibody against complement component C5	Treatment of atypical Haemolytic Uraemic Syndrome (aHUS) associated with an inherited abnormality of the complement system
Bulgarian	Рекомбинантно човешко минитяло срещу C5 компонента на комплемента	Лечение на атипичен хемолитичен уремичен синдром (аХУС) свързан с вродена аномалия на системата на комплемента
Czech	Rekombinantní lidská miniprotilátka proti C5 složce komplementu	Léčba atypického hemolyticko-uremického syndromu (aHUS) spojeného s vrozenou abnormalitou v komplementovém systému
Danish	Rekombinant humant antistoffragment mod komplement komponent C5	Behandling af atypisk hæmolytisk uræmisk syndrom (aHUS), der er forbundet med en nedarvet abnormalitet af komplementsystemet
Dutch	Recombinant humaan minilichaam gericht tegen complement component C5	Behandeling van het atypisch hemolytisch-uremisch syndroom (aHUS) geassocieerd met erfelijke aandoening van het complementsysteem
Estonian	Komplemendi osa C5 vastane rekombinantne inimese minikeha.	Päriliku komplemendisüsteemi puudulikkusega seotud atüüpilise hemolüütilis-ureemilise sündroomi (aHUS) ravi
Finnish	Rekombinantti humaani minivasta-aine komplementti komponentti C5:a vastaan	Komplementtijärjestelmän perinnölliseen poikkeavuuteen liittyvän epätyypillisen hemolyyttis-ureemisen oireyhtymän (aHUS) hoito
French	Minibody humain recombinant dirigé contre l'élément C5 du complément	Traitement du Syndrome Hémolytique Urémique atypique (SHUa) associé à une anomalie héréditaire du système du complément
German	Humaner rekombinanter Minibody gegen den Komplementfaktor C5	Behandlung des atypischen Hämolytisch-Urämischen Syndroms (aHUS), das in Zusammenhang mit einer erblichen Anomalie des Komplementsystems auftritt
Greek	Ανασυνδιασμενο ανθρώπινο μινίσωμα ενάντια στον παράγοντα C5 του συμπληρώματος	Θεραπεία του Άτυπου Αιμολυτικού Ουραιμικού Σύνδρόμου (aHUS) που συνδέεται με κληρονομική ανωμαλία του συστήματος του συμπληρώματος
Hungarian	C5 komplement-komponens ellenes rekombináns humán minitest	Örökletes komplementrendszer abnormalitással összefüggő atípusos haemolyticus uraemiás szindróma
Italian	Minianticorpo umano ricombinante diretto contro la componente C5 del complemento	Trattamento della sindrome uremico-emolitica (SUE) atipica associata ad un'anomalia ereditaria del sistema del complemento

[1] At the time of designation

Language	Active Ingredient	Indication
Latvian	Rekombinantas cilvēka minivielas pret komplementa C5 komponenti	Ar iedzimtu komplementa sistēmas anomāliju saistīta atipiska hemolītiska urēmiskā sindroma (aHUS) ārstēšana
Lithuanian	Rekombinantinis žmogaus minikūnelis prieš komplemento komponentą C5	Netipinio hemolizinio - ureminio sindromo (nHUS), susijusio su paveldimu komplemento sistemos pakitimu, gydymas
Maltese	Minikorp uman rikombinanti kontra l-komponent Ċ5 tal-komplement	Kura tas-sindrome uremiku emolitiku atipiku (aHUS) marbut ma' anomalija ereditarja tas-sistema tal-komplement
Polish	Rekombinowane ludzkie miniciało przeciwko składowej dopełniacza C5	Leczenie atypowego hemolitycznego zespołu mocznicowego (aHUS) w przebiegu dziedzicznej nieprawidłowości układu dopełniacza
Portuguese	Minicorpo recombinante de origem humana contra o componente C5 do complemento	Tratamento do Síndrome Hemolítico Urémico atípico (SHUa) associado a uma anomalia hereditária do sistema de complemento
Romanian	Minianticorp uman recombinant anti componenta C5 a complementului	Tratamentul sindromului hemolitic-uremic atipic asociat cu o anomalie ereditară a sistemului complementului
Slovak	Rekombinantná ľudská miniprotilátka proti zložke komplementu C5	Liečba atypického hemolyticko-uremického syndrómu (aHUS) spojeného s hereditárnou abnormalitou komplementového systému
Slovenian	Rekombinantno humano minitelo proti sestavini C5 komplementa	Zdravljenje atipičnega hemolitičnega uremičnega sindroma (aHUS), povezanega z dedno nepravilnostjo komplementa
Spanish	Minianticuerpo recombinante humano contra el factor C5 del complemento	Tratamiento del síndrome urémico hemolítico atípico asociado a una anomalía hereditaria del sistema del complemento
Swedish	Recombinant human minikropp riktat mot komplement komponenten C5	Behandling av atypiskt hemolytiskt uremiskt syndrom (aHUS) kopplat till en ärftlig felaktighet i komplementsystemet
Norwegian	Rekombinant humant antistoffragment mot komplementfaktor C5	Behandling av atypisk hemolytisk uremisk syndrom (atypisk HUS) forbundet med arvelig anomali i komplementsystemet
Icelandic	Raðbrigða manna minibody gegn komplementþætti C5	Meðferð við ódæmigerðu blóðlýsu-þvageitrunarheilkenni (aHUS) tengdu arfgengum afbrigðileika komplementkerfisins