13 March 2015
EMA/COMP/364582/2009 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Eculizumab for the treatment of atypical haemolytic uraemic syndrome (aHUS)

First publication	7 September 2009
Rev.1: administrative update	3 May 2011
Rev.2: information about Marketing Authorisation	25 July 2013
Rev.3: sponsor's change of address	13 March 2015

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 24 July 2009, orphan designation (EU/3/09/653) was granted by the European Commission to Alexion Europe SAS, France, for eculizumab for the treatment of atypical haemolytic uraemic syndrome (aHUS).

What is atypical haemolytic uraemic syndrome (aHUS)?

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).

aHUS is a long-lasting and life-threatening disease because of the risk of kidney failure.

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

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

At the time of designation, aHUS 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 designation, only supportive treatments for patients with aHUS were available. Transfusions of red blood cells and platelets are 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?

Eculizumab is a medicine that is already authorised in the EU for the treatment of paroxysmal nocturnal haemoglobinuria (PNH), a rare, life-threatening genetic disease that causes the red blood cells to be broken down too quickly. Eculizumab is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific structure (called an antigen) that is found in the body. Eculizumab has been designed to attach to a protein of the complement system called C5.

By blocking the C5 complement protein, eculizumab is expected to block the activation of the complement system in patients with aHUS, stopping it from attacking the cells lining the blood vessels. This may reduce their destruction and improve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of eculizumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with aHUS were ongoing.

At the time of submission, eculizumab was not authorised anywhere in the EU for aHUS or designated as orphan medicinal product elsewhere for this condition. Orphan designation of eculizumab had been granted in the United States of America for aHUS.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 June 2009 recommending the granting of this designation.

Update: eculizumab (Soliris) has been authorised in the EU since 24 November 2011 for the treatment of patients with atypical haemolytic uraemic syndrome (aHUS).

More information on Soliris can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

*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 504,800,000 (Eurostat 2009).

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:

Alexion Europe SAS
1-15, avenue Edouard Belin
92500 Rueil-Malmaison
France
Tel. +33 1 47 32 36 21
Fax +33 1 47 10 24 46
E-mail: medicalinformation.europe@alxn.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	Eculizumab	Treatment of atypical haemolytic uremic syndrome
Bulgarian	Екулизумаб	Лечение на атипичен хемолитичен уремичен синдром
Czech	Eculizumab	Léčba atypického hemolyticko-uremického syndromu
Danish	Eculizumab	Behandling af atypisk hæmolytisk uræmisk syndrom
Dutch	Eculizumab	Behandeling van atypisch hemolytisch uremisch syndroom
Estonian	Ekulizumab	Atüüpilise hemolüütilis-ureemilise sündroomi ravi
Finnish	Ekulitsumabi	Epätyypillisen hemolyyttis-ureemisen oireyhtymän hoito
French	Eculizumab	Traitement du syndrome hémolytique urémique atypique
German	Eculizumab	Behandlung des atypischen hämolytisch-urämischen Syndroms
Greek	Εκουλιζουμάβη	Θεραπεία του Άτυπου Αιμολυτικού Ουραιμικού Συνδρόμου
Hungarian	Eculizumab	Atípusos haemolyticus uraemiás szindróma kezelése
Italian	Eculizumab	Trattamento della sindrome uremico-emolitica atipica
Latvian	Ekulizumabs	Atipiska hemolītiska urēmiskā sindroma ārstēšana
Lithuanian	Ekulizumabas	Atipinio hemolizinio - ureminio sindromo gydymas
Maltese	Eculizumab	Kura tas-sindrome uremiku emolitiku atipiku
Polish	Ekulizumab	Leczenie atypowego zespołu hemolityczno-mocznicowego
Portuguese	Eculizumab	Tratamento do Síndrome Hemolítico Urémico atípico
Romanian	Eculizumab	Tratamentul sindromului hemolitic-uremic atipic
Slovak	Ekulizumab	Liečba atypického hemolyticko-uremického syndrómu
Slovenian	Ekulizumab	Zdravljenje atipičnega hemolitičnega uremičnega sindroma
Spanish	Eculizumab	Tratamiento del síndrome urémico hemolítico atípico
Swedish	Eculizumab	Behandling av atypiskt hemolytiskt uremiskt syndrom
Norwegian	Eculizumab	Behandling av atypisk hemolytisk uremisk syndrom
Icelandic	Eculizúmab	Meðferð við ódæmigerðu blóðlýsu-þvageitrunarheilkenni

[1] At the time of designation