13 March 2015
EMA/COMP/1523/2003 Rev.6
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Eculizumab for the treatment of paroxysmal nocturnal haemoglobinuria

First publication	16 March 2005
Rev.1: transfer of sponsorship	22 February 2007
Rev.2: administrative update	6 March 2007
Rev.3: information about Marketing Authorisation	3 July 2007
Rev.4: sponsor's change of address	27 August 2009
Rev.5: administrative update	3 May 2011
Rev.6: sponsor' 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 17 October 2003, orphan designation (EU/3/03/166) was granted by the European Commission to QuadraMed, United Kingdom, for eculizumab for the treatment of paroxysmal nocturnal haemoglobinuria.

The sponsorship was transferred to Alexion Europe SAS, France, in March 2006.

What is Paroxysmal Nocturnal Haemoglobinuria (PNH)?

PNH is a disorder in which red blood cells lack specific proteins on their surface which normally protect them from being destroyed (a process called haemolysis) by the body's normal defence system. PNH is characterised by the presence of brownish urine in the early morning hours. The characteristic colour of urine is due to the presence of products from destroyed red cells. Patients have a low count of red blood cells, and may have blood clots in the large vessels. PNH is a life-threatening condition.

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

At the time of designation, PNH affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 4,000 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?

There were no medicinal products authorised for the condition in the Community at the time of the submission of the orphan drug application. Bone marrow transplantation to replace the defective cells is the only curative therapy available to patients. This treatment is available to only a small proportion of patients since a suitable donor is required. Furthermore, transplantation maybe associated with substantial risks. Other methods such as blood transfusions and treatment to prevent clotting with blood thinning compounds can improve the symptoms in a small percentage of patients.

How is this medicine expected to work?

In the human body there is a group of proteins called complement system, which have inflammatory properties and can destroy cells. Usually this system is activated as a defensive mechanism of the body. Proteins from the complement system are named with a "C" letter and a number. Normally each of these proteins can activate by cleavage other proteins. Eculizumab is an antibody that binds to human C5, a complement protein with capacity to start an inflammatory reaction in humans. After several steps this can result on the destruction of blood cells. It is expected that the binding of eculizumab to C5 will inhibit its cleavage into pro-inflammatory components, and therefore the subsequent cell lysis.

What is the stage of development of this medicine?

The effects of eculizumab were evaluated in experimental models.

At the time of the orphan drug designation clinical trials were ongoing.

At the time of submission of the orphan designation application, eculizumab had not been marketed anywhere worldwide for PNH 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 10 September 2003 recommending the granting of this designation.

Update: Eculizumab (Soliris) has been authorised in the EU since 20 June 2007 for the treatment of patients with paroxysmal nocturnal haemoglobinuria (PNH).

Evidence of clinical benefit of Soliris in the treatment of patients with PNH is limited to patients with history of transfusions.

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.
At the time of designation, this represented a population of 382,800,000 (Eurostat 2003).

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:

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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	Eculizumab	Treatment of paroxysmal nocturnal haemoglobinuria
Czech	Eculizumab	K léčbě paroxysmální noční hemoglobinurie
Danish	Eculizumab	Behandling af paroksysmatisk nocturn hæmoglobinuria
Dutch	Eculizumab	Behandeling van paroxismale nachtelijke hemoglobinurie
Estonian	Ekulizumab	Paroksüsmaalse öise hemoglubinuuria ravi
Finnish	Ekulitsumabi	Paroksysmaalisen nokturnaalisen hemoglobinurian hoito
French	Eculizumab	Traitement de l'hémoglobinurie paroxystique nocturne
German	Eculizumab	Behandlung von paroxysmaler nächtlicher Hämoglobinurie
Greek	Εκουλιζουμάβη	Θεραπεία της παροξυσμικής νυκτερινής αιμοσφαιρινουρίας
Hungarian	Eculizumab	Paroxysmalis nocturnalis haemoglobinuria
Italian	Eculizumab	Trattamento dell'emoglobinuria parossistica notturna
Latvian	Ekulizumabs	Paroksismālas nakts hemoglobinūrijas ārstēšana
Lithuanian	Ekulizumabas	Priepuolinės naktinės hemoglobinurijos gydymas
Polish	Ekulizumab	Leczenie napadowej nocnej hemoglobinurii
Portuguese	Eculizumab	Tratamento da hemoglobinúria paroxística nocturna
Slovak	Ekulizumab	Liečba paroxyzmálnej nočnej hemoglobinúrie
Slovenian	Ekulizumab	Zdravljenje paroksizmalne nočne hemoglobinurije
Spanish	Eculizumab	Tratamiento de la hemoglobinuria paroxística nocturna
Swedish	Eculizumab	Behandling av paroxysmal nattlig hemoglobinuri

[1] At the time of transfer of sponsorship