European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Document Date: London, 12 August 2009
Doc.Ref.: EMEA/COMP/1641/02 Rev.3

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2009 on request of the Sponsor.

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of myristoylated-peptidyl-recombinant human CD59 for the treatment of paroxysmal nocturnal haemoglobinuria

On 11 September 2002, orphan designation (EU/3/02/114) was granted by the European Commission to Adprotech Ltd., United Kingdom, for myristoylated-peptidyl-recombinant human CD59 for the treatment of paroxysmal nocturnal haemoglobinuria.
The sponsorship was transferred to Orexo AB, Sweden, in September 2008.

What is Paroxysmal Nocturnal Haemoglobinuria (PNH)?
PNH is characterised by passing blood in the urine in the early morning hours. Patients have a low count of red blood cells, and may have blood clots in the large vessels. PNH is a life-threatening condition.

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 is equivalent to a total of around 3,800 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. 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?
A number of proteins are known to be lacking from blood cells of patients with PNH. One of these is called CD59. CD59 plays a role in the protection of cells from breakdown by a group of specific substances known as complement. CD59 may also play a role in the prevention of clotting and haemolysis (red cell destruction). The product is expected to act by replacing this missing protein and as a consequence would correct anomalies observed in patients suffering from PNH.

[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition.

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 75 23 70 40
E-mail: orphandrugs@emea.europa.eu http://www.emea.europa.eu

What is the stage of development of this medicine?
The evaluation of the effects of myristoylated-peptidyl-recombinant human CD59 in experimental models is ongoing.

At the time of the orphan drug designation the clinical development had not been initiated.

At the time of submission of the orphan designation application, myristoylated-peptidyl-recombinant human CD59 had not been marketed anywhere worldwide for PNH or designated as orphan medicinal product elsewhere for this condition.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 18 July 2002 a positive opinion recommending the grant of the above-mentioned 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 Community) 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:
Orexo AB
Virdings Allé 32 A
754 50 Uppsala
Sweden
Telephone: +46 18 780 8800
Telefax: +46 18 780 8888