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

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2009 on request of the Sponsor.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 SCR1-3 of human complement receptor type I for the prevention of post transplantation graft dysfunction

On 30 July 2002, orphan designation (EU/3/02/108) was granted by the European Commission to Adprotech Limited, United Kingdom, for myristoylated-peptidyl-recombinant SCR1-3 of human complement receptor type I (APT 070) for the prevention of post transplantation graft dysfunction. The sponsorship was transferred to Orexo AB, Sweden, in September 2008.

What is post transplantation graft dysfunction?
Organ transplant refers to the transfer of organs from one body to another. Once the organ is transplanted, it is called a graft. The transplant requires complex surgical procedures. Organs always suffer during this process, due to different causes. In some cases, the damage is such that the graft function becomes abnormal. This condition is called post transplantation graft dysfunction.
The dysfunction depends on the type of injury. For example, the disruption of blood supply may cause an immediate damage to the organ due to lack of oxygen. In other cases, the patient has an immune reaction against the graft he received. In the long run, the dysfunction can cause graft failure and rejection. Post transplantation graft dysfunction is a long term debilitating and life-threatening condition.

What is the estimated number of patients at risk of developing the condition?
At the time of designation, the number of patients at risk of post transplantation graft dysfunction was estimated to be approximately 0.5 people in 10,000 in the European Union (EU) [*]*. This is equivalent to a total of around 19,000 people, which 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 methods of prevention are available?
There were several medicines that had been authorised to prevent graft dysfunction, and organ rejection in the Community at the time of submission of the application for orphan drug designation. These medicines consist of chemicals and antibodies that inhibit the immune response against the graft. APT 070 may be of potential significant benefit as an additional treatment. This is because it might act differently from available treatments.

How is this medicine expected to work?
APT 070 is circulated through the organ, prior to transplantation. Here it becomes part of the surface of cells where it inhibits a set of proteins called the complement system. These proteins are found in

[*]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.

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the blood and are part of the immune system. Together with antibodies, these proteins cause a reaction against what they recognize as foreign, participating in the organ dysfunction.

What is the stage of development of this medicine?
The effects of APT 070 have been evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials had been initiated.

APT 070 had not been marketed anywhere worldwide for the prevention of post transplantation graft dysfunction or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 27 June 2002 a positive opinion recommending the grant of the above-mentioned designation.

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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.

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