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

Document Date: London, 18 August 2008
Doc.Ref.: EMEA/COMP/12306/03 Rev.1

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF inolimomab for treatment of graft versus host disease

On 5 March 2001, orphan designation (EU/3/01/028) was granted by the European Commission to OPi Orphan Pharma international, France, for inolimomab for the treatment of graft versus host disease.
OPi changed its name to EUSA Pharma SAS in February 2008.

What is graft versus host disease?

The bone marrow is the spongy tissue inside the large bones in the body. The bone marrow makes red blood cells (which carry oxygen and other materials to all tissues of the body), white blood cells (which fight infection), and platelets (which make the blood clot). Bone marrow transplantation (replacing with healthy marrow) is a treatment used against certain diseases of the bone marrow. A frequent complication of bone marrow transplantation is the development of a disease called graft versus host disease (GvHD). This disease involves a reaction between the donor cells and the recipient's native tissues leading to injury of the recipient's tissues. GvHD occurs in acute and chronic form. The organs most commonly affected in acute GvHD are the stomach and the intestines, the skin, and the liver. Chronic GvHD involves a much wider range of tissues than the acute form. The condition is chronically debilitating and life-threatening.

What are the methods of treatment available?

The methods of treatment authorised for GvHD in the Community, at the time of submission of the application for orphan designation, consisted of certain steroid hormones (corticosteroids, a group of chemical substances, which modulate the activity of certain organs and of the immune system) administered at high doses. Other therapies include drugs that inhibit the immune response (immunosuppressants).
Inolimomab might be of potential significant benefit for the treatment of GvHD. This assumption remains to be proven. This will be necessary to maintain the orphan status.

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

Based on the information provided by the sponsor and previous knowledge of the Committee, graft versus host disease was considered to affect approximately 0.13 in 10,000 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 5,000 persons.

* 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. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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

How is this medicinal product expected to act?
Natural antibodies are proteins produced by a specific type of cells in the body, that specifically recognise and attach themselves to certain foreign structures such as proteins found on the surface of cancer cells or bacteria. Inolimomab is an artificially produced antibody that specifically recognises a protein, the CD25 antibody, found on those cells of the immune system that are activated, thereby causing the graft versus host disease. Inolinomab is expected to bind to these cells and to stop their multiplication. Cells, which are not involved in the GvHD immune reaction and, therefore, not activated at the time of the administration of the product, would be preserved.

What is the stage of development of this medicinal product?
The effects of inolimomab were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with graft versus host disease were ongoing.

Inolimomab was not marketed anywhere worldwide for graft versus host disease 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 15 January 2001 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:
Sponsor's contact details:
EUSA Pharma SAS
3, Allée des Séquoias
69760 Limonest
France
Telephone: +33 4 37 49 85 85
Telefax: +33 4 37 49 86 01
E-mail: info@eusapharma.com

Patients' associations contact points: Not available

Translations of the active ingredient and indication in all EU languages

LANGUAGE	Active Ingredient	Indication
English	Inolimomab	Treatment of graft versus host disease
Danish	Inolimomab	Behandling af transplantat kontra recipient-sygdommen
Dutch	Inolimomab	Behandeling van graft-versus-host-reactie
Finnish	Inolimomabi	Hyljintäreaktion hoito
French	Inolimomab	Traitement de la maladie du greffon contre l'hôte
German	Inolimomab	Behandlung der Transplantat-Wirt-Reaktion
Greek	Inolimomab	Θεραπεία της νόσου του μοσχεύματος έναντι του ξενιστή
Italian	Inolimomab	Trattamento della reazione immunologica del trapianto contro l'ospite
Portuguese	Inolimomab	Tratamento da doença do enxerto contra o hospedeiro
Spanish	Inolimomab	Tratamiento de la enfermedad del injerto contra el huésped
Swedish	Inolimomab	Behandling av transplantat-värdreaktion