



COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

**PUBLIC SUMMARY OF
POSITIVE OPINION FOR ORPHAN DESIGNATION
OF
murine anti-idiotypic antibody against OC125 antibody
against CA125 antigen
for the treatment of ovarian cancer**

On 9 July 2003, orphan designation (EU/3/03/155) was granted by the European Commission to Cell Control Biomedical Laboratories AG, Germany, for murine anti-idiotypic antibody against OC125 antibody against CA125 antigen (ACA125) for the treatment of ovarian cancer. The sponsorship was transferred to Menarini Ricerche S.p.A. in December 2004.

What is ovarian cancer?

Tumours that begin in the ovaries are known as ovarian tumours. Tumours which have potential to grow rapidly and infiltrate surrounding healthy tissues are called ovarian cancers. Due to the absence of symptoms in early stages of the disease the majority of the patients are diagnosed when the tumours have spread locally or to distant parts of the body. Ovarian cancer is a life-threatening condition.

What are the methods of treatment available?

Several anticancer medicinal products had been authorised for the condition in the Community at the time of submission of the application for orphan designation. Although a significant percentage of patients respond to the initial chemotherapy (using drugs to kill cancer cells), most ovarian cancers grow again and respond moderately or poorly to subsequent chemotherapy.

ACA125 might be of potential significant benefit for the treatment of ovarian cancer, because it may offer a new mechanism of action to fight the disease. This assumption will have to be confirmed. This will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, ovarian cancer was considered to affect about 90,000 persons in the European Union.

How is this medicinal product expected to act?

Antibodies are proteins in the body that target specific shapes on the surface of foreign bodies, such as bacteria or cancer cells. CA125 is a protein that can be found on the surface of the ovarian cancer cells. ACA125 is a special type of antibody that mimicks CA125, and this is in turn expected to trigger the production of other antibodies against CA125. Antibodies such as ACA125 that trigger the production of other antibodies are called “anti-idiotypic” antibodies. This anti-idiotypic antibody is shaped as a “double mirror-image” of the CA125 protein. By mimicking the CA125 protein, ACA125 may help the body's immune system to attack and kill the cancer cells.

What is the stage of development of this medicinal product?

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

At the time of submission, ACA125 was not marketed anywhere worldwide for ovarian cancer 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 13 June 2003 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:

Menarini Ricerche S.p.A.

Via Tito Speri 10

00040 Pomezia (Roma)

Italy

Telephone: +39 05 55 68 03 13

Telefax: +39 05 55 68 09 95 4

E-mail: abagovomab@menarini-ricerche.it

*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.