The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

Document Date: London, 11 June 2003
EMEA/COMP/965/02 Rev. 3

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF miltefosine for the treatment of visceral leishmaniasis

On 12 June 2002, orphan designation (EU/3/02/104) was granted by the European Commission to Zentaris Aktiengesellschaft, Germany, for miltefosine for the treatment of visceral leishmaniasis.

What is leishmaniasis?
Leishmania are small organisms that consist of a single cell. They are called parasites because they live inside other cells, from which they obtain food. Leishmania can be transmitted to humans through the bite of sand flies, mostly in the southern part of Europe. Visceral leishmaniasis, the most severe form of the infection, is characterised by multiple abnormal functioning of the blood and immune system and frequent infections. A large part of leishmania cases in the European Union occur as co-infections in HIV infected patients in Spain, Portugal, France and Italy.

What are the methods of treatment available?
Several products were authorised for the condition in the Community at the time of submission of the application for orphan designation. In a significant percent of patients, the disease can be cured following initial therapy by means of chemicals. However, available therapy may not be sufficient in some patients, especially those that are also infected by HIV. In these patients, the disease does not respond well to the therapy and tends to come back. Miltefosine might be of potential significant benefit for the treatment of visceral leishmaniasis, particularly based on the effects demonstrated in experimental models and in clinical trials.

What is the estimated number of patients affected by the condition?
According to the information provided by the sponsor, visceral leishmaniasis was considered to affect about 4,000 people in the European Union.

How is this medicinal product expected to act?
Miltefosine limits the building up of certain fat substances that are produced and used by the leishmania cells. These substances are called sphingolipids. They play an important role in the function of the parasites.

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 visceral leishmaniasis were completed. The first authorisation for the treatment of visceral leishmaniasis was granted in India in March 2002.

No orphan designations had been obtasined in any non-European Union countries including the United States of America.

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According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 30 April 2002 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 have been 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:

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*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.