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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF thymalfasin for the treatment of hepatocellular carcinoma

On 30 July 2002, orphan designation (EU/3/02/110) was granted by the European Commission to SciClone Pharmaceuticals Italy S.r.l., Italy, for thymalfasin for the treatment of hepatocellular carcinoma.

What is hepatocellular carcinoma?
Tumours that begin in the liver are known as liver tumours. Liver tumours which have the potential to grow rapidly and infiltrate healthy tissues are called hepatocellular carcinomas. Hepatocellular carcinoma is a life-threatening condition.

What are the methods of treatment available?
No medicinal products for the treatment of the condition had been authorised in the European Union at the time of submission of the application for orphan drug designation.

What is the estimated number of patients affected by the condition ?
According to the information provided by the sponsor, hepatocellular carcinoma was considered to affect about 37,500 persons in the European Union.

How is this medicinal product expected to act?
Thymalfasin is a small protein. It was originally isolated from the thymus gland of cattle. Currently, this protein is produced by a process of chemical reactions. Thymalfasin acts on the immune system. This may increase the ability of immune cells called T-lymphocytes to kill tumour 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 hepatocellular carcinoma were ongoing.

In the European Union thymalfasin is authorised in Italy, where it is used to enhance the immune response stimulated by the influenza virus vaccine.

The sponsor has been granted orphan drug status for thymalfasin in treatment of hepatocellular carcinoma in the United States.

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 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.