



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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2004 on request from the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

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

On 31 July 2001, orphan designation (EU/3/01/052) was granted by the European Commission to Leo Pharmaceutical Products, Denmark, for seocalcitol for the treatment of hepatocellular carcinoma.

What is hepatocellular carcinoma?

Tumours growing 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?

Seocalcitol is similar to vitamin D (a so-called vitamin D analogue) but with a slightly altered structure. Specific receptors (cell proteins that can only bind other substances having a specific shape, in order to activate certain biologic reactions of the cell) within liver cells can bind to the vitamin D and its analogues. It seems that hepatocellular carcinoma cells have a higher number of these receptors than in normal liver tissue. It has been shown that the binding of the vitamin D to its receptor might help in reducing the growth rate of cancer cells. Being similar to vitamin D, Seocalcitol might thereby stop further growth and spreading of the tumour cells.

What is the stage of development of this medicinal product?

The effects of seocalcitol were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with hepatocellular carcinoma were ongoing.

Seocalcitol was not marketed anywhere worldwide for the treatment of hepatocellular carcinoma 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 12 June 2001 a positive opinion recommending the grant of the above-mentioned designation.

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: mail@emea.eu.int www.emea.eu.int

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:

Leo Pharmaceutical Products

Industriparken 55

DK-2750 Ballerup

Denmark

Telephone: (45-44) 92 38 00

Telefax: (45-44) 53 50 88

E-mail: gms@leo-pharma.com

Patients associations' contact points:

CancerBACUP

3 Bath Place, Rivington Street

London EC2A 3JR

United Kingdom

Tel: +44 (0)20 7696 9003

Ligue Nationale Contre le Cancer

14 rue Corvisart

F-75013 Paris

France

*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.

Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Seocalcitol	Treatment of hepatocellular carcinoma
Danish	Seocalcitol	Behandling af hepatocellulært carcinom
Dutch	Seocalcitol	Behandeling van hepatocellulair carcinoom
Finnish	Seokalsitoli	Hepatosellulaarisen karsinooman hoito
French	Séocalcitol	Traitement du carcinome hépatocellulaire
German	Seocalcitol	Behandlung des Leberzellkarzinoms
Greek	Σεοκαλσιτόλ	Θεραπεία του ηπατοκυτταρικού καρκινώματος
Italian	Seocalcitol	Trattamento del carcinoma epatocellulare
Portuguese	Seocalcitol	Tratamento do carcinoma hepatocelular
Spanish	Seocalcitol	Tratamiento de carcinoma hepatocelular
Swedish	Seokalcitol	Behandling av hepatocellulärt karcinom