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

Document Date: London, 22 February 2007
Doc.Ref.: EMEA/COMP/1228/03 Rev.1

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in October 2006 on request of the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF aldesleukin (inhalation use) for the treatment of renal cell carcinoma

On 30 June 2003, orphan designation (EU/3/03/146) was granted by the European Commission to Chiron B. V., The Netherlands, for aldesleukin (inhalation use) for the treatment of renal cell carcinoma.
The sponsorship was transferred to Chiron Corporation Limited, United Kingdom, in November 2005.

What is renal cell carcinoma?
Renal cell carcinoma (also called cancer of the kidney or renal adenocarcinoma) is a disease in which cancer (malignant) cells are found in certain tissues of the kidney. Inside each kidney are tiny tubules that filter and clean the blood, taking out waste products, and making urine. Renal cell carcinoma is a cancer of the lining of the tubules in the kidney. Renal cell carcinoma accounts for approximately 85% of all kidney cancers. Signs of cancer are difficult to detect in early stages of the disease, and about half of the patients are diagnosed when the disease has spread around the kidney or to distant parts of the body. Surgery is a common treatment of renal cell cancer, and allows taking out the cancer in an operation, although the cancer may appear again. Renal cell carcinoma is life-threatening.

What are the methods of treatment available?
There are treatments for most patients with renal cell cancer. These may include surgery (taking out the cancer in an operation), chemotherapy (using drugs to kill cancer cells), radiation therapy (using high-dose x-rays or other high-energy rays to kill cancer cells), hormone therapy (using hormones to stop cancer cells from growing), and biological therapy (using the body's immune system to fight cancer). Biologic agents, such as aldesleukin and interferon-α, are the primary therapies for advanced cancer. These molecules are generally injected under the skin. Other anticancer agents have been authorised in the Community for treatment of renal cell carcinoma at the time of submission of the application for orphan designation. A preparation of aldesleukin to be given by inhalation (drawn into the lungs) might be of potential significant benefit for the treatment of renal cell carcinoma. This could represent an additional treatment option for patients with renal cell carcinoma, when cancer cells have spread to the lungs. 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?
According to the information provided by the sponsor, renal cell carcinoma was considered to affect about 115,000 persons in the European Union.

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 www.emea.europa.eu

How is this medicinal product expected to act?
Aldesleukin is obtained using a technique which combines genetic materials to produce certain proteins that occur in the human body. This is called genetic engineering. Proteins produced using this technology are called recombinant proteins. Aldesleukin is a recombinant protein which corresponds to a human protein called interleukin 2. Interleukin 2 is important for the body's defense mechanisms. Interleukin 2 can activate certain cells of the immune system which can kill tumour cells. Thus, aldesleukin given by inhalation may be able to activate the patient's immune system against renal cell carcinoma cells that have spread to the lung.

What is the stage of development of this medicinal product?
At the time of submission of the application for orphan designation, clinical trials with aldesleukin (inhalation use) were ongoing.

Aldesleukin (inhalation use) has not been marketed anywhere worldwide for renal cell carcinoma, at the time of submission. No other Orphan Drug Designation was granted elsewhere.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 8 May 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:
Chiron Corporation Limited
Symphony House
7 Cowley Business Park, High Street
Cowley, Uxbridge
UB8 2AD
United Kingdom
Telephone: +44 20 85 80 40 00
Telefax: +44 20 85 80 40 97

*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.

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