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

**Document Date:** London, 18 July 2007
Doc.Ref.: EMEA/COMP/206437/2007

**COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

# PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF talactoferrinum alpha for the treatment of renal cell carcinoma

On 5 June 2007, orphan designation (EU/3/07/448) was granted by the European Commission to Agennix Limited, United Kingdom, for talactoferrinum alpha for the treatment of renal cell carcinoma.

## 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 there 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 these tubules in the kidney. Renal cell carcinoma accounts for approximately 85% of all kidney cancers. Signs of this 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 carcinoma, 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). The primary therapy for advanced renal cell carcinoma is chemotherapy with biologic agents such as interleukin-2 and interferon-α. Recently, other anticancer drugs (sorafenib and sunitinib) aimed at stopping cancer cells growth have also been authorised in the Community for the treatment of renal cell carcinoma.
The sponsor has submitted satisfactory argumentation to justify the assumption that talactoferrinum alpha 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. This assumption will have to be confirmed at the time of marketing authorisation; 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 less than 193,000 persons in the European Union.

## How is this medicinal product expected to act?

Talactoferrinum alpha is a protein that is normally present in the human body. Talactoferrinum alpha binds to the cells that cover the gut, and stimulates an immune reaction in the so-called Gut Associated Lymphoid Tissue (GALT). GALT is a complex system of cells located in the gut that react to different stimuli, and respond by starting immune responses. When GALT response is triggered by

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

talactoferrinum, certain substances are produced and poured into the blood; these substances, in turn, stimulate other cells involved in anti-tumour immune reactions, even in distant organs (such as the kidney in this case).

**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 renal cell carcinoma were ongoing.

Talactoferrinum alpha was not authorised anywhere in the world for renal cell 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 April 2007 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:
Agennix Limited
Carmelite
50 Victoria Embankment
Blackfriars
London, EC4Y 0DX
United Kingdom
Telephone: +44 20 73 00 46 55
Telefax: +44 20 7438 07 64
E-mail: dduke@agennix.com

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Patients' associations contact points:

**Kidney Cancer UK**
11 Hathaway Road
Tile Hill Village
CV4 9HW
Coventry
Telephone: +44 24 76 47 49 93
E-mail: kidneycanceruk@hotmail.com

**Cancer BACUP**
3 Bath Place
Rivington Street
London
EC2A 3JR
United Kingdom
Telephone: +44 20 76 96 90 03 / 0808 800 1234 (freephone for UK)
Telefax: +44 20 76 96 90 02

**Ligue Nationale contre le Cancer (LNCC)**
12, rue Corvisart
75013 Paris
France
Tel : +33 1 53 55 24 00
Telefax : +33 1 43 36 91 10
E-mail: ligue@ligue-cancer.net

**Translations of the active ingredient and indication in all EU languages and Norwegian and Icelandic**

| Language | Active Ingredient | Indication |
|---|---|---|
| English | Talactoferrinum alfa | Treatment of renal cell carcinoma |
| Bulgarian | Талактоферин алфа | Лечение на бъбречно клетъчен карцином |
| Czech | Talaktoferrin alfa | Léčba karcinomu ledvin |
| Danish | Talactoferrinum alfa | Behandling af renalcellekarcinom |
| Dutch | Talactoferrin alfa | Behandeling van niercelcarcinoom |
| Estonian | Talaktoferriin alfa | Neeruvähi ravi |
| Finnish | Talaktoferriini alfa | Munuaiskarsinooman hoito |
| French | Talactoferrine alpha | Traitement du carcinome rénal |
| German | Talactoferrin alfa | Behandlung des Nierenzellkarzinoms |
| Greek | Ταλακτοφερρίνη άλφα | Θεραπεία του νεφροκυτταρικού καρκινώματος |
| Hungarian | Talactoferrin alfa | Vesekarcinoma kezelése |
| Italian | Talattoferrina alfa | Trattamento del carcinoma renale |
| Latvian | Alfa-talaktoferrīns | Nieru karcinomas ārstēšana |
| Lithuanian | Talaktoferinas alfa | Inkstų adenokarcinomos gydymas |
| Maltese | Talactoferrinum alfa | Kura tal-karċinoma taċ-ċelluli renali |
| Polish | Talaktoferryna alfa | Leczenie raka nerki |
| Portuguese | Talactoferrina alfa | Tratamento do carcinoma das células renais |
| Romanian | Alfa talactoferin | Tratamentul carcinomului renal |
| Slovak | Talaktoferín alfa | Liečba karcinómu obličky |
| Slovenian | Talaktoferin alfa | Zdravljenje raka ledvičnih celic |
| Spanish | Talactoferrina alfa | Tratamiento del carcinoma de células renales |
| Swedish | Talaktoferin alfa | Behandling av njurcellscancer |
| Norwegian | Talaktoferrin alfa | Behandling av nyrecellekarsinom |
| Icelandic | Talactóferrinum alfa | Meðferð á nýrnafrumukrabbameini |