



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Naptumomab estafenatox for the treatment of renal cell carcinoma

On 14 September 2007, orphan designation (EU/3/07/480) was granted by the European Commission to Active Biotech Research AB, Sweden, for naptumomab estafenatox for the treatment of renal cell carcinoma.

Active Biotech Research AB changed its name to Active Biotech AB in August 2011.

#### **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, producing 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 the 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 for renal cell cancer, and allows the removal of the cancer, although the cancer may appear again. Renal cell carcinoma is life-threatening.

#### **What is the estimated number of patients affected by the condition?**

At the time of designation, renal cell carcinoma affected less than 4.2 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of fewer than 209,000 people, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

#### **What treatments are available?**

At the time of the application for orphan designation, there were several treatments available for most patients with renal cell carcinoma. These included surgery (removal of the cancer in an operation), chemotherapy (using drugs to kill cancer cells), radiation therapy (using high-dose x-rays or other

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\*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 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.



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 therapies for advanced cancer are biologic agents, such as interleukin-2 and interferon-alpha. Other anticancer agents had also been authorised in the Community for treatment of renal cell carcinoma at the time of submission of the application for orphan designation.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that naptumomab estafenatox might be of potential significant benefit for the treatment of renal cell carcinoma as it might act in a different way to other available medicines and potentially improve the long-term outcome of the patients. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

### **How is this medicine expected to work?**

Antibodies are proteins produced by the immune system in the body that target specific shapes (antigens) on the surface of foreign bodies, such as bacteria or cancerous cells. Naptumomab estafenatox is a fusion protein consisting of one antigen binding fragment (Fab) from a cancer cell binding antibody and a bacterial superantigen. The latter is thought to bind specific types of cells of the immune system called T lymphocytes. By binding these cells, it is thought that the fusion protein can stimulate them to attack and kill the cancer cells recognized by the antibody part of this targeted drug.

### **What is the stage of development of this medicine?**

The effects of naptumomab estafenatox were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with renal cell carcinoma were ongoing.

Naptumomab estafenatox was not authorised anywhere in the world for the treatment of renal cell carcinoma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 25 July 2007 recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

## For more information

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

## Translations of the active ingredient and indication in all official EU languages<sup>1</sup>, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Naptumomab estafenatox	Treatment of renal cell carcinoma
Bulgarian	Наптумомаб естафенатокс	Лечение на бъбречно клетъчен карцином
Czech	Naptumomab estafenatox	Léčba karcinomu ledvin
Danish	Naptumomab estafenatox	Behandling af renalcellekarzinom
Dutch	Naptumomab estafenatox	Behandeling van niercelcarcinoom
Estonian	Naptumomab estafenatox	Neeruvähi ravi
Finnish	Naptumomabiestafenatoksi	Munuaiskarsinooman hoito
French	Naptumomab estafénatox	Traitement du carcinome rénal
German	Naptumomab estafenatox	Behandlung des Nierenzellkarzinoms
Greek	Naptumomab estafenatox	Θεραπεία του νεφροκυτταρικού καρκινώματος
Hungarian	Naptumomab estafenatox	Vesekarcinoma kezelése
Italian	Naptumomab estafenatox	Trattamento del carcinoma renale
Latvian	Naptumomaba estafenatoks	Nieru karcinomas ārstēšana
Lithuanian	Naptumomabo estafenatoksas	Inkstų adenokarcinomos gydymas
Maltese	Naptumomab estafenatox	Kura tal-karċinoma taċ-ċelluli renali
Polish	Naptumomab estafenatoks	Leczenie raka nerki
Portuguese	Naptumomab estafenatox	Tratamento do carcinoma das células renais
Romanian	Naptumomab estafenatox	Tratamentul carcinomului renal
Slovak	Naptumomab estafenatox	Liečba karcinómu obličky
Slovenian	Naptumomab estafenatoks	Zdravljenje raka ledvičnih celic
Spanish	Naptumomab estafenatox	Tratamiento del carcinoma de células renales
Swedish	Naptumomab estafenatox	Behandling av njurcellscancer
Norwegian	Naptumomab estafenatox	Behandling av nyrecellekarsinom
Icelandic	Naptúmomab estafenatox	Meðferð á nýrnafrumukrabbameini

<sup>1</sup> At the time of designation