European Medicines Agency
*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 recombinant modified vaccinia Ankara expressing human 5T4 for the treatment of renal cell carcinoma

On 26 January 2007, orphan designation (EU/3/06/429) was granted by the European Commission to Oxford Biomedica (UK) Ltd, United Kingdom, for recombinant modified vaccinia Ankara expressing human 5T4 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 are tiny tubules that filter and clean the blood, taking out waste products, and making urine. Renal cell carcinoma is a cancer 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 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 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 immunotherapy (using the body's immune system to fight cancer). The primary therapies for advanced cancer are biologic agents, such as interleukin-2 and interferon-α and chemotherapeutics such as sorafenib and sunitinib. Other anticancer agents had also been authorised in the Community for the 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 recombinant modified vaccinia Ankara expressing human 5T4 might be of potential significant benefit for the treatment of renal cell carcinoma. The 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 about 165,000 persons in the European Union.

[*] 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.

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**How is this medicinal product expected to act?**
The product is composed by an inactivated (damaged) virus, which includes an extra gene with the information for the synthesis of the protein 5T4 (which is a protein normally produced by kidney cancer cells). The virus cannot cause any disease, but is able to insert the gene for 5T4 in normal cells The objective of the treatment is to introduce this gene in the human body, in order to produce the 5T4 protein which then stimulates an immune response by the body immune system against itself. As 5T4 is a protein that is present in renal cell carcinoma cells, this immune response will also have an effect on cancer cells, potentially destroying them.

**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.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 6 December 2006 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 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.

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

| Language | Active Ingredient | Indication |
|---|---|---|
| English | Recombinant modified vaccinia Ankara expressing human 5T4 | Treatment of renal cell carcinoma |
| Bulgarian | Рекомбинантен модифициран vaccinia Ankara, експресиращ човешки 5T4 | Лечение на бъбречно клетъчен карцином |
| Czech | Rekombinantní modifikovaná vakcínie Ankara vytlačující humánní 5T4 | Léčba karcinomu ledvin |
| Danish | Rekombinant modificeret vaccinia Ankara, der udtrykker human 5T4 | Behandling af renalcellekarcinom |
| Dutch | Recombinant gemodificeerd vaccinia Ankara welke humaan 5T4 uitdrukt | Behandeling van niercelcarcinoom |
| Estonian | Rekombinantne modifitseeritud vaktsiiniaviirus Ankara, mis ekspresseerib inimese 5T4 | Neeru vähi-ravi |
| Finnish | Ihmisen 5T4-antigeenia ilmentävä rekombinoitu modifioitu Vaccinia Ankara | Munuaiskarsinooman hoito |
| French | Virus recombinant de la vaccine (souche modifiée Ankara) exprimant la protéine 5T4 humaine | Traitement du carcinome rénal |
| German | Humanes, 5T4 exprimierendes rekombinantes „Modified Vaccinia Ankara" (MVA) | Behandlung des Nierenzellkarzinoms |
| Greek | Γενετικά ανασυνδυασμένος τροποποιημένος ιός vaccinia Ankara (MVA) που εκφράζει το ανθρώπινο αντιγόνο 5T4 | Θεραπεία του νεφροκυτταρικού καρκινώματος |
| Hungarian | Rekombináns módosított Ankara vakcina által expresszált humán 5T4 | Vesekarcinoma kezelése |
| Italian | MVA (Virus vaccinico Ankara modificato) ricombinante esprimente 5T4 umano | Trattamento del carcinoma renale |
| Latvian | Rekombinantā modificētā baku vakcīnas vīrusa Ankara štamms ar ekspresētu cilvēka 5T4 antigēnu | Nieru karcinomas ārstēšana |
| Lithuanian | Rekombinantinis modifikuotas *Vaccinia* viruso Ankara vektorius, ekspresuojantis žmogaus 5T4 antigeną | Inkstų adenokarcinomos gydymas |
| Polish | Rekombinowany modyfikowanyszczep wirusa krowianki (Ankara) z ekspresją ludzkiego 5T4 | Leczenie raka nerki |
| Portuguese | Vacínia Ankara recombinante modificada exprimindo 5T4 humano | Tratamento do carcinoma das células renais |
| Romanian | Vaccinia Ankara modificată recombinant care exprimă proteina umană 5T4 | Tratamentul carcinomului renal |
| Slovak | Rekombinantná modifikovaná vakcína Ankara vytlačujúca humánne 5T4 | Liečba karcinómu obličky |
| Slovenian | Rekombinantno modificirano cepivo Ankara z ekspresijo humanega 5T4 | zdravljenje raka ledvičnih celic |

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| Spanish | Virus Ankara de la vacuna recombinante modificado que expresa la proteína humana 5T4a | Tratamiento del carcinoma de células renales |
| Swedish | Rekombinant modifierat vaccinia Ankara som kodar för mänskligt 5T4 | Behandling av njurcellscancer |
| Norwegian | Rekombinant, modifisert vaccinia Ankara som utrykker humant 5T4 | Behandling av nyrecellekarsinom |
| Icelandic | Raðbrigða breyttar vaccinia Ankara veirur sem tjá manna 5T4 | Meðferð á nýrnafrumukrabbameini |