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Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Temsirolimus for the treatment of renal cell carcinoma

On 6 April 2006, orphan designation (EU/3/06/365) was granted by the European Commission to Wyeth Europa Limited, United Kingdom, for temsirolimus for the treatment of renal cell carcinoma.

The sponsorship was transferred to Pfizer Limited, United Kingdom, in September 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, taking out waste products, and 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 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. Renal cell carcinoma is life-threatening.

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

At the time of designation renal carcinoma affected approximately 3.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 161,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

### What treatments are available?

There are treatments for most patients with renal cell carcinoma. 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 therapies for advanced cancer are biologic agents, such as interleukin-2

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

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and interferon-alpha. Other anticancer agents were also authorised in the Community for treatment of renal cell carcinoma at the time of submission of the application for orphan designation.

Temsirolimus might be of potential significant benefit for the treatment of renal cell carcinoma because it might act in a different way and thereby improve the long-term outcome of the patients. The 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?

Enzymes are proteins that speed up (catalyse) chemical reactions. Temsirolimus blocks the enzyme serine/threonine kinase. Serine/threonine kinase transmits signals from outside inside the cell via a cascade of molecular reactions and controls cell division and growth. In cancer, the function of this enzyme is disturbed causing uncontrolled growth and multiplication of the cancer cells. By inhibiting serine/threonine kinase, temsirolimus might delay or completely stop the further growth of cancer cells.

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

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

Temsirolimus was not authorised anywhere worldwide for renal cell carcinoma, at the time of submission. Orphan designation of temisirolimus was granted in the United States for renal cell carcinoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 March 2006 recommending the granting of this designation.

Update: Temsirolimus (Torisel) has been authorised in the EU since 19 November 2007.

TORISEL is indicated for the first-line treatment of patients with advanced renal cell carcinoma who have at least three of six prognostic risk factors.

More information on Torisel can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

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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[1], Norwegian and Icelandic

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

[1] At the time of transfer of sponsorship