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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sorafenib tosylate for the treatment of renal cell carcinoma

On 29 July 2004, orphan designation (EU/3/04/207) was granted by the European Commission to Bayer Healthcare AG, Germany, for sorafenib tosylate for the treatment of renal cell carcinoma.

The sponsorship was transferred to Bayer Shering Pharma AG, Germany, in April 2009. Bayer Shering Pharma AG changed its name to Bayer Pharma AG in October 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 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 is the estimated number of patients affected by the condition?

At the time of designation, renal cell carcinoma affected approximately 3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 116,000 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 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

*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 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition.

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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. 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 sorafenib tosylate 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.

How is this medicine expected to work?

Sorafenib tosylate is a chemically synthesised product which might play a role in the series of reactions by which an external signal (e.g. a hormone) interacts with a cell and triggers a reaction in the cell itself leading to a change in the function of the cell. The sponsor suggested two types of possible ways of actions on renal cell carcinoma. The first one would concern the series of reactions playing a role in the multiplication of cancer cells (the so-called tumour proliferation). The second way sorafenib tosylate might play a role, is in the series of reactions controlling the viability of the cancer and the process by which the blood vessels are made within the cancer mass (angiogenesis).

What is the stage of development of this medicine?

The evaluation of the effects of sorafenib tosylate in experimental models is ongoing.

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

Sorafenib tosylate was not marketed anywhere worldwide for 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 16 June 2004 recommending the granting of this designation.

Update: Sorafenib tosylate (Nexavar) has been authorised in the EU since 19 July 2006 for treatment of patients with advanced renal cell carcinoma who have failed prior interferon-alpha or interleukin-2 based therapy or are considered unsuitable for such therapy.

More information on Nexavar 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	Sorafenib tosylate	Treatment of renal cell carcinoma
Bulgarian	Сорафениб тосилат	Лечение на бъбречно клетъчен карцином
Czech	Sorafenib tosylat	Léčba renálního karcinomu
Danish	Sorafenibtosylat	Behandling af renalcellekarcinom
Dutch	Sorafenib tosylaat	Behandeling van renaal celcarcinoom
Estonian	Sorafeniibtosülaat	Neeruvähi ravi
Finnish	Sorafenibtosylatti	Munuaissolukarsinooman hoito
French	Tosylate de sorafénib	Traitement du carcinome rénal
German	Sorafenibtosylat	Behandlung von Nierenzellkarzinom
Greek	Sorafenib tosylate	Θεραπεία του νεφροκυτταρικού καρκινώματος
Hungarian	Sorafenib tosylate	Vesesejtes carcinoma kezelése
Italian	Sorafenib tosilato	Trattamento del carcinoma a cellule renali
Latvian	Sorafeniba tosilāts	Nieru karcinomas ārtēšana
Lithuanian	Sorafenibo tozilatas	Inkstų ląstelių karcinomos gydymas
Maltese	Sorafenib tosylate	Kura tal-karċinoma taċ-ċelluli renali
Polish	Tosylat sorafenibu	Leczenie raka nerki
Portuguese	Tosilato de sorafenib	Tratamento do carcinoma das células renais
Romanian	Tosilat de sorafenib	Tratamentul carcinomului renal
Slovak	Tosylát sorafenibu	Liečba karcinómu obličky
Slovenian	Sorafenib-tozilat	Zdravljenje karcinoma ledvičnih celic
Spanish	Sorafenib tosilato	Tratamiento del carcinoma de células renales
Swedish	Sorafenibtosylat	Behandling av njurcellskarcinom
Norwegian	Sorafenibtosylat	Behandling av nyrecellekarsinom
Icelandic	Sorafenibtosylat	Meðferð á nýrafrumukrabbameini

[1] At the time of transfer of sponsorship