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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pazopanib hydrochloride for the treatment of renal cell carcinoma

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in April 2010 on request of the sponsor.

On 29 June 2006, orphan designation (EU/3/06/382) was granted by the European Commission to GlaxoSmithKline Research & Development Limited, United Kingdom, for pazopanib hydrochloride for the treatment of renal cell carcinoma.

The sponsorship was transferred to Glaxo Group Limited, United Kingdom, in December 2008.

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 and it accounts for approximately 85% of all kidney cancers. Signs of renal 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.

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

At the time of designation, renal cell 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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

*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).

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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 immuno-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 pazopanib hydrochloride might be of potential significant benefit for the treatment of renal cell carcinoma because it might act in a different way from other available medicines, and might improve the long-term outcome of the patients and counteract the development of drug resistance. 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?

Pazopanib hydrochloride is a chemically synthesised product that is thought to be involved in the series of reactions by which an external signal (e.g. a hormone) interacts with a cell triggering a reaction in the cell. Pazopanib hydrochloride might play a role in the series of reactions that control the survival of the cancer cells and also in the process called angiogenesis (building of blood vessels) within the tumour.

What is the stage of development of this medicine?

The evaluation of the effects of pazopanib hydrochloride 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.

Pazopanib hydrochloride 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 May 2006 recommending the granting of this designation.

Update: Pazopanib hydrochloride (Votrient) has been authorised in the EU since 14 June 2010 for the first line treatment of advanced Renal Cell Carcinoma (RCC) and for patients who have received prior cytokine therapy for advanced disease.

More information on Votrient 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 European Union) 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	Pazopanib hydrochloride	Treatment of renal cell carcinoma
Bulgarian	Пазопаниб хидрохлорид	Лечение на бъбречно клетъчен карцином
Czech	Pazopanib hydrochloridum	Léčba karcinomu ledvin
Danish	Pazopanibhydrochlorid	Behandling af renalcellekarcinom
Dutch	Pazopanib hydrochloride	Behandeling van niercelcarcinoom
Estonian	Pazopaniib hüdrokloriid	Neeru vähi-ravi
Finnish	Patsopanibihydrokloridi	Munuaiskarsinooman hoito
French	Chlorhydrate de pazopanib	Traitement du carcinome rénal
German	Pazopanibhydrochlorid	Behandlung des Nierenzellkarzinoms
Greek	Υδροχλωρική παζοπανίμπη	Θεραπεία του νεφροκυτταρικού καρκινώματος
Hungarian	Pazopanib hidroklorid	Vesekarcinoma kezelése
Italian	Pazopanib cloridrato	Trattamento del carcinoma renale
Latvian	Pazopaniba hidrohlorīds	Nieru karcinomas ārstēšana
Lithuanian	Pazopanibo hidrochloridas	Inkstų adenokarcinomos gydymas
Maltese	Pazopanib hydrochloride	Kura tal-karċinoma taċ-ċelluli renali
Polish	Chlorowodorek pazopanibu	Leczenie raka nerki
Portuguese	Cloridrato de pazopanib	Tratamento do carcinoma das células renais
Romanian	Clorhidrat de pazopanib	Tratamentul carcinomului renal
Slovak	Pazopanibiumchlorid	Liečba karcinómu obličky
Slovenian	Pazopanib klorid	zdravljenje raka ledvičnih celic
Spanish	Hidrocloruro de pazopanib	Tratamiento de carcinoma de células renales
Swedish	Pazopanibhydroklorid	Behandling av njurcellscancer
Norwegian	Pazopanibhydroklorid	Behandling av nyrecellekarsinom
Icelandic	Pazópaníb hýdróklóríð	Meðferð á nýrnafrumukrabbameini

[1] At the time of transfer of sponsorship