28 November 2012
EMA/COMP/2608/2011, Rev. 1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Axitinib for the treatment of renal cell carcinoma

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in July 2012 on request of the sponsor

On 23 February 2011, orphan designation (EU/3/10/844) was granted by the European Commission to Pfizer Limited, United Kingdom, for axitinib for the treatment renal cell carcinoma.

What is renal cell carcinoma?

Renal cell carcinoma is a type of kidney cancer that originates in the cells lining the kidney tubules. These are small tubes that filter waste products out from the blood and make urine. Signs of renal cell carcinoma are difficult to detect in the early stages of the disease, and about half of the patients are diagnosed when the cancer has spread around the kidney or to other 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 4 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 202,520 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 designation, several medicines were authorised in the EU for the treatment of renal cell carcinoma. The main treatment was surgery, which was combined with radiotherapy (treatment with radiations) and chemotherapy (medicines to treat cancer) when the cancer had spread outside of the kidneys.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with renal cell carcinoma as it could, through its mechanism of action, complement

*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 506,300,000 (Eurostat 2011).

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medicines currently available, as shown in early studies. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Axitinib is expected to work by blocking the activity of 'vascular endothelial growth factor' (VEGF) receptors. These receptors, which can be found on the surface of cancer cells, are involved in the development of new blood vessels that supply the tumours. By blocking VEGF receptors, this medicine is expected to slow down the growth of cancer cells by reducing their blood supply.

What is the stage of development of this medicine?

The effects of axitinib have been evaluated in experimental models.

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

At the time of submission, axitinib was not authorised anywhere in the EU for renal cell carcinoma or designated as an orphan medicinal product elsewhere for this condition

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 December 2010 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[1], Norwegian and Icelandic

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

[1] At the time of designation