4 February 2015
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Everolimus for the treatment of renal cell carcinoma

First publication	18 July 2007
Rev.1: information about Marketing Authorisation	17 November 2009
Rev.2: withdrawal from the Community Register	1 September 2011
Rev.3: sponsor's change of address	4 February 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in July 2011 on request of the sponsor.

On 5 June 2007, orphan designation (EU/3/07/449) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for everolimus 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 there 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 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 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.

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

At the time of designation, renal cell carcinoma affected less than 4.2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 210,000 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 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 and interferon-α. Recently, other anticancer agents (sorafenib and sunitinib) aimed at stopping cancer cells growth have also been authorised in the Community for the treatment of renal cell carcinoma.

The sponsor submitted satisfactory argumentation to justify the assumption that everolimus might be of potential significant benefit for the treatment of renal cell carcinoma. This could represent an additional treatment option for patients with 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?

Enzymes are proteins produced by the human body that speed up the conversion of certain chemical substances of the body into other substances. Everolimus blocks (inhibits) a particular enzyme, called serine/threonine kinase. This enzyme plays a role in a number of molecular reactions, which control the growth and the division of the cells. In cancer cells, the function of this enzyme is disturbed, causing uncontrolled growth and multiplication of the cancer cells. Everolimus might, by inhibition of this enzyme activity, help in slowing down or stopping the further growth of the 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.

Everolimus was not authorised anywhere in the world for 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 12 April 2007 recommending the granting of this designation.

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*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.
At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

Update: Everolimus (Afinitor) has been authorised in the EU since 3 August 2009 for the treatment of patients with advanced renal cell carcinoma, whose disease has progressed on or after treatment with VEGF-targeted therapy.

More information on Afinitor 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.

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

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[1] At the time of designation