



4 February 2015
EMA/COMP/546609/2011 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Everolimus for the treatment of gastric cancer

First publication	20 September 2011
Rev.1: withdrawal from the Community Register	3 July 2012
Rev.2: 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Please note that this product was withdrawn from the Community register of designated orphan medicinal products in July 2012 on request of the sponsor.

On 30 August 2011, orphan designation (EU/3/11/894) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for everolimus for the treatment of gastric cancer.

What is gastric cancer?

Gastric cancer is a cancer that starts in the stomach, generally in the glandular cells lining the inside of the stomach. Gastric cancer is often detected late as the early signs of the disease are the same as those of less serious stomach conditions (heartburn, gas, excessive belching). At a later stage, gastric cancer causes unexplained weight loss, loss of appetite and general decline in health. Bleeding can occur, leading to anaemia (low red blood cell counts). Men are about twice as likely to develop the disease as women.

Gastric cancer is a serious and life-threatening illness that is associated with shortened life expectancy.



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

At the time of designation, gastric cancer affected approximately 3.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 198,000 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, some patients with gastric cancer were treated with surgery to remove part or the whole of the stomach. Chemotherapy (medicines to treat cancer) was generally used after surgery or on its own if surgery was not possible or the disease had spread to other parts of the body. Several chemotherapy medicines were authorised in the EU for use in gastric cancer, such as cisplatin, docetaxel, doxorubicin, capecitabine, epirubicin, 5-fluorouracil, mitomycin and trastuzumab. They were often used in combination.

The sponsor has provided sufficient information to show that everolimus might be of significant benefit for patients with gastric cancer, because it works in a different way to existing treatments and is administered in a more convenient manner than current treatments since it can be taken by mouth, which may improve the outcome of the treatment. 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?

Everolimus blocks the action of an enzyme called 'mammalian target of rapamycin' (mTOR) which regulates the growth and division of cells in the body and which has increased activity in patients with gastric cancer. In the body, everolimus first attaches to a protein called FKBP-12 that is found inside cells to make a 'complex'. This complex then blocks mTOR. Since mTOR is involved in the control of cell division and the growth of blood vessels, everolimus prevents the division of tumour cells and reduces their blood supply. This slows down the growth and proliferation of cancer cells.

What is the stage of development of this medicine?

The effects of everolimus have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with everolimus in patients with gastric cancer were ongoing.

At the time of submission, everolimus was not authorised anywhere in the EU for gastric cancer or designated as an orphan medicinal product elsewhere for this condition. At the time of submission, everolimus was authorised throughout the EU for the treatment of advanced renal cell carcinoma (kidney cancer that has started to spread).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 June 2011 recommending the granting of this designation.

*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 507,700,000 (Eurostat 2011).

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Everolimus	Treatment of gastric cancer
Bulgarian	Еверолимус	Лечение на карцином на стомаха
Czech	Everolimus	Léčba karcinomu žaludku
Danish	Everolimus	Behandlingen af cancer ventriculi
Dutch	Everolimus	Behandeling van maagkanker
Estonian	Everoliimus	Maovähi ravi
Finnish	Everolimuusi	Mahasyövän hoito
French	Évérolimus	Traitement du cancer gastrique
German	Everolimus	Behandlung von Magenkrebs
Greek	Everolimus	Θεραπεία του γαστρικού καρκίνου
Hungarian	Everolimus	Gyomorrák kezelése
Italian	Everolimus	Trattamento del cancro gastrico
Latvian	Everolīms	Kuņģa vēža ārstēšana
Lithuanian	Everolimuzas	Skrandžio vėžio gydymas
Maltese	Everolimus	Kura tal-kanċer gastriku
Polish	Ewerolimus	Leczenie raka żołądka
Portuguese	Everolimus	Tratamento do carcinoma gástrico
Romanian	Everolimus	Tratamentul cancerului gastric
Slovak	Everolimus	Liečba rakoviny žalúdka
Slovenian	Everolimus	Zdravljenje karcinoma želodca
Spanish	Everolimus	Tratamiento del cáncer de estómago
Swedish	Everolimus	Behandling av magcancer
Norwegian	Everolimus	Behandling av magekreft
Icelandic	Everolímus	Meðferð við magakrabbameini

¹ At the time of designation