



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

15 November 2010  
EMA/COMP/20891/2006 Rev.1  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation vandetanib for the treatment of medullary thyroid carcinoma

***Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in August 2010 on request of the sponsor.***

On 24 January 2006, orphan designation (EU/3/05/344) was granted by the European Commission to AstraZeneca UK Limited, United Kingdom, for vandetanib for the treatment of medullary thyroid carcinoma.

### **What is medullary thyroid carcinoma?**

Thyroid cancer is a disease in which cancer (malignant) cells are found in certain tissues of the thyroid. The thyroid is a gland in the neck that is composed of mainly two different cell types: the follicular and parafollicular cells. The so-called follicular cells help to concentrate iodine and produce thyroid hormones. These hormones are important for the body growth and metabolism. The parafollicular cells produce a hormone called calcitonin that diminishes the calcium level in the blood. Depending on the type of cell in which the cancer cells originate, different types of thyroid cancer exist.

Medullary thyroid carcinoma is originating from the parafollicular cells (also called C cells), and represents only 5-9% of all thyroid cancers. Signs of cancer are difficult to detect in early stages of the disease and is often limited to a single local swelling of the thyroid gland which is not painful but can be felt by touching. Patients are frequently diagnosed when the disease has spread locally giving symptoms such as shortness of breath, difficulties in swallowing or changes in the voice. Some patients may have severe diarrhoea as a first sign of the disease.

Medullary thyroid carcinoma is a life-threatening disease.

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

At the time of designation, medullary thyroid carcinoma affected less than 0.7 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of fewer than 32,000 people, and is below the

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\*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).



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?**

No medicinal products were authorised for the treatment of medullary thyroid carcinoma in the Community at the time of submission of the application for orphan drug designation. The treatment consists of a complete surgical removal of the thyroid. When patients can no longer be managed by surgery alone, alternative effective treatment approaches are lacking.

### **How is this medicine expected to work?**

Enzymes are proteins produced by the human body that speed up the transformation of certain substances into other substances. Vandetanib blocks (inhibits) a certain class of enzymes called tyrosine kinases. These enzymes play a role in a cascade of molecular reactions to bring a certain signal from outside the cell into the cell thereby controlling the growth of cells. In medullary thyroid carcinoma, the function of some of these enzymes is disturbed causing uncontrolled growth and multiplication of the cancer cells. By inhibiting this enzyme activity, Vandetanib might help in slowing down or stopping the further growth of the cancer cells.

### **What is the stage of development of this medicine?**

The effects of vandetanib were evaluated in experimental models.

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

Vandetanib was not authorised anywhere worldwide for medullary thyroid carcinoma, at the time of submission. Orphan designation of vandetanib was granted in the United States for the treatment of patients with follicular thyroid carcinoma, medullary thyroid carcinoma and anaplastic thyroid carcinoma and locally advanced or metastatic papillary thyroid carcinoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 December 2005 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:

AstraZeneca UK Limited  
Medical Information  
Horizon Place  
600 Capability Green  
Luton LU1 3LU  
United Kingdom  
E-mail: [medical.informationuk@astrazeneca.com](mailto:medical.informationuk@astrazeneca.com)

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<sup>1</sup>, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Vandetanib	Treatment of medullary thyroid carcinoma
Czech	Vandetanib	Léčba medulárního karcinomu štítné žlázy
Danish	Vandetanib	Behandling af medullært thyreoideakarcinom
Dutch	Vandetanib	Behandeling van medullair schilkkliercarcinoom
Estonian	Vandetaniib	Medullaarse kilpnäärmevähi ravi
Finnish	Vandetanibi	Medullaarisen kilpirauhaskarsinooman hoito
French	Vandétanib	Traitement du cancer médullaire de la thyroïde
German	Vandetanib	Behandlung des medullären Schilddrüsenkarzinoms
Greek	Βαντετανίμπη	Θεραπεία του μυελοειδούς καρκινώματος του θυρεοειδούς.
Hungarian	Vandetanib	Medulláris thyroid carcinoma kezelése
Italian	Vandetanib	Trattamento del carcinoma midollare della tiroide
Latvian	Vandetanībs	Medulāras vairogdziedzera karcinomas ārstēšanai
Lithuanian	Vandetanibas	Medulinės skydliaukės karcinomos gydymas
Polish	Wandetanib	Leczenie raka rdzeniastego tarczycy
Portuguese	Vandetanib	Tratamento do carcinoma medular da tiróide
Slovak	Vandetanib	Liečba medulárneho karcinómu štítnej žľazy
Slovenian	Vandetanib	Zdravljenje medularnega karcinoma ščitnice
Spanish	Vandetanib	Tratamiento del cáncer medular de tiroides
Swedish	Vandetanib	Behandling av medullär thyreoideacancer
Norwegian	Vandetanib	Behandling av medullært thyreoideacarcinom
Icelandic	Vandetaníþ	Meðferð á merggerðarkrabbameini í skjaldkirtli

<sup>1</sup> At the time of designation