7 May 2013
EMA/COMP/193931/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lenvatinib for the treatment of follicular thyroid cancer

On 26 April 2013, orphan designation (EU/3/13/1119) was granted by the European Commission to Eisai Europe Limited, United Kingdom, for lenvatinib for the treatment of follicular thyroid cancer.

What is follicular thyroid cancer?

Thyroid cancer is a type of cancer affecting the thyroid, a small gland at the base of the neck that produces thyroid hormones. The thyroid is composed of two main cell types: follicular cells, which produce hormones that help regulate growth and metabolism (the process of breaking down substances in the body), and parafollicular cells, which produce a hormone called calcitonin that helps to regulate calcium levels in the blood. Follicular thyroid cancer originates in the follicular cells and it can spread to other parts of the body, usually via the blood stream.

Signs of follicular thyroid cancer are difficult to detect in the early stages of the disease and are usually limited to local swelling of the thyroid gland. Patients are often diagnosed when the disease has spread locally giving symptoms such as shortness of breath, difficulties in swallowing or changes in the voice.

Follicular thyroid cancer is a long-term debilitating disease which is life threatening if it does not respond to treatment and if the cancer spreads to other parts of the body.

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

At the time of designation, follicular thyroid cancer affected less than 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 10,000 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, the main treatment for follicular thyroid cancer in the EU was surgery to remove the thyroid. Therapy using radioactive iodine (^{131}I) to destroy thyroid cells was also used.

*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 509,000,000 (Eurostat 2013).

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Hormonal therapy was used as an additional treatment for preventing re-occurrence of the disease. In addition, the anticancer medicine doxorubicin was authorised for the treatment of follicular thyroid cancer in some EU Member States.

The sponsor has provided sufficient information to show that lenvatinib might be of significant benefit for patients with follicular thyroid cancer because early studies indicate that it might improve the outcome of patients whose cancer does not respond to therapy with radioactive iodine. 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?

Lenvatinib is a 'tyrosine kinase inhibitor'. This means that it blocks the activity of enzymes known as tyrosine kinases. These enzymes can be found in certain receptors (such as VEGF, FGFR and RET receptors) on the surface of cancer cells, where they activate several processes including cell division and the growth of new blood vessels. By blocking the activity of VEGF receptors, the medicine reduces the blood supply to the cancer cells, slowing down the cancer's growth. Lenvatinib also blocks the activity of FGFR and RET receptors, which appear to play a role in the growth of thyroid cancer cells.

The medicine is expected to be taken by mouth.

What is the stage of development of this medicine?

The effects of lenvatinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with lenvatinib in patients with follicular thyroid cancer were ongoing.

At the time of submission, lenvatinib was not authorised anywhere in the EU for follicular thyroid cancer. Orphan designation of lenvatinib had been granted in the United States of America for the treatment of follicular, medullary, anaplastic, and metastatic or locally advanced papillary thyroid cancer. Orphan designation had also been granted in Japan for the treatment of thyroid cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 March 2013 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	Lenvatinib	Treatment of follicular thyroid cancer
Bulgarian	Ленватиниб	Лечение на фоликулярен тироиден карцином
Czech	Lenvatinib	Léčba folikulárního karcinomu štítné žlázy
Danish	Lenvatinib	Behandling af follikulær tyreoideacancer
Dutch	Lenvatinib	Behandeling van folliculaire schildklierkanker
Estonian	Lenvatinib	Follikulaarse kilpnäärmevähi ravi
Finnish	Lenvatinibi	Follikulaarisen kilpirauhassyövän hoito
French	Lenvatinib	Traitement du cancer thyroïdien folliculaire
German	Lenvatinib	Behandlung des follikulären Schilddrüsenkarzinoms
Greek	Λενβατινίμπη	Θεραπεία του θυλακιώδους καρκινώματος του θυρεοειδούς
Hungarian	Lenvatinib	Follicularis pajzsmirigyrák kezelése
Italian	Lenvatinib	Trattamento del carcinoma follicolare della tiroide
Latvian	Lenvatinibs	Folikulāra vairogdziedzera vēža ārstēšana
Lithuanian	Lenvatinibas	Folikulinio skydliaukės vėžio gydymas
Maltese	Lenvatinib	Kura ta' kanċer follikulari tat-tirojde
Polish	Lenwatynib	Leczenie raka pęcherzykowego tarczycy
Portuguese	Lenvatinib	Tratamento do carcinoma folicular da tiroide
Romanian	Lenvatinib	Tratamentul cancerului tiroidian folicular
Slovak	Lenvatinib	Liečba folikulárneho karcinómu štítnej žľazy
Slovenian	Lenvatinib	Zdravljenje folikularnega raka ščitnice
Spanish	Lenvatinib	Tratamiento del carcinoma folicular de tiroides
Swedish	Lenvatinib	Behandling av follikulär sköldkörtelcancer
Norwegian	Lenvatinib	Behandling av follikulær thyreoideacancer
Icelandic	Lenvatíiníib	Meðferð skjaldbúskrabbameins

[1] At the time of designation