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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sorafenib tosylate for the treatment of papillary thyroid cancer

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Rev.1: sponsor's name change and information about Marketing Authorisation	27 June 2014

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.

On 13 November 2013, orphan designation (EU/3/13/1200) was granted by the European Commission to Bayer HealthCare AG, Germany, for sorafenib tosylate for the treatment of papillary thyroid cancer.

In May 2014, the sponsor changed name to Bayer Pharma AG.

What is papillary thyroid cancer?

Papillary 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. Papillary thyroid cancer originates in the follicular cells and it can spread to other parts of the body, usually via the lymphatic system.

Signs of papillary 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.

Papillary 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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

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

At the time of designation, papillary thyroid cancer affected between 1 and 3 in 10,000 people in the European Union (EU). This was equivalent to a total of between 51,000 and 154,000 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 papillary thyroid cancer in the EU was surgery to remove the thyroid. Therapy using radioactive iodine (^{131}I) to destroy thyroid cells was also used.

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

The sponsor has provided sufficient information to show that sorafenib tosylate might be of significant benefit for patients with papillary thyroid cancer because clinical 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?

Sorafenib tosylate is a cancer medicine already authorised as Nexavar for the treatment of liver and kidney cancer. The medicine is a 'protein kinase inhibitor'. This means that it blocks some specific enzymes known as protein kinases. These enzymes can be found in some receptors on the surface of cancer cells, where they are involved in the growth and spread of cancer cells, and in the blood vessels that supply the tumours, where they are involved in the development of new blood vessels. By blocking these enzymes, the medicine is expected to reduce the growth of cancer cells in patients with papillary thyroid cancer and cut off the blood supply that keeps cancer cells growing.

What is the stage of development of this medicine?

The effects of sorafenib tosylate have been evaluated in experimental models.

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

At the time of submission, sorafenib tosylate was authorised in the EU for the treatment of hepatocellular carcinoma (a type of liver cancer) and advanced renal-cell carcinoma (a type of kidney cancer).

At the time of submission, sorafenib tosylate was not authorised anywhere in the EU for papillary thyroid cancer. Orphan designation of sorafenib tosylate had been granted in the United States, Australia and Switzerland for some types of thyroid cancer, including papillary thyroid cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 October 2013 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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,200,000 (Eurostat 2013).

Update: sorafenib tosylate (Nexavar) has been authorised in the EU since 23 May 2014 for the treatment of patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma, refractory to radioactive iodine.

More information on Nexavar 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.

For more information

Sponsor's contact details:

Bayer Pharma AG
Müllerstrasse 178
13353 Berlin
Germany
Tel. +49 30 468 1111
Fax +49 30 468 15305
E-mail: bayer-pharma-gra-eu@bayer.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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sorafenib tosylate	Treatment of papillary thyroid cancer
Bulgarian	Сорафениб-тозилат	Лечение на папиларен тироиден карцином
Croatian	Sorafenib (u obliku sorafenibtosilata)	Liječenje papilarnog raka štitnjače
Czech	Sorafenib mesylát	Léčba papilárního karcinomu štítné žlázy
Danish	Sorafenib som tosylat	Behandling af papillær tyreoideacancer
Dutch	Sorafenib tosylaat	Behandeling van papillaire schildklierkanker
Estonian	Sorafeniib tosülaat	Papillaarse kilpnäärmevähi ravi
Finnish	Sorafenibitosylaatti	Papillaarisen kilpirauhassyövän hoito
French	Sorafénib (sous forme de tosylate)	Traitement du cancer thyroïdien papillaire
German	Sorafenib (als Tosilat)	Behandlung des papillären Schilddrüsenkarzinoms
Greek	Σοραφενίμπη τοσυλική	Θεραπεία του θηλώδους καρκινώματος του θυρεοειδούς
Hungarian	Szorafenib tozilát	Papillaris pajzsmirigyrák kezelése
Italian	Sorafenib tosilato	Trattamento del carcinoma papillare della tiroide
Latvian	Sorafeniib tosülaat	Papillāra vairogdziedzera vēža ārstēšana
Lithuanian	Sorafenibo tozilatas	Papilinio skydliaukės vėžio gydymas
Maltese	Sorafenib tosylate	Kura ta' kanċer papillari tat-tirojde
Polish	Sorafenibu tozylan	Leczenie raka brodawkowatego tarczycy
Portuguese	Sorafenib (na forma de tosilato)	Tratamento do carcinoma papilar da tiroide
Romanian	Sorafenib (sub forma de tosilat)	Tratamentul cancerului tiroidian papilar
Slovak	Sorafenib tozylát	Liečba papilárneho karcinómu štítnej žľazy
Slovenian	Sorafenib (v obliki tozilata)	Zdravljenje papilarnega raka ščitnice
Spanish	Sorafenib tosilato	Tratamiento del carcinoma papilar de tiroides
Swedish	Sorafenibtosylat	Behandling av papillär sköldkörtelcancer
Norwegian	Sorafenibtosylat	Behandling av papillær thyreoideacancer
Icelandic	Sórafeníb tósýlat	Meðferð totukrabbameins í skjaldkirtli

[1] At the time of designation