



1 June 2015
EMA/COMP/280092/2012 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea for the treatment of ovarian cancer

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| First publication | 2 July 2012 |
| Rev.1: transfer of sponsorship | 11 March 2013 |
| Rev.2: withdrawal from the Community Register | 1 June 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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2015 on request of the Sponsor.

On 6 June 2012, orphan designation (EU/3/12/1001) was granted by the European Commission to Abbott Laboratories, United Kingdom, for 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea for the treatment of ovarian cancer.

The sponsorship was transferred to AbbVie Ltd, United Kingdom, in January 2013.

What is ovarian cancer?

Ovarian cancer is cancer of the ovaries (two organs in the female reproductive system that produce eggs). Most ovarian cancers occur in women over the age of 50 years. Due to the absence of symptoms in the early stages of the disease, the majority of patients are diagnosed when the cancer has spread to other parts of the body.

Ovarian cancer is a life-threatening disease that is associated with poor long-term survival.



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

At the time of designation, ovarian cancer affected not more than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 153,000 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, several medicines were authorised in the EU for the treatment of ovarian cancer. The choice of treatment depended mainly on how advanced the disease was. Treatments included surgery and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with ovarian cancer because it works in a different way to existing treatments, and early studies in experimental models show that it may be of benefit when used alone and in combination with the anticancer medicine paclitaxel. Its use in combination with paclitaxel may offer an alternative to patients with 'platinum-resistant' ovarian cancer for whom existing treatments do not work. 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?

This medicine belongs to a group called 'Aurora kinase inhibitors'. These compounds work by blocking enzymes known as Aurora kinases, which play an important role in the control of cell division. Aurora kinases can be found in high amounts in cancer cells where they no longer function normally. By blocking these enzymes, the medicine is expected to stop the cancer cells from dividing.

The medicine is also expected to block enzymes known as tyrosine kinases, which are involved in the growth and spread of the cancer cells and in the development of new blood vessels that supply the tumours.

What is the stage of development of this medicine?

The effects of this medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with ovarian cancer were ongoing.

At the time of submission, this medicinal product was not authorised anywhere in the EU for ovarian cancer or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 April 2012 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 509,000,000 (Eurostat 2012).

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 | 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea | Treatment of ovarian cancer |
| Bulgarian | 1-(4-{4-амино-7-[1-(2-хидроксиетил)-1H-пиразол-4-ил]тиено[3,2-с]пиридин-3-ил}фенил)-3-(3-флуорофенил)уреа | Лечение на рак на яйчниците |
| Czech | 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}feryl)-3-(3-fluorofenyl)urea | Léčba karcinomu vaječníků |
| Danish | 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urinstof | Behandling af ovarie cancer |
| Dutch | 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl]-thieno-[3,2-c]-pyridin-3-yl}-feryl)-3-(3-fluorofenyl)-ureum | Behandeling van ovariumkanker |
| Estonian | 1-(4-{4-amino-7-[1-(2-hüdroksüetüül)-1H-pürasool-4-üül]tieno[3,2-c]püridiin-3-üül}fenüül)-3-(3-fluorofenüül)uurea | Munasarjavähi ravi |
| Finnish | 1-(4-{4-amino-7-[1-(2-hydroksietyyli)-1H-pyratsoli-4-yl]tieno[3,2-c]pyridiini-3-yl}fennyli)-3-(3-fluorofenyylil)urea | Munasarjasyövän hoito |
| French | 1-(4-{4-amino-7-[1-(2-hydroxyéthyl)-1H-pyrazol-4-yl]thieno[3,2-c]pyridine-3-yl}phényl)-3-(3-fluorophényl)urée | Traitement du cancer de l'ovaire |
| German | 1-(4-{4-Amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)harnstoff | Behandlung des Ovarialkarzinoms |
| Greek | 1-(4-{4-αμινο-7-[1-(2-υδροξυαιθυλο)-1H-πυραζολο-4-υλο]θειενο[3,2-с]πυριδινο-3-υλ}φαινυλο)-3-(3-φλουοροφαινυλο) ουρία | Θεραπεία του καρκίνου των ωοθηκών |
| Hungarian | 1-(4-{4-amino-7-[1-(2-hidroxietyl)-1H-pirazol-4-il]tieno[3,2-c]piridin-3-il}fenil)-3-(3-fluorofenil)urea | Petefészekrák kezelése |
| Italian | 1-(4-{4-amino-7-[1-(2-idrossietil)-1H-pirazol-4-il]tieno[3,2-c]piridin-3-il}fenil)-3-(3-fluorofenil)urea | Trattamento del carcinoma dell'ovaio |
| Latvian | 1-(4-{4-amino-7-[1-(2-hidroksietil)-1H-pirazol-4-il]tiēno[3,2-c]piridīn-3-il}fenil)-3-(3-fluorofenil)urīnviela | Olnīcu vēža ārstēšanai |
| Lithuanian | 1-(4-{4-amino-7-[1-(2-hidroksietil)-1H-pirazol-4-il]tieno[3,2-c]piridin-3-il}fenil)-3-(3-fluorofenil)urėja | Kiaušidžių vėžio gydymas |

¹ At the time of designation

| Language | Active ingredient | Indication |
|------------|--|-----------------------------------|
| Maltese | 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea | Kura għal kanċer tal-ovarji |
| Polish | 1-(4-{4-amino-7-[1-(2-hydroksyetylo)-1H-pirazolo-4-yl]tieno[3,2-c]pirydyn-3-yl}fenylo)-3-(3-fluorofenylo)mocznik | Leczenie raka jajnika |
| Portuguese | 1-(4-{4-amino-7-[1-(2-hidroxietil)-1H-pirazol-4-il]tieno[3,2-c]piridina-3-il}fenil)-3-(3-fluorofenil)ureia | Tratamento do carcinoma do ovário |
| Romanian | 1-(4-{4-amino-7-[1-(2-hidroxietil)-1H-pirazol-4-il]tieno[3,2-c]piridin-3-il}fenil)-3-(3-fluorofenil)uree | Tratamentul cancerului ovarian |
| Slovak | 1-(4-{4-amino-7-[1-(2-hydroxyetyl)-1H-pyrazol-4-yl]tieno[3,2-c]pyridín-3-yl}fenyl)-3-(3-fluorofenyl)močovina | Liečba rakoviny vaječníkov |
| Slovenian | 1-(4-{4-amino-7-[1-(2-hidroksietil)-1H-pirazol-4-yl]tieno[3,2-c]piridin-3-il}fenil)-3-(3-fluorofenil)sečnina | Zdravljenje raka na jajčnikih |
| Spanish | 1-(4-{4-amino-7-[1-(2-hidroxietil)-1H-pirazol-4-il]tieno[3,2-c]piridin-3-il}fenil)-3-(3-fluorofenil)urea | Tratamiento del cáncer de ovario |
| Swedish | 1-(4-{4-amino-7-[1-(2-hydroxyetyl)-1H-pyrazol-4-yl]tieno[3,2-c]pyridin-3-yl}fenyl)-3-(3-fluorofenyl)urea | Behandling av ovarialcancer |
| Norwegian | 1-(4-{4-amino-7-[1-(2-hydroksyetyl)-1H-pyrazol-4-yl]tieno[3,2-c]pyridin-3-yl}fenyl)-3-(3-fluorofenyl)urea | Behandling av eggstokkreft |
| Icelandic | 1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl]thieno[3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea | Meðferð eggjastokkkrabbameins |