22 June 2015
EMA/COMP/269491/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

{2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide for the treatment of ovarian cancer

On 21 May 2015, orphan designation (EU/3/15/1488) was granted by the European Commission to Right Track Regulatory Limited, United Kingdom, for {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide for the treatment of ovarian cancer.

What is ovarian cancer?

Ovarian cancer is cancer of the ovaries, the two organs in the female reproductive system that produce egg cells. 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 long-term debilitating and 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 less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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, 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 treatment and

*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 512,900,000 (Eurostat 2015).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

results from experimental studies suggest that use with existing treatment (i.e. pegylated liposomal doxorubicin) could increase its effectiveness. 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?

The medicine acts on certain receptors (known as Toll-like receptors) found on cells of the immune system (the body's natural defences). By its action on these receptors, the medicine is expected to stimulate the immune cells, increasing the ability of the immune system to recognise and attack cancer cells. When given in combination with other cancer treatments, the medicine is expected to help kill the cancer cells.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for ovarian cancer. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 April 2015 recommending the granting of this designation.

_____

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:

Right Track Regulatory Limited
2nd Floor Titan Court
3 Bishop Square
Hatfield AL10 9NA
United Kingdom
Tel. +44 (0)1707 226 025
E-mail: info@rtregulatory.co.uk

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	{2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide	Treatment of ovarian cancer
Bulgarian	{2-амино-8-[4-(пиролидинилкарбонил)фенил]-(3H-бензо[f]азепин-4-ил)}-N,N-дипропилкарбоксамид	Лечение на рак на яйчниците
Croatian	{2-amino-8-[4-(pirolidinilkarbonil)fenil]-(3H-benzo[f]azepin-4-il)}-N,N-dipropilkarboksamid	Liječenje raka jajnika
Czech	{2-amino-8-[4-(pyrrolidinylkarbonyl)fenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylkarboxamid	Léčba karcinomu vaječníků
Danish	{2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamid	Behandling af ovarie cancer
Dutch	{2-amino-8-[4-(pyrrolidinylcarbonyl)fenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide	Behandeling van ovariumkanker
Estonian	{2-amino-8-[4-(pürrolidinüülkarbonüül)fenüül]-(3H-benso[f]asepiin-4-üül)}-N,N-dipropüülkarboksamiid	Munasarjavähi ravi
Finnish	{2-amino-8-[4-(pyrrolidinyylikarbonyyli)fenyyli]-(3H-bentso[f]atsepiini-4-yl)}-N,N-dipropyylikarboksamiidi	Munasarjasyövän hoito
French	{2-amino-8-[4-(pyrrolidinylcarbonyl)phényl]-(3H-benzo[f]azépine-4-y l)}-N,N-dipropycarboxamide	Traitement du cancer de l'ovaire
German	{2-Amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamid	Behandlung des Ovarialkarzinoms
Greek	{2-αμινο-8-[4-(πυρρολιδινυλοκαρβονυλο)φαινυλο]-(3H-βενζο[φ]αζεπιν-4-υλ)}-Ν,Ν-διπροπυλκαρβοξαμίδη	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	{2-amino-8-[4-(pirrolidinilkarbonil)fenil]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropilkarboxamid	Petefészekrák kezelése
Italian	{2-ammino-8-[4-(pirrolidinilcarbonil)fenil]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropilcarbossamide	Trattamento del carcinoma dell'ovaio
Latvian	{2-amino-8-[4-(pirolidinilkarbonil)fenil]-(3H-benzo[f]azepīn-4-il)}-N,N-dipropilkarboksamīds	Olnīcu vēža ārstēšana
Lithuanian	{2-amino-8-[4-(pirolidinilkarbonil)fenil]-(3H-benzo[f]azepin-4-il)}-N,N-dipropilkarboksamidas	Kiaušidžių vėžio gydymas
Maltese	{2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide	Kura tal-kanċer ta' l-ovarji
Polish	{2-amino-8-[4-(pirolidynylokarbonylo)fenyl]-(3H-benzo[f]azepin-4-il)}-N,N-dipropylokarboksamid	Leczenie raka jajnika
Portuguese	{2-amino-8-[4-(pirrolidinilcarbonil)fenil]-(3H-benzo[f]azepin-4-il)}-N,N-dipropilcarboxamida	Tratamento do carcinoma do ovário
Romanian	{2-amino-8-[4-(pirolidinilcarbonil)fenil]-(3H-benzo[f]azepin-4-il)}-N,N-dipropilcarboxamidă	Tratamentul cancerului ovarian
Slovak	{2-amino-8-[4-(pyrolidinylkarbonyl)fenyl]-(3H-benzo[f]azepín-4-yl)}-N,N-dipropylkarboxamid	Liečba rakoviny vaječníkov

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	{2-amino-8-[4-(purolidinilkarbonil)fenil]-(3H-benzo[f]azepin-4-il)}-N,N-dipropilkarboksamid	Zdravljenje raka na jajčnikih
Spanish	{2-amino-8-[4-(pirrolidinilcarbonil)phenil]-(3H-benzo[f]azepin-4-il)}-N,N-dipropilcarboxamida	Tratamiento del cáncer de ovario
Swedish	{2-amino-8-[4-(pyrrolidinylkarbonyl)fenyl]-(3H-benso[f]azepin-4-yl)}-N,N-dipropylkarboxamid	Behandling av ovarialcancer
Norwegian	{2-amino-8-[4-(pyrrolidinylkarbonyl)fenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylkarboksamid	Behandling av eggstokkreft
Icelandic	{2-mínó-8-[4-(pýrrólidínýlkarbónýl)fenýl]-(3H-benzó[f]asepín-4-ýl)}-N,N-díprópýlkarboxamíð	Meðferð eggjastokkakrabbameins