EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 September 2014
EMA/COMP/457059/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide for the treatment of small cell lung cancer

On 22 August 2014, orphan designation (EU/3/14/1324) was granted by the European Commission to DualTpharma B.V., the Netherlands, for 2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide for the treatment of small cell lung cancer.

What is small cell lung cancer?

Small cell lung cancer is a type of lung cancer that usually develops in the central part of the lungs, and in which the cancer cells are small compared with other types of lung cancer. Small cell lung cancer is almost always caused by smoking. The cancer is difficult to detect in the early stages of the disease, and the majority of the patients are diagnosed when the cancer has spread and cannot be removed by surgery.

Small cell lung 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, small cell lung cancer affected approximately 1.12 in 10,000 people in the European Union (EU). This was equivalent to a total of around 57,000 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 small cell lung cancer. The choice of treatment depended mainly on how advanced the disease was. Treatments included chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that 2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide might be of significant benefit for patients with small cell lung cancer because

*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 511,100,000 (Eurostat 2014).

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

An agency of the European Union

© European Medicines Agency, 2014. Reproduction is authorised provided the source is acknowledged.

experimental studies suggest that when used in combination with radiotherapy it could reduce tumour growth and increase survival. 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 is a synthetic product with two main components and two mechanisms of action.

The sulfamide component works by blocking carbonic anhydrase IX, an enzyme that helps to maintains acid balance within cells. Although there are several types of carbonic anhydrase enzymes in the body, carbonic anhydrase IX is found mostly in cancer cells, which depend on the enzyme to prevent them from becoming too acidic.

The second component, 5-nitroimidazole, is what is known as a 'hypoxic radiosensitiser'. Cancer cells that grow at lower levels of oxygen than normal cells are more resistant to the effects of radiation. Hypoxic radiosensitisers make such cancer cells more sensitive and less resistant to radiotherapy.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with small cell lung cancer had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for small cell lung 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 10 July 2014 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:

DualTpharma B.V.
Bosschstraat 111 D 01
6211AW Maastricht
The Netherlands
Tel. +31 884 4556 66
Fax +31 884 4557 76
E-mail: info@dualtpharma.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	2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide	Treatment of small cell lung cancer
Bulgarian	2-(2-метил-5-нитро-1H-имидазол-1-ил)етил сулфамид	Лечение на дребноклетъчен карцином на белия дроб
Croatian	2-(2-metil-5-nitro-1H-imidazol-1-il)etil sulfamid	Liječenje karcinoma pluća malih stanica
Czech	2-(2-metyl-5-nitro-1H-imidazol-1-yl)etylsulfamidem	Léčba malobuněčného karcinomu plic
Danish	2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamid	Behandling af småcellet lungecancer
Dutch	2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide	Behandeling van kleincellig longcarcinoom
Estonian	2-(2-metüül-5-nitro-1H-imidasool-1-yl)etüülsulfamiidiga	Väikeserakulise kopsuvähi ravi
Finnish	2-(2-metyyli-5-nitro-1H-imidatsoli-1-yl)etyylisulfamidia	Keuhkojen pienisolusyövän hoito
French	2-(2-méthyl-5-nitro-1H-imidazol-1-yl)éthylsulfamide	Traitement du cancer du poumon à petites cellules
German	2-(2-Methyl-5-Nitro-1H-Imidazol-1-yl)äthylsulfamid	Behandlung des kleinzelligen Lungenkarzinoms
Greek	2-(2-μεθυλο-5-νιτρο-1Η-ιμιδαζολο-1-υλο)αιθυλοσουλφαμίδιο	Θεραπεία του μικροκυτταρικού καρκίνου του πνεύμονα
Hungarian	2-(2-metil-5-nitro-1H-imidazol-1-yl)etilszulfamid	Kissejtes tüdőrák kezelése
Italian	2-(2-metile-5-nitro-1H-imidazolo-1-yl)etilicosulfamide	Trattamento del cancro del polmone a piccole cellule (microcitoma)
Latvian	2-(2-metil-5-nitro-1H-imidazol-1-il)etilsulfamīds	Sīkšūnu plaušu vēža ārstēšana
Lithuanian	2-(2-metil-5-nitro-1H-imidazol-1-il)etilsulfamidas	Smulkialąstelinio plaučių vėžio gydymas
Maltese	2-(2-methyl-5-nitro-1H-imidazol-1-yl)ethylsulfamide	Kura tal-kanċer tal-pulmun b'ċelloli żgħar
Polish	2-(2-metyl-5-nitro-1H-imidazol-1-yl)etylsulfamid	Leczenie raka drobnokomórkowego pluc
Portuguese	2-(2-metil-5-nitro-1H-imidazol-1-il)etilo sulfamida	Tratamento do carcinoma de pequenas células do pulmão
Romanian	2-(2-metil-5-nitro-1H-imidazol-1-il)etilsulfamidă	Tratamentul cancerului pulmonar cu celule mici
Slovak	2-(2-metyl-5-nitro-1H-imidazol-1-yl)etylsulfamid	Liečba malobunkového karcinómu pľúc

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	2-(2-metil-5-nitro-1H-imidazol-1-il)etil sulfamide	Zdravljenje drobnocelicnega raka pljuc
Spanish	2-(2-metilo-5-nitro-1H-imidazol-1-yl)etílicosulfamida	Carcinoma de pulmón de células pequeñas
Swedish	2-(2-metyl-5-nitro-1H-imidazol-1-yl)etyl sulfamid	Behandling av småcellig lungcancer
Norwegian	2-(2-metyl-5-nitro-1H-imidazol-1-yl)etyl sulfamid	Behandling av småcellet lungekreft
Icelandic	2-(2-metýl-5-nítró-1H-ímídasól-1-ýl)etýlsúlfamíð	Til meðferðar við lungnakrabbameini af smáfrumugerð