



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

N-(bromoacetyl)-3,3-dinitroazetidide for the treatment of small cell lung cancer

On 17 January 2018, orphan designation (EU/3/17/1966) was granted by the European Commission to Sirius Regulatory Consulting Limited, United Kingdom, for N-(bromoacetyl)-3,3-dinitroazetidide (also known as RRx-001) 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 1.2 in 10,000 persons in the European Union (EU). This was equivalent to a total of around 62,000 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 the medicine might be of significant benefit for patients with small cell lung cancer, with early studies suggesting that this medicine may

*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 515,700,000 (Eurostat 2017).



improve the effectiveness of platinum-based chemotherapy. 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?

Although it is not known exactly how this medicine works, it is thought that it works mainly by encouraging macrophages (cells of the immune (defence) system) to attack cancer cells and protecting normal cells from the toxic effects of radio- and chemotherapy.

What is the stage of development of this medicine?

The effects of the 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 small cell lung cancer were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for small cell lung 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 7 December 2017 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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	N-(bromoacetyl)-3,3-dinitroazetidine	Treatment of small cell lung cancer
Bulgarian	N-(бромоацетил)-3,3-динитроазетидин	Лечение на дребноклетъчен карцином на белия дроб
Croatian	N-(bromoacetil)-3,3-dinitroazetidín	Liječenje karcinoma pluća malih stanica
Czech	N-(bromoacetyl)-3,3-dinitroazetidín	Léčba malobuněčného karcinomu plic
Danish	N-(bromoacetyl)-3,3-dinitroazetidín	Behandling af småcellet lungecancer
Dutch	N-(bromoacetyl)-3,3-dinitroazetidine	Behandeling van kleincellig longcarcinoom
Estonian	N-(bromoatsetüül)-3,3-dinitroasetidiin	Väikeserakulise kopsuvähi ravi
Finnish	N-(bromiasetyyli)-3,3-dinitroatsetidiini	Keuhkojen pienisolusyvän hoito
French	N-(bromo-acétyl)-3,3-dinitroazétidine	Traitement du cancer du poumon à petites cellules
German	N-(bromacetyl)-3,3-dinitroazetidín	Behandlung des kleinzelligen Lungenkarzinoms
Greek	N-(βρωμοακετυλο)-3,3-δινιτροαζετιδίνη	Θεραπεία του μικροκυτταρικού καρκίνου του πνεύμονα
Hungarian	N-(brómacetil)-3,3-dinitroazetidín	Kissejtes tüdőrák kezelése
Italian	N-(bromoacetil)-3,3-dinitroazetidina	Trattamento del cancro del polmone a piccole cellule (microcitoma)
Latvian	N-(bromacetil)-3,3-dinitroazetidīns	Sīkšūnu plaušu vēža ārstēšana
Lithuanian	N-(bromoacetil)-3,3-dinitroazetidinas	Smulkialąstelinio plaučių vėžio gydymas
Maltese	N-(bromoacetil)-3,3-dinitroazetidina	Kura tal-kanċer tal-pulmun b'ċelloli żgħar
Polish	N-(bromoacetylo)-3,3-dinitroazetydyna	Leczenie raka drobnokomórkowego płuc
Portuguese	N-(bromoacetil)-3,3-dinitroazetidina	Tratamento do carcinoma de pequenas células do pulmão
Romanian	N-(bromoacetil)-3,3-dinitroazetidină	Tratamentul cancerului pulmonar cu celule mici
Slovak	N-(brómacetyl)-3,3-dinitroazetidín	Liečba malobunkového karcinómu pľúc
Slovenian	N-(bromoacetil)-3,3-dinitroazetidín	Zdravljenje drobnocelicnega raka pljuč
Spanish	N-(bromoacetil)-3,3-dinitroazetidina	Carcinoma de pulmón de células pequeñas
Swedish	N-(bromacetyl)-3,3-dinitroazetidín	Behandling av småcellig lungcancer
Norwegian	N-(bromacetyl)-3,3-dinitroazetidín	Behandling av småcellet lungekreft
Icelandic	N-(brómóasetýl)-3,3-dínitróazetidín	Til meðferðar við lungnakrabbameini af smáfrumugerð

¹ At the time of designation