20 June 2016
EMA/COMP/303792/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Rovalpituzumab tesirine for the treatment of small cell lung cancer

On 30 May 2016, orphan designation (EU/3/16/1667) was granted by the European Commission to Aceso Biologics Consulting Ltd, United Kingdom, for rovalpituzumab tesirine 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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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 rovalpituzumab tesirine might be of significant benefit for patients with small cell lung cancer because early studies indicate that it has

*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 513,700,000 (Eurostat 2016).

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

improved anticancer effects compared with currently authorised medicines. 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?

Rovalpituzumab tesirine is made up of two components, which are linked together:

- a monoclonal antibody (a type of protein) that has been designed to recognise and attach to delta-like 3 (DLL3), a protein that is found on the surface of the cancerous cells in small cell lung cancer;
- a substance toxic for cells.

When injected into the patient, the medicine is expected to attach to the cancerous cells. The medicine is then expected to release the toxic component inside the cells, causing them to die.

What is the stage of development of this medicine?

The effects of rovalpituzumab tesirine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with rovalpituzumab tesirine in patients with small cell lung cancer were ongoing.

At the time of submission, rovalpituzumab tesirine was not authorised anywhere in the EU for small cell lung cancer. Orphan designation of the medicine has 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 21 April 2016 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Rovalpituzumab tesirine	Treatment of small cell lung cancer
Bulgarian	Ровалпитузумаб тесирин	Лечение на дребноклетъчен карцином на белия дроб
Croatian	Rovalpituzumab tesirin	Liječenje karcinoma pluća malih stanica
Czech	Rovalpituzumab teserin	Léčba malobuněčného karcinomu plic
Danish	Rovalpituzumab tesirin	Behandling af småcellet lungecancer
Dutch	Rovalpituzumab tesirine	Behandeling van kleincellig longcarcinoom
Estonian	Rovalpituzumab tesiriin	Väikeserakulise kopsuvähi ravi
Finnish	Rovalpitutsumabi tesiriini	Keuhkojen pienisolusyövän hoito
French	Rovalpituzumab tésirine	Traitement du cancer du poumon à petites cellules
German	Rovalpituzumab tesirine	Behandlung des kleinzelligen Lungenkarzinoms
Greek	Ροβαλπιτουζουμάμπη τεσιρίνη	Θεραπεία του μικροκυτταρικού καρκίνου του πνεύμονα
Hungarian	Rovalpituzumab tezirin	Kissejtes tüdőrák kezelése
Italian	Rovalpituzumab tesirina	Trattamento del cancro del polmone a piccole cellule (microcitoma)
Latvian	Rovalpituzumaba tesirīns	Sīkšūnu plaušu vēža ārstēšana
Lithuanian	Rovalpituzumabas tesirinas	Smulkialąstelinio plaučių vėžio gydymas
Maltese	Rovalpituzumab tesirine	Kura tal-kanċer tal-pulmun b'ċelloli żgħar
Polish	Rowalpituzumab tezyryna	Leczenie raka drobnokomórkowego pluc
Portuguese	Rovalpituzumab tesirina	Tratamento do carcinoma de pequenas células do pulmão
Romanian	Rovalpituzumab tesirin	Tratamentul cancerului pulmonar cu celule mici
Slovak	Rovalpituzumab tesirin	Liečba malobunkového karcinómu pľúc
Slovenian	Rovalpituzumab tesirin	Zdravljenje drobnocelicnega raka pljuc
Spanish	Rovalpituzumab tesirina	Carcinoma de pulmón de células pequeñas
Swedish	Rovalpituzumab tesirin	Behandling av småcellig lungcancer
Norwegian	Rovalpituzumab tesirine	Behandling av småcellet lungekreft
Icelandic	Róvalpítúzúmab tesírín	Til meðferðar við lungnakrabbameini af smáfrumugerð

[1] At the time of designation