

7 May 2018
EMA/112323/2018

Public summary of opinion on orphan designation

Tazemetostat for the treatment of malignant mesothelioma

On 21 March 2018, orphan designation (EU/3/18/2006) was granted by the European Commission to Quintiles Ireland Limited, Ireland, for tazemetostat for the treatment of malignant mesothelioma.

In May 2018, Quintiles Ireland Limited changed name to IQVIA RDS Ireland Limited.

What is malignant mesothelioma?

Malignant mesothelioma is a cancer that affects the mesothelial cells (found on the inner linings of the organs), mainly in the pleura (the lining of the lungs) and in the peritoneum (the lining of the abdominal cavity). It is usually caused by contact with asbestos. Mesothelioma of the pleura causes difficulty breathing and chest pain, and mesothelioma of the peritoneum causes ascites (a build-up of fluid in the abdomen) and abdominal pain.

Malignant mesothelioma is life-threatening because it may lead to heart or breathing problems, lung infections and bowel obstruction. Patients have very poor survival, only living for a year, on average, after diagnosis.

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

At the time of designation, malignant mesothelioma affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 52,000 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, the main treatments for malignant mesothelioma were surgery and chemotherapy (medicines to treat cancer) with or without radiotherapy (treatment with radiation). If the disease was too advanced for surgery, chemotherapy alone was used. One medicine, pemetrexed, was authorised throughout the EU for the treatment of malignant pleural mesothelioma in combination

*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 517,400,000 (Eurostat 2018).

with cisplatin. A second medicine, raltitrexed, was authorised for the treatment of malignant pleural mesothelioma in some European countries.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with malignant mesothelioma because early results from studies indicate that tazemetostat might stop the disease getting worse in patients whose disease has not responded to authorised medicines or has come back afterwards. 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?

Tazemetostat blocks the action of a protein called EZH2. Some patients with malignant mesothelioma produce large amounts of the EZH2 protein, which encourages abnormal growth and development of mesothelial cells. By blocking EZH2, tazemetostat is expected to lead to the death of the mesothelial cells and thus reduce the growth of the cancer.

What is the stage of development of this medicine?

The effects of tazemetostat have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with tazemetostat in patients with malignant mesothelioma were ongoing.

At the time of submission, tazemetostat was not authorised anywhere in the EU for malignant mesothelioma. Orphan designation of the medicine had been granted in the United States for mesothelioma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 February 2018 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	Tazemetostat	Treatment of malignant mesothelioma
Bulgarian	Таземетостат	Лечение на малигнен мезотелиом
Croatian	Tazemetostat	Liječenje malignog mezotelioma
Czech	Tazemetostat	Léčba maligního mezoteliomu
Danish	Tazemetostat	Behandling af malignt mesotheliom
Dutch	Tazemetostat	Behandeling van maligne mesotheliom
Estonian	Tasemetostaat	Pahaloomulise mesoteliomi ravi
Finnish	Tatsetmetostaatti	Malignin mesotelioman hoito
French	Tazemetostat	Traitement du mésothéliome malin
German	Tazemetostat	Behandlung des malignen Mesothelioms
Greek	Ταζεμετοστάτη	Θεραπεία κακοήθους μεσοθηλιώματος
Hungarian	Tazemetostat	Roszzindulatú mesothelioma kezelése
Italian	Tazemetostat	Trattamento del mesotelioma maligno
Latvian	Tazemetostats	Ļaundabīgas mezoteliomas ārstēšana
Lithuanian	Tazemetostatas	Piktybinės mezoteliomos gydymas
Maltese	Tażemetostat	Kura tal-mesoteljoma malinna
Polish	Tazemetostat	Leczenie złośliwego międzybłoniaka
Portuguese	Tazemetostate	Tratamento do Mesotelioma maligno
Romanian	Tazemetostat	Tratamentul mezoteliomului malign
Slovak	Tazemetostat	Liečba malígneho mezoteliómu
Slovenian	Tazemetostat	Zdravljenje malignega mezotelioma
Spanish	Tazemetostat	Tratamiento del mesotelioma maligno
Swedish	Tazemetostat	Behandling av malignt mesoteliom
Norwegian	Tazemetostat	Behandling av malignt mesoteliom
Icelandic	Tazemetóstat	Meðferð við illkynja miðþekjuæxli

¹ At the time of designation