10 February 2021
EMADOC-628903358-3458

Public summary of opinion on orphan designation

Tislelizumab for the treatment of oesophageal cancer

On 13 November 2020, orphan designation EU/3/20/2357 was granted by the European Commission to BeiGene Ireland Limited, Ireland, for tislelizumab (also known as BGB-A317) for the treatment of oesophageal cancer.

What is oesophageal cancer?

Oesophageal cancer is a type of cancer that starts in the oesophagus (foodpipe). Symptoms of the disease include difficulty swallowing, weight loss, symptoms of indigestion, nausea, vomiting, cough or hoarseness, pain in the throat and chest and bleeding. Oesophageal cancer spreads to other parts of the body in the majority of patients are diagnosed.

Oesophageal cancer is a debilitating and life-threatening condition because of its complications such as bleeding, pain and difficulty swallowing, and is associated with poor long-term survival.

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

At the time of designation, oesophageal cancer 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 cancer medicine 5-fluorouracil and photodynamic therapy (treatment using light) with the medicine porfimer sodium were authorised for treatment of oesophageal cancer in some countries in the European Union. Management of the condition included surgery, chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation). The effectiveness of these therapies in advanced oesophageal cancer was poor.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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The sponsor has provided sufficient information to show that tislelizumab might be of significant benefit for patients with oesophageal cancer because early results showed that the medicine could be beneficial in patients in whom the disease has come back or when other medicines are not effective.

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?

Tislelizumab is a monoclonal antibody (a type of protein) that has been designed to recognise and block a receptor (target) called PD-1 found on certain cells of the immune system. Some cancers make a protein that attaches to PD-1 and switches off the immune cells' ability to attack the cancer. By blocking PD-1, the medicine stops the cancer switching off these immune cells, thereby increasing the immune system's ability to kill the cancer cells.

What is the stage of development of this medicine?

The effects of tislelizumab have been evaluated in experimental models.

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

At the time of submission, tislelizumab was not authorised anywhere in the EU for the treatment of oesophageal cancer. Orphan designation of tislelizumab had been granted in the United States for oesophageal cancer.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 8 October 2020, 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

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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.