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Public summary of opinion on orphan designation

Retifanlimab for the treatment of anal cancer

On 19 October 2020, orphan designation EU/3/20/2343 was granted by the European Commission to Incyte Biosciences Distribution B.V, Netherlands, for retifanlimab for the treatment of anal cancer.

What is anal cancer?

Anal cancer is a cancer of the tissues of the anus. Bleeding from the anus is often the first sign of the disease. Other symptoms include pain, discomfort and itching and small lumps around the anus, difficulty controlling bowels (faecal incontinence) and ulcers. Sometimes the cancer causes no symptoms.

Anal cancer is a long-term debilitating disease due to its symptoms such as incontinence, bleeding, itching and non-healing ulcers. This disease is also life threatening and associated with shortened life expectancy.

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

At the time of designation, anal cancer affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 104,000 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, mitomycin was authorised for the treatment of anal cancer in the EU. Other treatments for anal cancer included radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with anal cancer because early studies found that the cancer shrank in patients

*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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whose cancer came back after treatment with platinum-based cancer 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?

Retifanlimab is a monoclonal antibody, a protein that has been designed to block a receptor ('target') called PD-1 found on certain cells of the immune system (the body's natural defences). Some cancers can make a protein that attaches to PD-1 and switches off the immune cells' ability to attack the cancer. By blocking PD-1, retifanlimab is expected to stop 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 retifanlimab have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 10 September 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.