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Public summary of opinion on orphan designation

Humanised IgG1K monoclonal antibody against interferon beta for the treatment of dermatomyositis

On 6 January 2021, orphan designation EU/3/20/2392 was granted by the European Commission to Pfizer Europe MA EEIG, Belgium, for humanised IgG1K monoclonal antibody against interferon beta (also known as PF-06823859) for the treatment of dermatomyositis.

What is dermatomyositis?

Dermatomyositis is an inflammatory disease of the muscles and the skin which causes muscle weakness and severe skin rash. Although skeletal muscle and skin problems are the most frequent signs of the disease, inflammation may also affect the muscles of the oesophagus (the passage from the mouth to the stomach), the lungs and the heart, leading to difficulties in eating and breathing.

Dermatomyositis is an auto-immune disease. This means that it is caused by the body's immune (defence) system attacking its own tissues. The reason why the immune system acts in this way is not known.

Dermatomyositis is a life-threatening and long-term debilitating condition due to the severe skin problems, muscle weakness and heart problems, and also because patients with the condition are at higher risk of developing cancer.

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

At the time of designation, dermatomyositis affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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).

* 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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What treatments are available?

At the time of designation, azathioprine and corticosteroid medicines were authorised for dermatomyositis in some EU countries.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with dermatomyositis because early studies showed that it may improve certain symptoms in patients who did not respond to other 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?

The medicine is a monoclonal antibody (a type of protein) that is designed to attach to an immune system protein called interferon beta. Patients with dermatomyositis have too much interferon beta, leading to inflammation. By binding to interferon beta, the medicine is expected to prevent its activity and improve the symptoms of the condition.

What is the stage of development of this medicine?

The effects of humanised IgG1K monoclonal antibody against interferon beta 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 dermatomyositis were ongoing.

At the time of submission, humanised IgG1K monoclonal antibody against interferon beta was not authorised anywhere in the EU for the treatment of dermatomyositis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 3 December 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.