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Public summary of opinion on orphan designation

Rilonacept for the treatment of idiopathic pericarditis

On 6 January 2021, orphan designation EU/3/20/2390 was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for rilonacept for the treatment of idiopathic pericarditis.

What is idiopathic pericarditis?

Patients with idiopathic pericarditis have inflammation of the pericardium (the membrane around the heart). Idiopathic means that the cause of the disease is not known. Signs and symptoms include chest pain, pericardial friction rub (the pericardium rubbing against the heart and producing an abnormal heart sound), changes in electrocardiogram (ECG, a test of the heart's electrical activity), and pericardial effusion (presence of fluid around the heart).

Idiopathic pericarditis is a long-term debilitating and life-threatening condition, because of persistent recurrences of the condition in some patients, the risk of cardiac tamponade (compression of the heart caused by fluid build-up), and constrictive pericarditis, which can lead to heart failure.

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

At the time of designation, idiopathic pericarditis affected approximately 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 130,000 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, treatments for pericarditis included aspirin and other non-steroidal anti-inflammatory drugs (NSAIDs) and colchicine. Surgery to remove a layer of the pericardium was sometimes used in patients for whom medicines did not work.

* 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 the medicine might be of significant benefit for patients with idiopathic pericarditis. Studies showed that patients treated with the medicine had fewer recurrences of the condition compared with those treated with 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?

This medicine blocks the action of interleukin-1 alpha (IL-1 alpha) and interleukin-1 beta (IL-1 beta), a chemical messenger in the body that are involved in producing inflammation in patients with idiopathic pericarditis. By blocking IL-1 alpha and IL-1 beta, the medicine is expected to reduce inflammation, relieve symptoms of the condition and prevent recurrences.

What is the stage of development of this medicine?

The effects of rilonacept have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with rilonacept in patients with idiopathic pericarditis had been conducted.

Rilonacept is authorised in the United States for the treatment of cryopyrin-associated periodic syndromes (CAPS) and deficiency of the interleukin-1 receptor antagonist (DIRA).

At the time of submission, rilonacept was not authorised anywhere in the EU for the treatment of idiopathic pericarditis. Orphan designation of rilonacept had been granted in the United States for the treatment of pericarditis.

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.