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Public summary of opinion on orphan designation

Celecoxib, ciprofloxacin for the treatment of amyotrophic lateral sclerosis

On 6 January 2021 orphan designation EU/3/20/2395 was granted by the European Commission to Morrison & Foerster, Belgium, for celecoxib, ciprofloxacin (also known as PrimeC) for the treatment of amyotrophic lateral sclerosis.

What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis (ALS) is a progressive disease of the nervous system, where nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate, causing loss of muscle function and paralysis. The exact causes are unknown but are believed to include genetic and environmental factors. The symptoms of ALS depend on which muscles weaken first, and include loss of balance, loss of control of hand and arm movement, and difficulty speaking, swallowing and breathing. ALS usually starts in midlife and men are more likely to develop the disease than women.

ALS is a debilitating and life-threatening disease because of the gradual loss of function and its paralysing effect on muscles used for breathing, which usually leads to death from respiratory failure.

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

At the time of designation, amyotrophic lateral sclerosis affected less than 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 62,000 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, riluzole was authorised in the EU to treat ALS. Patients also received supportive treatment, such as physiotherapy and breathing support, to relieve the symptoms of the disease.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with ALS. Studies showed that the medicine could slow down the disease in

*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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patients being treated with riluzole. 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 made up of two substances: the antibacterial ciprofloxacin and the anti-inflammatory celecoxib. Ciprofloxacin increases RNA interference, a biological process that switches off genes. This process is significantly reduced in ALS, which is thought to play a role in the damage to nerve cells. Celecoxib blocks COX-2, an enzyme that triggers pain, fever, and especially inflammation, which is central to the disease process of ALS. The two are expected to work in different ways to improve symptoms of the disease.

What is the stage of development of this medicine?

The effects of the medicine 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 amyotrophic lateral sclerosis were ongoing.

At the time of submission, celecoxib and ciprofloxacin were authorised in the EU as an anti-inflammatory and as an antibacterial, respectively.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of amyotrophic lateral sclerosis. Orphan designation had been granted in the United States 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.