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Public summary of opinion on orphan designation

Rezafungin acetate for the treatment of invasive candidiasis

On 6 January 2021, orphan designation EU/3/20/2385 was granted by the European Commission to Mundipharma Corporation (Ireland) Limited, Ireland, for rezafungin acetate for the treatment of invasive candidiasis.

What is invasive candidiasis?

Invasive candidiasis is an infection caused by yeast called Candida that has spread widely in the body and may also be present in blood. The infection generally occurs in patients whose immune system (the body's natural defences) has been weakened or when damage in body tissues allows the infection to spread.

Invasive candidiasis can cause fever and chills which do not improve with antibiotics. The infection may cause the patient to go into shock with low blood pressure, racing heartbeat and rapid breathing. Spread of the infection can damage organs such as kidneys, heart, liver, spleen, lungs, eyes and brain.

Invasive candidiasis is a life-threatening disease that can be fatal due to damage to vital organs.

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

At the time of designation, invasive candidiasis affected approximately 1.2 people in 10,000 per year in the European Union (EU). This was equivalent to a total of around 62,000 people* per year and is below the ceiling for orphan designation. 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 submission of the application for orphan drug designation, several medicines, including amphotericin B, azole medicines (such as fluconazole, itraconazone, posaconazole and voriconazole) and echinocandin medicines (such as anidulafungin, caspofungin and micafungin) were authorised for the treatment of invasive candidiasis.

*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 invasive candidiasis because treatment with the medicine was more successful than with caspofungin, a medicine considered more effective and safer than other authorised treatments. 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?

Rezafungin acetate belongs to the group of antifungal medicines called echinocandins. It interferes with the production of an essential component of the Candida cell wall called BDG. This causes the yeast cells to be incomplete or have defective cell walls. Treatment with rezafungin acetate is expected to make Candida cells fragile and unable to grow and so bring invasive candidiasis under control and reduce damage from the disease.

What is the stage of development of this medicine?

The effects of rezafungin acetate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with rezafungin acetate in patients with invasive candidiasis were ongoing.

At the time of submission, rezafungin acetate was not authorised anywhere in the EU for the treatment of invasive candidiasis. Orphan designation of the medicine had been granted in the United States for invasive candidiasis and candidaemia.

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.