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Public summary of opinion on orphan designation

Sotatercept for the treatment of pulmonary arterial hypertension

On 9 December 2020, orphan designation EU/3/20/2369 was granted by the European Commission to IDEA Innovative Drug European Associates (Ireland) Limited, Ireland, for sotatercept for the treatment of pulmonary arterial hypertension.

What is pulmonary arterial hypertension?

Pulmonary arterial hypertension is a condition in which patients have abnormally high blood pressure in the arteries in the lungs. In patients with this condition, the walls of the arteries that carry blood to the lungs become thick and stiff. This narrows the space through which the blood flows, making it harder for the heart to pump blood into the lungs.

Pulmonary arterial hypertension is a long-term debilitating and life-threatening condition that shortens patients' life expectancy because it may lead to breathing difficulty and heart failure.

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

At the time of designation, pulmonary arterial hypertension affected approximately 1.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 73,000 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?

Several medicines were authorised for the treatment of pulmonary arterial hypertension in the EU at the time of designation. They included ambrisentan, bosentan, epoprostenol, iloprost, macitentan, riociguat, selexipag, sildenafil, tadalafil and treprostinil. Some patients also underwent lung transplantation or atrial septostomy (an operation to create a small hole between the upper two chambers of the heart, the atria).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with pulmonary arterial hypertension. Early data indicate that adding the medicine

*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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to patients' standard treatment could improve the distance patients can walk in a given time. 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?

In the body, proteins called activins attach to a receptor (target) called ActRIIA to stimulate the growth of cells that make up the blood vessels. These receptors are thought to be over-active in patients with pulmonary arterial hypertension. This medicine is a copy of ActRIIA, and because it also attaches to activins, it prevents them from activating the receptor. In this way, the medicine is expected to reduce the growth of new blood vessel cells, leading to reduced narrowing and thickening of the blood vessels, thus improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of sotatercept have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with sotatercept in patients with pulmonary arterial hypertension were ongoing.

At the time of submission, sotatercept was not authorised anywhere in the EU for the treatment of pulmonary arterial hypertension. The medicine was designated as an orphan medicinal product in the United States for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 5 November 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.