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Public summary of opinion on orphan designation

Perflubron for the treatment of respiratory distress syndrome

On 9 December 2020, orphan designation EU/3/20/2383 was granted by the European Commission to Boyd Consultants Limited, Ireland, for perflubron for the treatment of respiratory distress syndrome.

What is respiratory distress syndrome?

Respiratory distress syndrome (RDS) is a lung disorder that affects newborns, especially those born prematurely. RDS causes difficulty in breathing soon after birth because the lungs are not fully developed and do not produce enough surfactant (a substance that helps keep the lungs inflated and prevents them from collapsing).

RDS is a debilitating condition that may be life-threatening because it can cause lung problems and a lack of oxygen supply to the brain, resulting in long-term developmental disabilities.

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

At the time of designation, respiratory distress syndrome affected approximately 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 130,000 people*, which was considered to be 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). 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, several surfactant replacement medicines were authorised for RDS in some countries in the EU. Treatment for RDS also involved oxygen therapy as well as intubation and mechanical ventilation. Before birth, the mother may also be given steroids to stimulate the unborn baby's lung development.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with respiratory distress syndrome because laboratory studies show that perflubron

*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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used together with surfactants may improve the intake of oxygen, compared with the use of surfactant alone.

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?

Perflubron is a liquid that is delivered into the patient's lung through a small tube inserted in the patient's airways. The medicine is expected to help the lung gently expand and take up oxygen into the bloodstream. This should help the lung to develop and work more normally and reduce inflammation and damage to the lung tissue caused by mechanical ventilation.

What is the stage of development of this medicine?

The effects of perflubron 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 respiratory distress syndrome were ongoing.

At the time of submission, perflubron was not authorised anywhere in the EU for the treatment of respiratory distress syndrome. Orphan designation of perflubron had been granted in the EU for congenital pulmonary hypoplasia and in the United States for congenital pulmonary hypoplasia in infancy and respiratory distress syndrome.

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.