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Public summary of opinion on orphan designation

Perflubron for the treatment of congenital pulmonary hypoplasia

On 13 November 2020, orphan designation EU/3/20/2361 was granted by the European Commission to Boyd Consultants Limited, Ireland, for perflubron for the treatment of congenital pulmonary hypoplasia.

What is congenital pulmonary hypoplasia?

Congenital pulmonary hypoplasia is a condition where one or both the lungs are underdeveloped at birth, causing breathing difficulties and, in more severe cases, respiratory distress with rapid breathing and cyanosis (blue colouration of the skin due to a lack of oxygen in the blood). It is usually associated with other conditions that affect lung development.

Congenital pulmonary hypoplasia is a debilitating disease that is long lasting and may be life-threatening because it causes respiratory distress and has a poor survival rate in severe cases. In less severe cases it causes chronic (long-term) lung problems, leading to reduced exercise tolerance and recurrent chest infections.

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

At the time of designation, congenital pulmonary hypoplasia affected approximately 0.17 in 10,000 people in the European Union (EU). This was equivalent to a total of around 9,000 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, no medicines were authorised for the condition in the EU. Treatment for congenital pulmonary hypoplasia involved intubation with mechanical ventilation, as well as inhaled nitric oxide and extracorporeal membrane oxygenation (ECMO, a technique to oxygenate the blood outside the body using a device similar to a heart-lung machine).

*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 congenital pulmonary hypoplasia because early studies show that the medicine might stimulate lung development and remove the need or reduce duration of mechanical ventilation or ECMO.

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. Used together with mechanical ventilation, the medicine is expected to help the lung gently expand and take up oxygen into the bloodstream, thereby helping the lung to work and develop more normally.

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 congenital pulmonary hypoplasia had finished.

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 8 October 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.