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Public summary of opinion on orphan designation

Sildenafil for the treatment of congenital diaphragmatic hernia

On 20 June 2017, orphan designation (EU/3/17/1885) was granted by the European Commission to Avivia Beheer BV, the Netherlands, for sildenafil for the treatment of congenital diaphragmatic hernia.

What is congenital diaphragmatic hernia?

Congenital diaphragmatic hernia is a birth defect in which the contents of a baby's abdomen (such as parts of the intestines, stomach and liver) move up into the chest through the diaphragm, the thin muscle that separates the chest from the abdomen.

In babies with the condition, the lungs may not function or develop properly, resulting in complications including pulmonary hypertension (high blood pressure in the lungs).

In severe cases, the condition may be long-term debilitating and life-threatening because of its effects on the lungs and heart.

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

At the time of designation, congenital diaphragmatic hernia affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 67,000 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 the orphan designation, there were no satisfactory treatments authorised in the EU for treating patients with congenital diaphragmatic hernia. Several medicines were authorised for treating pulmonary hypertension.

*Disclaimer: 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 (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).

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How is this medicine expected to work?

Sildenafil is expected to be given during pregnancy to mothers carrying babies with congenital diaphragmatic hernia. It works by blocking an enzyme (called PDE5) in the blood vessels of the lungs. When this enzyme is blocked, the walls of the blood vessels are expected to relax and the blood vessels to widen, thereby preventing blood pressure in the lungs from becoming too high after birth.

Sildenafil is already authorised in the EU for treating pulmonary hypertension after birth.

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, no clinical trials with sildenafil in patients with congenital diaphragmatic hernia had been started.

At the time of submission, sildenafil was not authorised anywhere in the EU for the treatment of congenital diaphragmatic hernia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 May 2017 recommending the granting of this designation.

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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

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sildenafil	Treatment of congenital diaphragmatic hernia
Bulgarian	Силденафил	Лечение на вродена диафрагмална херния
Croatian	Sildenafil	Liječenje kongenitalne dijagfragmalne hernije
Czech	Sildenafili	Léčba kongenitální diafragmatické hernie
Danish	Sildenafil	Behandling af medfødt diaphragma hernie
Dutch	Sildenafil	Behandeling van congenitaal hernia diaphragmatica
Estonian	Sildenafiil	Kaasasündinud diafragmaalsonga ravi
Finnish	Sildenafiili	Synnynnäisen palleatyrän hoito
French	Sildénafil	Traitement de la hernie diaphragmatique congénitale
German	Sildenafil	Behandlung von angeborenen diaphragmalen Hernien
Greek	Σιλντεναφίλη	Θεραπεία συγγενούς διαγραγματοκήλης
Hungarian	Szildenafil	Veleszületett rekeszizomsérv kezelése
Italian	Sildenafil	Trattamento dell'ernia diaframmatica congenita
Latvian	Sildenafils	Iedzimtas diafragmas trūces ārstēšana
Lithuanian	Sildenafilis	Įgimtos diafragmos išvaržos gydymas
Maltese	Sildenafil	Kura ta' ftuq dijaframmatiku konġenitali
Polish	Syldenafil	Leczenie wrodzonej przepukliny przeponowej
Portuguese	Sildenafil	Tratamento da hérnia diafragmática congénita
Romanian	Sildenafil	Tratamentul herniei diafragmatice congenitale
Slovak	Sildenafil	Liečba kongenitálnej diafragmatickej hernie.
Slovenian	Sildenafil	Zdravljenje prirojene diafragmalne hernije
Spanish	Sildenafilo	Tratamiento de la hernia diafragmatica congenital
Swedish	Sildenafil	Behandling av kongenitalt diafragmabråck
Norwegian	Sildenafil	Behandling av medfødt diafragmahernie
Icelandic	Síldenafíl	Meðferð á meðfæddu þindar kviðsliti

[1] At the time of designation