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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF

sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleyl phosphatidylglycerol and palmitic acid for the treatment of meconium aspiration syndrome

On 19 September 2001, orphan designation (EU/3/01/054) was granted by the European Commission to Discovery Laboratories Inc., United Kingdom, for sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleyl phosphatidylglycerol and palmitic acid (Surfaxin) for the treatment of meconium aspiration syndrome.

What is meconium aspiration syndrome?

Meconium aspiration syndrome is a condition that affects newborns in consequence of inhalation of meconium (baby's first feces, ordinarily passed after birth) during delivery. Affected newborns develop respiratory distress (increasing difficulty in breathing) soon after birth. In brief, tiny air sacs called alveoli are located at the tips of the lungs' smallest breathing tubes, called bronchi. The alveoli are responsible for passing oxygen into the blood. When meconium is aspirated in the lungs, oxygen cannot enter the alveoli, which means that oxygen is no longer getting into the blood. Because the lungs are inflamed and filled with fluid, it is increasingly difficult to take breaths. Furthermore, the inflammation in the lungs leads to scarring. The lungs eventually become stiff with scar tissue and breathing becomes very difficult. Meconium aspiration syndrome is potentially chronically debilitating and life threatening.

What are the methods of treatment available?

At the time of submission of the application for orphan drug designation, no authorised medicinal products were available in the European Union. However, several therapeutic methods are available such as mechanical breathing assistance and/or removal of meconium through suction of the area of the throat at the back of the mouth and the windpipe.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleyl phosphatidylglycerol and palmitic acid might be of significant benefit for the treatment of meconium aspiration syndrome. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, meconium aspiration syndrome was considered to affect about 9,400 persons in the European Union.

How is this medicinal product expected to act?

Lung surfactant is a protein and fat complex that coats and keeps open the alveoli of the lung, thus ensuring a stable surface for oxygen passage. Surfaxin is a synthetic lung surfactant and the sponsor's hypothesis is that it will ultimately help the patient to take breaths more easily.

What is the stage of development of this medicinal product?

The effects of sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleyl phosphatidylglycerol and palmitic acid were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with meconium aspiration syndrome were ongoing.

Sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleyl phosphatidylglycerol and palmitic acid was not marketed anywhere worldwide for treatment of meconium aspiration syndrome, at the time of submission. Orphan designation of sinapultide, dipalmitoylphosphatidylcholine, palmitoyl-oleyl phosphatidylglycerol and palmitic acid was granted in United States for treatment of meconium aspiration syndrome in newborn infants, treatment of acute respiratory distress syndrome in adults and in premature infants.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 18 July 2001 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyloleoyl phosphatidylglycerol and palmitic acid	Treatment of Meconium Aspiration Syndrome
Danish	Sinapultid Dipalmitoylphosphatidylcholin, palmitoyloleoyl phosphatidylglycerol og palmitinsyre	Behandling af Meconium Aspiration Syndrome
Dutch	Sinapultide dipalmitoylphosphatidylcholine, palmitoyloleoyl phosphatidylglycerol en palmitinezuur	Behandeling van Meconium Aspiratie Syndroom
Finnish	Sinapultiidi Dipalmitolifosfatidylkoliini, palmitoliroleoli fosfatidylglyseroli ja palmitiinihappo	Mekoniumaspiraatio-oireyhtymän hoito
French	Sinapultide, dipalmitoylphosphatidylcholine, palmitoyloleoylphosphatidylglycerol et acide palmitique	Traitement de l'inhalation méconiale
German	Sinapultide Dipalmitoylphosphatidylcholin, Palmitoyloleoylphosphatidylglycerol und Palmitinsäure	Behandlung des Mekonium Aspirationssyndroms
Greek	Σιναπουλτιδη, διοφωσφατιδυλοχολίνη, παλμιτοϋλική-ελαιοϋλική φωσφατιδυλογλυκερόλη και παλμιτικό οξύ	Θεραπεία συνδρόμου εισροφήσεως μυκωνίου
Italian	Sinapultide, dipalmitoilfosfatidilcolina, palmitoiloleoil fosfatidilglicerolo e acido palmitico	Trattamento della Sindrome da Aspirazione del Meconio
Portuguese	Sinapultide, dipalmitoilfosfatidilcolina, palmitoiloleoilfosfatidilglicerol e ácido palmitico	Tratamento do síndrome de aspiração do mecónio
Spanish	Sinapultida, dipalmitoilfosfatidilcolina, palmitoiloleoilfosfatidilglicerol y ácido palmítico	Tratamiento del Síndrome de Aspiración del Meconio
Swedish	Sinapultid, Dipalmitylphosphatidylkolin, palmityloleoylphosphatidylkolin och palmitinsyra	Behandling av mekoniumaspirationssyndrom