



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Dipalmitoylphosphatidylcholine, 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol, sodium salt, synthetic surfactant protein C analogue and synthetic surfactant protein B analogue for the treatment of respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age

On 2 April 2012, orphan designation (EU/3/12/982) was granted by the European Commission to Chiesi Farmaceutici S.P.A., Italy, for dipalmitoylphosphatidylcholine, 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol, sodium salt, synthetic surfactant protein C analogue and synthetic surfactant protein B analogue, for the treatment of respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age.

What is respiratory distress syndrome in premature neonates?

Respiratory distress syndrome in premature neonates (newborn babies) is a breathing disorder in which the baby's immature lungs are not producing enough surfactant. Surfactant is a substance lining the airways that allows the lungs to expand properly and is required in sufficient amounts for the newborn to start breathing after birth. Premature neonates with respiratory distress syndrome have problems breathing and require extra oxygen within minutes of being born.

Respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age is a long-term and life-threatening condition because of the damage to the brain due to the lack of oxygen, and because of other complications resulting from treatment and mechanical ventilation (using a machine to help them to breathe).

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

At the time of designation, respiratory distress syndrome in premature neonates of less than 37 weeks gestational age affected approximately 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 101,000 people, and is below the ceiling for orphan designation, which

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).



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, several surfactant products were authorised for the condition in the EU. Other methods of treatment included the use of mechanical ventilation.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for premature neonates with respiratory distress syndrome because early studies in experimental models indicate that it may act for longer than other surfactants and may improve the treatment of patients with this condition. 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?

This medicine is a synthetic surfactant designed to be very similar to natural surfactant. The medicine is expected to work by replacing the missing surfactant in the lungs of premature infants with respiratory distress syndrome. This will improve the child's lung function by keeping the airways open and allowing sufficient oxygen to be taken up.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in premature neonates with respiratory distress syndrome had been started.

At the time of designation, the medicine was not authorised anywhere in the EU for respiratory distress syndrome in premature neonates. Orphan designation of this medicine had been granted in the United States of America for the treatment of preterm neonatal respiratory distress syndrome.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 February 2012 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

Sponsor's contact details:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Dipalmitoylphosphatidylcholine, 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol, sodium salt, synthetic surfactant protein C analogue and synthetic surfactant protein B analogue	Treatment of respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age
Bulgarian	Дипалмитоилфосфатидилхолин, 1-палмитоил-2-олеоил-sn-глицеро-3-фосфоглицерол, натриева сол, синтетичен сърфактант протеин C аналог и синтетичен сърфактант протеин B аналог	Лечение на респираторен дистрес синдром при недоносени новородени, с гестационна възраст по-малка от 37 седмици
Czech	Dipalmitoylfosfatidylcholin, 1-palmitoyl-2-oleoyl-sn-glycero-3-fosfoglycerát sodný, syntetický surfaktant analogu proteinu C a analogu proteinu B	Léčba syndromu dechové tísně u předčasně narozených novorozenců před 37. týdnem gestačního věku
Danish	Dipalmitoylfosfatidylcholin, 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol, natriumsalt, syntetisk surfaktant protein C-analog og syntetisk surfaktant protein B-analog.	Behandling af respiratory distress syndrom hos for tidligt fødte med en gestationsalder mindre end 37 uger
Dutch	Dipalmitoylfosfatidylcholine, 1-palmitoyl-2-oleoyl-sn-glycero-3-fosfoglycerol, natriumzout, synthetisch surfactant-proteïne C-analoog en synthetisch surfactant-proteïne B-analoog.	Behandeling van Respiratoir Distress Syndroom in premature pasgeborenen met een draagtijd van minder dan 37 weken
Estonian	Dipalmitoüülfosfatidüülkoliin, 1-palmitoüül-2-oleoüül-sn-glütsero-3-fosfoglütserool, naatriumsool, sünteetilise surfaktant proteiin C analoog ja sünteetilise surfaktant proteiin B analoog.	Enne 37 rasedusnädalat sündinud enneagse vastsündinu respiratoorse distress sündroomi ravi
Finnish	Dipalmitoyylifosfatidyylikoliinin, 1-palmitoyyli-2-oleoyyli-sn-glysero-3-fosfoglyserolin, natriumsuolan, synteettisen surfaktanttiproteiini C:n analogin ja synteettisen surfaktanttiproteiini B:n analogin yhdistelmä	Ennen 37. raskausviikkoa syntyneiden keskosten hengitysvaikeusoireyhtymän hoito
French	Dipalmitoylfosfatidylcholine, 1-palmitoyl-2-oléoyl-sn-glycéro-3-	Traitement du syndrome de détresse respiratoire du nouveau-né prématuré âgé

¹ At the time of designation

Language	Active ingredient	Indication
	phosphoglycérol, sel de sodium, analogue synthétique des protéines C et B du surfactant (PS-C, PS-B)	de moins de 37 semaines
German	Dipalmitoylphosphatidylcholin, 1-Palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol, Natriumsalz, synthetisches Surfactant Protein C-Analog und synthetisches Surfactant Protein B-Analog.	Behandlung des respiratorischen Stress-Syndroms in Frühgeborenen mit einem Gestationsalter unter 37 Wochen
Greek	Διπαλμιτοΐλοφωσφατιδυλοχολίνη, 1-παλμιτοΐλ-2-ελαιοΐλ-sn-γλυκερο-3-φωσφογλυκερόλη, άλας νατρίου, συνθετικό ανάλογο επιφανειοδραστικής πρωτεΐνης C και συνθετικό ανάλογο επιφανειοδραστικής πρωτεΐνης B.	Αντιμετώπιση του Συνδρόμου Αναπνευστικής Δυσχέρειας σε πρόωρα νεογνά ηλικίας κύησης μικρότερης από 37 εβδομάδες.
Hungarian	Dipalmitoil-foszfátidil-kolin, 1-palmitoil-2-oleoil-sn-glycero-3-phosphoglycerol, nátrium só, szintetikus, felületaktív Protein C analóg és szintetikus, felületaktív Protein B analóg.	Respirációs distress szindróma kezelése koraszülötteknél 37 hétnél rövidebb gesztációs idő esetén
Italian	Dipalmitoilfosfatidilcolina, 1-palmitoil-2-oleoil-sn-glicero-3-fosfoglicerolo, sale di sodio, analogo sintetico della proteina C del surfattante e analogo sintetico della proteina B del surfattante.	Terapia della Sindrome da Distress Respiratorio nei neonati prematuri di meno di 37 settimane di età gestazionale
Latvian	Dipalmitoilfosfatidilholīns, 1-palmitoil-2-oleoil-sn-glicero-3-fosfoglicerola nātrija sāļš, sintētisks surfaktanta C proteīna analogs un sintētisks surfaktanta B proteīna analogs	Respiratorā distresa sindroma ārstēšana priekšlaicīgi dzimušiem jaundzimušajiem ar gestācijas vecumu mazāku par 37 nedēļām
Lithuanian	Dipalmitoilfosfatidilcholinai, 1-palmitoil-2-oleoil-sn-glicero-3-fosfoglicerolis, natrio druska, sintetinis surfaktanto C baltymo analogas ir sintetinis surfaktanto B baltymo analogas	Mažesnių nei 37 gestacijos savaitių neišnešiotų naujagimių respiracinio distreso sindromo gydymas
Maltese	Dipalmitoylphosphatidylcholine, 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol, melh tas-sodium, analogu sintetiku ta' Proteina C tas-Surfactant u analogu sintetiku ta' Proteina B tas-Surfactant.	Treatment of respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age
Polish	Dipalmitoilofosfatydylocholina, sól sodowa 1-palmitoil-2-oleoil-sn-glicero-3-fosfoglicerolu, syntetyczny analog białka C surfaktantu i syntetyczny analog białka B surfaktantu	Leczenie zespołu zaburzeń oddechowych u wcześniaków urodzonych przed 37 tygodniem ciąży
Portuguese	Dipalmitoilfosfatidilcolina, 1-palmitoil-2-oleoil-sn-glicero-3-fosfoglicerol, sal de	Tratamento do síndrome de dificuldade respiratória em recém nascidos prematuros

Language	Active ingredient	Indication
	sódio, surfactante sintético análogo da proteína C e surfactante sintético análogo da proteína B	com menos de 37 semanas de gestação
Romanian	Dipalmitoilfosfatidilcolină, 1-palmitoil-2-oleoil-sn-glicero-3-fosfoglicerol, sare sodică, analog sintetic de proteină C a surfactantului și analog sintetic de proteină B a surfactantului	Tratamentul sindromului de detresă respiratorie a nou-născutului prematur cu vârsta gestațională de sub 37 de săptămâni
Slovak	Sodná soľ dipalmitoylfosfatidylcholín, 1-palmitoyl-2-oleoyl-sn-glycero-3-fosfoglycerolu, syntetický analóg proteínu C surfaktantu a syntetický analóg proteínu B surfaktantu	Liečba syndrómu dychovej tiesne u predčasne narodených novorodencov gestačným vekom menej ako 37 týždňov
Slovenian	Dipalmitoil fosfatidilholin, 1-palmitoil-2-oleoil-sn-glicero-3-fosfoglicerol, natrijeva sol, sintetični analog surfaktanta proteina C in sintetični analog surfaktanta proteina B	Zdravljenje sindroma dihalne stiske novorojenčkov pri nedonošenčkih gestacijske starosti manj kot 37 tednov
Spanish	Dipalmitoilfosfatidilcolina, 1-palmitoil-2-oleoil-sn-glicero-3-fosfoglicerol, sal sódica, análogo de la proteína C tensioactiva sintética y análogo de la proteína B tensioactiva sintética.	Tratamiento del síndrome de distrés respiratorio en neonatos prematuros de menos de 37 semanas de edad gestacional
Swedish	Dipalmitylfosfatidylkolin, 1-palmitoyl-2-oleoyl-sn-glycero-3-fosfoglycerol, natriumsalt, syntetisk surfaktant protein C-analog och syntetisk surfaktant protein B-analog	Behandling av Respiratory Distress Syndrome hos prematurt nyfödda barn med en fostertidslängd av mindre än 37 veckor
Norwegian	Dipalmitoylfosfatidylkolin, 1-palmitoyl-2-oleoyl-sn-glysero-3-fosfoglyserol, natriumsalt, syntetisk overflateprotein C-analog og syntetisk overflateprotein B-analog.	Behandling av respiratorisk distress-syndrom hos premature nyfødte med gestasjonsalder under 37 uker
Icelandic	Dípalmítóýl phosfatatdýlkólín 1-palmítóýl-2-sn-glýceró-3-phosfóglýceról natríum salt tilbúið yfirborðsvirkt protein B hliðstæða	Meðferð öndunarnauðar heilkennis hjá fyrirburum eftir minna en 37 vikna meðgöngu