EMA/788583/2016

European Medicines Agency decision

P/0363/2016

of 21 December 2016

on the agreement of a paediatric investigation plan and on the granting of a waiver for dipalmitoylphosphatidylcholine / 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol sodium salt / (EMEA-001780-PIP01-15) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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on the agreement of a paediatric investigation plan and on the granting of a waiver for dipalmitoylphosphatidylcholine / 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol sodium salt /synthetic surfactant protein C analogue / synthetic surfactant protein B analogue (CHF 5633) (EMEA-001780-PIP01-15) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Chiesi Farmaceutici SpA on 25 January 2016 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 November 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 13 of said Regulation,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the agreement of a paediatric investigation plan and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a waiver.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for dipalmitoylphosphatidylcholine / 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol sodium salt /synthetic surfactant protein C analogue / synthetic surfactant protein B analogue (CHF 5633), endotracheopulmonary instillation, suspension, endotracheopulmonary use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A waiver for dipalmitoylphosphatidylcholine / 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol sodium salt /synthetic surfactant protein C analogue / synthetic surfactant protein B analogue (CHF 5633), endotracheopulmonary instillation, suspension, endotracheopulmonary use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Chiesi Farmaceutici SpA, Via Palermo 26/A, 43122 - Parma, Italy.

EMA/PDCO/157006/2016
London, 11 November 2016

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMEA-001780-PIP01-15

Scope of the application

Active substance(s):

Dipalmitoylphosphatidylcholine / 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol sodium salt / synthetic surfactant protein C analogue / synthetic surfactant protein B analogue (CHF 5633)

Condition(s):

Treatment of respiratory distress syndrome

Pharmaceutical form(s):

Endotracheopulmonary instillation, suspension

Route(s) of administration:

Endotracheopulmonary use

Name/corporate name of the PIP applicant:

Chiesi Farmaceutici SpA

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Chiesi Farmaceutici SpA submitted for agreement to the European Medicines Agency on 25 January 2016 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 1 March 2016.

Supplementary information was provided by the applicant on 4 August 2016. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of respiratory distress syndrome

The waiver applies to:

- the paediatric population from 37 weeks gestational age to less than 18 years
- endotracheopulmonary instillation, suspension, endotracheopulmonary use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of respiratory distress syndrome (RDS)

2.1.1. Indication(s) targeted by the PIP

Treatment and early treatment of respiratory distress syndrome (RDS) in preterm neonates below 37 weeks of gestational age

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

Pre-term neonates from birth to less than 37 weeks gestational age

2.1.3. Pharmaceutical form(s)

Endotracheopulmonary instillation, suspension

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1 Development of a sterile suspension pharmaceutical form suitable for the administration to preterm neonates via endotracheopulmonary instillation.
Non-clinical studies	0	Not applicable.
Clinical studies	3	Study 2 Multicentre, open label single-escalating dose, per-cohort design study to evaluate safety, tolerability and activity of CHF 5633 in preterm neonates with RDS with a long-term follow-up at a corrected

		age of 24 months. (FIH, first in human study) Study 3 Multicentre, double blind, randomized, single dose, active-controlled study to evaluate efficacy, safety and tolerability of CHF 5633 versus porcine surfactant (Poractant Alfa, Curosurf) to treat respiratory distress syndrome in preterm neonates from 24+0 to 29+6 weeks gestational age with a long-term follow-up at a corrected age of 24 months. (POC, proof of concept study) Study 4 Multicentre, double blind, randomized, non-inferiority, active-controlled study to evaluate efficacy, safety and tolerability of CHF 5633 versus porcine surfactant (Poractant Alfa, Curosurf) to treat respiratory distress syndrome in preterm neonates from 24+0 to 32+6 weeks gestational age. (Pivotal study)
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By July 2022
Deferral for one or more measures contained in the paediatric investigation plan:	No