EMA/86893/2020

European Medicines Agency decision

P/0066/2020

of 28 February 2020

on the agreement of a paediatric investigation plan and on the granting of a waiver for mecasermin rinfabate (EMEA-000534-PIP03-17) 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.

European Medicines Agency decision

P/0066/2020

of 28 February 2020

on the agreement of a paediatric investigation plan and on the granting of a waiver for mecasermin rinfabate (EMEA-000534-PIP03-17) 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 Premacure AB, a member of the Shire group of companies on 12 June 2017 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 December 2019, in accordance with Article 17 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 mecasermin rinfabate, solution for infusion, intravenous 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 mecasermin rinfabate, solution for infusion, intravenous 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 Premacure AB, a member of the Shire group of companies, 1, rue Hildegard von Bingen, L-1282, Luxembourg, Luxembourg.

EMA/PDCO/550825/2019
Amsterdam, 11 December 2019

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

EMEA-000534-PIP03-17

Scope of the application

Active substance(s):

Mecasermin rinfabate

Condition(s):

Prevention of chronic lung disease of prematurity

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Premacure AB, a member of the Shire group of companies

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Premacure AB, a member of the Shire group of companies submitted for agreement to the European Medicines Agency on 12 June 2017 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 18 July 2017.

Supplementary information was provided by the applicant on 9 September 2019.

A meeting with the Paediatric Committee took place on 10 December 2019.

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 17(1) 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)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population, and 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 subsets 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:

Prevention of chronic lung disease of prematurity

The waiver applies to:

- the paediatric population of preterm infants from 28 weeks to 36 weeks gestational age;
- solution for infusion; intravenous use;
- on the grounds that the specific medicinal product is likely to be ineffective.

and

- the paediatric population from 36 weeks gestational age to less than 18 years of age;
- solution for infusion; intravenous use;
- on the grounds that the condition for which the specific medicinal product is intended does not occur in the specified subsets of the paediatric population.

2. Paediatric investigation plan

2.1. Condition:

Prevention of chronic lung disease of prematurity

2.1.1. Indication(s) targeted by the PIP

Prevention of chronic lung disease of prematurity

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

From birth to less than 28 weeks gestational age

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	2	Study 1 (Study Identifier not yet available) Compatibility of an age-appropriate dosage form for parenteral use of mecasermin rinfabate with infusion set-ups Study 2 (Study Identifier not yet available) Compatibility of an age-appropriate dosage form for parenteral use of mecasermin rinfabate with commonly used medicines in the neonatal environment that are co-administered through a terminal injection site.

Non-clinical studies	4	Study 3 (N10836M-SHP607) Juvenile animal study to assess the effect of a 3-day intravenous infusion of mecasermin rinfabate in a model of premature birth in sheep exposed to 3 days of invasive mechanical ventilation. Study 4 (R11067M-SHP607) Juvenile animal study to investigate the effect of different doses of mecasermin rinfabate on lung structure and function in a rodent model of bronchopulmonary dysplasia (BPD) induced by chorioamnionitis (CA) or preeclampsia (PE). Study 5 (R11572M-SHP607) Juvenile animal study to evaluate the effect of mecasermin rinfabate on neonatal lung structure and function in a rodent model of bronchopulmonary dysplasia (BPD) induced by hyperoxia. Study 6 (Study Identifier not yet available) Juvenile animal study to assess the effect of a 7-day intravenous (IV) infusion of mecasermin rinfabate in a model of premature birth in sheep exposed to 7 days of invasive mechanical ventilation followed by 3 days of non-invasive ventilation.
Clinical studies	3	*Study 7 (SHP607-201)* Multicentre open-label non-interventional follow-up study to evaluate long-term efficacy (assessed by retinopathy of prematurity (ROP)-associated visual outcomes) and safety following short-term exposure to mecasermin rinfabate versus standard neonatal care in Study ROPP-2008-01 on retinopathy of prematurity (Section D). *Main objective of this study to the purpose of this PIP is to collect safety data to support the development of mecasermin rinfabate in the treatment of chronic lung disease of prematurity.* Study 8 (SHP607-202) Multicentre, randomized, controlled study to evaluate the effect of 2 doses of mecasermin rinfabate compared to standard of care in time to final weaning off respiratory technology support (RTS) through 12 months corrected age (CA) in preterm infants. Study 9 (SHP607-30X) Multicentre, open-label, randomized, controlled study to evaluate efficacy and safety of mecasermin rinfabate compared to standard neonatal care on the time to final weaning off respiratory technology support (RTS) through 12 months corrected age (CA) in preterm infants.
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:	Yes
Date of completion of the paediatric investigation plan:	By October 2026
Deferral for one or more measures contained in the paediatric investigation plan:	No