EMA/251730/2024

European Medicines Agency decision

P/0187/2024

of 14 June 2024

on the acceptance of a modification of an agreed paediatric investigation plan for mecasermin rinfabate (EMEA-000534-PIP03-17-M01) 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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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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0066/2020 issued on 28 February 2020,

Having regard to the application submitted by OHB Neonatology Ltd on 22 January 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 April 2024, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for mecasermin rinfabate, solution for infusion, intravenous use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to OHB Neonatology Ltd, 1 Ashley Road, 3rd Floor, WA14 2DT - Altrincham, United Kingdom.

EMA/PDCO/60838/2024
Amsterdam, 26 April 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-000534-PIP03-17-M01

Scope of the application

Active substance(s):

Mecasermin rinfabate

Invented name and authorisation status:

See Annex II

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:

OHB Neonatology Ltd

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, OHB Neonatology Ltd submitted to the European Medicines Agency on 22 January 2024 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0066/2020 issued on 28 February 2020.

The application for modification proposed changes to the agreed paediatric investigation plan.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

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

2. The measures and timelines of the 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 annexes 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	Description
Quality-related studies	**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	**Study 3 (N10836M-SHP607)**

studies	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	**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	Not applicable
Other studies	Not applicable
Other measures	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 2030
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

The product is not authorised anywhere in the European Community.