EMA/444192/2024

European Medicines Agency decision

P/0355/2024

of 25 October 2024

on the acceptance of a modification of an agreed paediatric investigation plan for vamorolone (Agamree), (EMEA-001794-PIP02-16-M09) 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/0084/2018 issued on 16 March 2018, the decision P/0288/2018 issued on 12 September 2018, the decision P/0032/2020 issued on 29 January 2020, the decision P/0080/2021 issued on 17 March 2021, the decision P/0515/2021 issued on 3 December 2021, the decision P/0295/2022 issued on 11 August 2022, and the decision P/0515/2023 issued on 29 December 2023,

Having regard to the application submitted by Santhera Pharmaceuticals (Deutschland) GmbH on 3 June 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 6 September 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 vamorolone (Agamree), oral suspension, oral 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 Santhera Pharmaceuticals (Deutschland) GmbH, Marie-Curie Strasse 8, 79539 – Lörrach, Germany.

EMA/PDCO/285956/2024
Amsterdam, 6 September 2024

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

EMEA-001794-PIP02-16-M09

Scope of the application

Active substance(s):

Vamorolone

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Duchenne muscular dystrophy

Pharmaceutical form(s):

Oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Santhera Pharmaceuticals (Deutschland) GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Santhera Pharmaceuticals (Deutschland) GmbH submitted to the European Medicines Agency on 3 June 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0084/2018 issued on 16 March 2018, the decision P/0288/2018 issued on 12 September 2018, the decision P/0032/2020 issued on 29 January 2020, the decision P/0080/2021 issued on 17 March 2021, the decision P/0515/2021 issued on 3 December 2021, the decision P/0295/2022 issued on 11 August 2022, and the decision P/0515/2023 issued on 29 December 2023.

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

The procedure started on 8 July 2024.

Scope of the modification

Some measures 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition

Treatment of Duchenne muscular dystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne muscular dystrophy

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Oral suspension

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of an age appropriate formulation for paediatric patients from birth to less than 2 years of age
Non-clinical studies	**Study 2** Definitive juvenile toxicity study in mice **Study 3** Quantitative Whole Body Radiography (QWBA) study in rats **Study 4** Study deleted in EMEA-001794-PIP02-16-M09
Clinical studies	**Study 5** Multiple doses, open label study to assess the safety and tolerability, pharmacokinetics and pharmacodynamics of vamorolone in ambulant steroid naïve male paediatric subjects from 4 to less than 7 years of age with Duchenne muscular dystrophy (DMD) - VBP15-002. **Study 6** Open-label study of vamorolone to evaluate long-term safety, tolerability, efficacy and pharmacodynamics in steroid naïve paediatric

	subjects from 4 to less than 8 years of age with Duchenne muscular dystrophy - VBP15-003. **Study 7** Long-term safety tolerability, efficacy and pharmacodynamic study extension study in in steroid naïve male paediatric subjects from 4 to less than 8 years of age with Duchenne muscular dystrophy - VBP15-LTE. **Study 8** Double-blind, randomised, multi-centre, parallel, active and placebo controlled, efficacy and safety study in ambulatory male paediatric subjects from 4 to less than 7 years of age with Duchenne muscular dystrophy - VBP15-004. ***Study 9*** *Study deleted with procedure EMEA-001794-PIP02-16-M01.* **Study 10** Multiple ascending dose study to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of vamorolone in male paediatric subjects steroid-naïve from 2 to less than 4 years and from 7 to less than 18 years of age male paediatric subjects with Duchenne muscular dystrophy (DMD). **Study 11** Randomised, double-blind, placebo controlled study to assess long-term safety, tolerability, efficacy and pharmacodynamic of vamorolone in male paediatric subjects from 2 to less than 4 years of age with Duchenne muscular dystrophy - VBP15-007. **Study 12** Long-term safety, and efficacy versus historical controls study of vamorolone in steroid naïve and steroid treated male paediatric subjects from 7 to less than 18 years of age with Duchenne muscular dystrophy - VBP15-008. **Study 13** Open-label, controlled with placebo study to assess the long-term safety and efficacy of vamorolone in male paediatric subjects from birth to less than 2 years old, with Duchenne muscular dystrophy - VBP15-009.
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 December 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of Duchenne muscular dystrophy

Authorised indication(s):

- Agamree is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients aged 4 years and older
  - Invented name(s): Agamree
  - Authorised pharmaceutical form(s): oral suspension
  - Authorised route(s) of administration: oral use
  - Authorised via centralised procedure