EMA/12125/2020

European Medicines Agency decision

P/0032/2020

of 29 January 2020

on the acceptance of a modification of an agreed paediatric investigation plan for vamorolone (EMEA-001794-PIP02-16-M02) 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 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 European Medicines Agency's decision P/0084/2018 issued on 16 March 2018 and the decision P/0288/2018 issued on 12 September 2018,

Having regard to the application submitted by ReveraGen BioPharma Ltd. on 9 September 2019 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 11 December 2019, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for vamorolone, 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 ReveraGen BioPharma Ltd., The Oaks, 3 Village Road, Wirral, CH48 3JN - West Kirby, United Kingdom.

EMA/PDCO/513855/2019
Amsterdam, 11 December 2019

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

EMEA-001794-PIP02-16-M02

Scope of the application

Active substance(s):

Vamorolone

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:

ReveraGen BioPharma Ltd.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, ReveraGen BioPharma Ltd. submitted to the European Medicines Agency on 9 September 2019 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 and the decision P/0288/2018 issued on 12 September 2018.

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

The procedure started on 15 October 2019.

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

2. The measures and timelines of the paediatric investigation plan 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

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	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age appropriate formulation for paediatric patients from birth to less than 2 years of age
Non-clinical studies	3	**Study 2** Definitive juvenile toxicity study in mice **Study 3** Quantitative Whole Body Radiography (QWBA) study in rats **Study 4** Definitive juvenile toxicity study in mice (long term administration)
Clinical studies	8	**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 paeditric 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	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 December 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes