EMA/88205/2018

European Medicines Agency decision

P/0084/2018

of 16 March 2018

on the agreement of a paediatric investigation plan and on the granting of a deferral for vamorolone (EMEA-001794-PIP02-16) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Contact EMA www.ema.europa.eu/contact

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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 application submitted by ReveraGen BioPharma Ltd on 16 January 2017 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 January 2018, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 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 deferral.

(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 deferral.

[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 vamorolone, oral suspension, oral 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 deferral for vamorolone, oral suspension, oral 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 ReveraGen BioPharma Ltd, The Oaks, 3 Village Road, Wirral, CH48 3JN - West Kirby, United Kingdom.

EMA/PDCO/756214/2017
London, 26 January 2018

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

EMEA-001794-PIP02-16

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 16(1) of Regulation (EC) No 1901/2006 as amended, ReveraGen BioPharma Ltd submitted for agreement to the European Medicines Agency on 16 January 2017 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 21 February 2017.

Supplementary information was provided by the applicant on 6 November 2017. The applicant proposed modifications to the paediatric investigation plan.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5510
Send a question via our website www.ema.europa.eu/contact

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 deferral in accordance with Article 21 of said Regulation.

   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 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	9	**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.

Area	Number of measures	Description
		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** Randomized two dose groups (low vs. high dose) extension study to evaluate long-term safety, tolerability, efficacy and pharmacodynamic vs historical controls in ambulatory steroid naïve paediatric subjects from 4 to less than 7 years of age with Duchenne muscular dystrophy - VBP15-005. **Study 10** Multiple ascending dose study to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of vamorolone in 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 - VBP15-006. **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.

Area	Number of measures	Description
		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 November 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes