MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100811-PIP01-22) and to the deferral

MHRA-100811-PIP01-22-M01

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

Santhera Pharmaceuticals (Deutschland) GmbH

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Santhera Pharmaceuticals (Deutschland) GmbH submitted to the licensing authority on 05/01/2023 16:50 GMT an application for a Modification

The procedure started on 14/02/2023 15:54 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100811-PIP01-22-M01

Of 06/03/2023 07:00 GMT

On the adopted decision for Vamorolone (MHRA-100811-PIP01-22-M01) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for Vamorolone, Oral suspension , ORAL USE .

This decision is addressed to Santhera Pharmaceuticals (Deutschland) GmbH, Marie-Curie-Straße 8, Lörrach, GERMANY, 79539

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Duchenne Muscular Dystrophy

2.2 Indication(s) targeted by the PIP:

Treatment of Duchenne Muscular Dystrophy

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Oral suspension

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	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 (VBP15-002) 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 years to less than 7 years of age with Duchenne muscular dystrophy (DMD). Study 6 (VBP15-003) Open-label study of vamorolone to evaluate long-term safety, tolerability, efficacy and pharmacodynamics in steroid naïve paediatric subjects from 4 years to less than 8 years of age with DMD. Study 7 (VBP15-LTE) Long-term safety tolerability, efficacy and pharmacodynamic study extension study in in steroid naïve male paediatric subjects from 4 years to less than 8 years of age with DMD. Study 8 (VBP15-004) Double-blind, randomised, multi-centre, parallel, active and placebo controlled, efficacy and safety study in ambulatory male paediatric

		subjects from 4 years to less than 7 years of age with DMD. Study 9 Deleted during procedure EMEA-001794-PIP02-16-M01. Study 10 (VBP15-006) Multiple ascending dose study to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of vamorolone in steroid naïve from 2 years to less than 4 years and from 7 to less than 18 years of age male paediatric subjects with DMD. Study 11 (VBP15-007) Randomised, double-blind, placebo controlled study to assess long term safety, tolerability, efficacy and pharmacodynamic of vamorolone in male paediatric subjects from 2 years to less than 4 years of age with Duchenne muscular dystrophy. Study 12 (VBP15-008) Long-term safety, and efficacy versus historical controls study of vamorolone in steroid naïve and steroid treated male paediatric subjects from 7 years to less than 18 years of age with Duchenne muscular dystrophy. Study 13 (VBP15-009) 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 DMD.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	31/12/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes