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-M04) and to the deferral

MHRA-100811-PIP01-22-M05

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 18/03/2026 12:12 GMT an application for a Modification

The procedure started on 19/03/2026 13:15 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-M05

Of 25/03/2026 14:13 GMT

On the adopted decision for VAMOROLONE (MHRA-100811-PIP01-22-M05) 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-Str.8, 79539 Lörrach, Lörrach, GERMANY, 79539

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Duchenne muscular dystrophy. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age. Pharmaceutical form(s): Oral suspension Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

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:

The paediatric population from 2 years 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	0	Study 1 deleted during procedure MHRA-100811-PIP01-22-M04.
Non-Clinical Studies	2	Study 2 Definitive juvenile toxicity study in mice. Study 3 Quantitative Whole Body Radiography (QWBA) study in rats. Study 4 deleted during procedure MHRA-100811-PIP01-22-M03.
Clinical Studies	6	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 Duchenne muscular dystrophy. 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 Duchenne muscular dystrophy. 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 Duchenne muscular dystrophy. 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 Duchenne muscular dystrophy. 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 deleted during procedure MHRA-100811-PIP01-22-M04. Study 13 deleted during procedure MHRA-100811-PIP01-22-M04.
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:	31/12/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes