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-M01) and to the deferral

MHRA-100811-PIP01-22-M02

Scope of the Application

Active Substance(s)

Vamorolone

Condition(s)

Treatment of Duchenne muscular dystrophy

Pharmaceutical Form(s)

Oral solution

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 12/12/2023 15:27 GMT an application for a

The procedure started on 31/01/2024 15:40 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-M02

Of 13/02/2024 15:48 GMT

On the adopted decision for Vamorolone (MHRA-100811-PIP01-22-M02) 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 for Vamorolone, Oral solution , 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:

The paediatric population from birth to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Oral solution.

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). Study 6 (VBP15-003). Study 7 (VBP15-LTE). Study 8 (VBP15-004). Study 9 Deleted. Study 10 (VBP15-006). Study 11 (VBP15-007). Study 12 (VBP15-008). Study 13 (VBP15-009).
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