Clinical trial results:
A phase II open-label, multiple dose study to assess the safety, tolerability, pharmacokinetics, pharmacodynamics, and exploratory efficacy of vamorolone in boys ages 2 to <4 years and 7 to <18 years with Duchenne Muscular Dystrophy (DMD)

Summary

EudraCT number	2025-000201-16
Trial protocol	Outside EU/EEA
Global end of trial date	17 June 2024

Results information

Result version number	v1 (current)
This version publication date	01 August 2025
First version publication date	01 August 2025

Trial information

Trial identification

Sponsor protocol code	VBP15-006

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05185622
WHO universal trial number (UTN)	-
Other trial identifiers	IND: 118,942

Notes:

Sponsors

Sponsor organisation name	Santhera Pharmaceuticals (Schweiz) AG
Sponsor organisation address	Hohenrainstr. 24, Pratteln, Switzerland, 4133
Public contact	Chief Medical Officer, Santhera Pharmaceuticals (Schweiz) AG, office@santhera.com
Scientific contact	Chief Medical Officer, Santhera Pharmaceuticals (Schweiz) AG, office@santhera.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMEA-001794-PIP02-16
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	07 November 2024
Is this the analysis of the primary completion data?	Yes
Primary completion date	17 June 2024
Global end of trial reached?	Yes
Global end of trial date	17 June 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety and tolerability of vamorolone administered orally at daily doses of 2.0 mg/kg and 6.0 mg/kg over a 3-month treatment period in boys ages 2 to <4 and 7 to <18 years with DMD.

Protection of trial subjects:

No specific measures.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	21 March 2022
Long term follow-up planned	Yes
Long term follow-up rationale	Safety, Ethical reason
Long term follow-up duration	5 Years
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Canada: 54
Worldwide total number of subjects	54
EEA total number of subjects	0

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	42
Adolescents (12-17 years)	12
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

The study comprised a 5-week Pretreatment Screening Period; a 1-day Pretreatment Baseline Period. A total of 57 subjects were screened. Three subjects did not enter the Treatment Period due to failure to meet the inclusion criteria.

Period 1

Period 1 title	5-week Pretreatment Screening Period
Is this the baseline period?	No
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	Screening
Arm description: -	
Arm type	No intervention
No investigational medicinal product assigned in this arm	

Number of subjects in period 1	Screening
Started	54
Completed	54

Period 2

Period 2 title	1-day Pretreatment Baseline Period
Is this the baseline period?	Yes[1]
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes

Arm title	Age 2-<4 yrs - vamorolone 2 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 2 mg / kg	
Arm title	Age 2-<4 yrs - vamorolone 6 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 6 mg / kg	
Arm title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 2 mg / kg	
Arm title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 6 mg / kg	
Arm title	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 2 mg / kg	

Arm title	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 6 mg / kg	

Notes:

[1] - Period 1 is not the baseline period. It is expected that period 1 will be the baseline period.

Justification: Baseline Period is Period 2, when the assessments prior to 1st dose happen

Number of subjects in period 2	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Started	10	10	6
Completed	10	10	6

Number of subjects in period 2	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Started	6	6	16
Completed	6	6	16

Period 3

Period 3 title	3-month open-label Treatment Period
Is this the baseline period?	No
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes
Arm title	Age 2-<4 yrs - vamorolone 2 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 2 mg/kg	
Arm title	Age 2-<4 yrs - vamorolone 6 mg/kg

Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 6 mg/kg	
Arm title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 2 mg/kg	
Arm title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 6 mg/kg	
Arm title	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 2 mg/kg	
Arm title	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	vamorolone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Suspension for oral suspension
Routes of administration	Oral use
Dosage and administration details: 6 mg/kg	

Number of subjects in period 3	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Started	10	10	6
Completed	10	10	6

Number of subjects in period 3	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Started	6	6	16
Completed	6	6	16

Baseline characteristics

Reporting groups	
Reporting group title	Age 2-<4 yrs - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 2-<4 yrs - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Reporting group description: -	

Reporting group values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Number of subjects	10	10	6
Age categorical Units: Subjects			
Age 2 to <4 Years	10	10	0
Age 7 to <18 Years	0	0	6
Age continuous Units: years			
arithmetic mean	3.3	3.5	9.9
standard deviation	± 0.45	± 0.32	± 3.57
Gender categorical Units: Subjects			
Female	0	0	0
Male	10	10	6
Race Units: Subjects			
Asian	5	1	1
Black or African American	0	1	0
White	5	8	5
Multiple	0	0	0
Unknown	0	0	0
Age at 1st symptoms Units: months			
arithmetic mean	20.8	14.2	30.0
standard deviation	± 5.67	± 4.47	± 15.18

Reporting group values	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Number of subjects	6	6	16

Age categorical Units: Subjects			
Age 2 to <4 Years	0	0	0
Age 7 to <18 Years	6	6	16
Age continuous Units: years			
arithmetic mean	8.3	10.7	13.2
standard deviation	± 1.48	± 3.61	± 2.70
Gender categorical Units: Subjects			
Female	0	0	0
Male	6	6	16
Race Units: Subjects			
Asian	2	2	3
Black or African American	0	0	0
White	3	3	13
Multiple	1	0	0
Unknown	0	1	0
Age at 1st symptoms Units: months			
arithmetic mean	51.0	32.2	42.8
standard deviation	± 23.92	± 14.26	± 27.59

Reporting group values	Total		
Number of subjects	54		
Age categorical Units: Subjects			
Age 2 to <4 Years	20		
Age 7 to <18 Years	34		
Age continuous Units: years			
arithmetic mean			
standard deviation	-		
Gender categorical Units: Subjects			
Female	0		
Male	54		
Race Units: Subjects			
Asian	14		
Black or African American	1		
White	37		
Multiple	1		
Unknown	1		
Age at 1st symptoms Units: months			
arithmetic mean			
standard deviation	-		

Subject analysis sets	
Subject analysis set title	Safety set
Subject analysis set type	Safety analysis

Subject analysis set description:

All subjects who receive at least one dose of study medication. Is the analysis set for all analyses, except PK analyses.

Subject analysis set title	PK set
Subject analysis set type	Sub-group analysis

Subject analysis set description:

All subjects who receive at least one dose of vamorolone study medication and have sufficient data for PK analysis. Is the analysis set for PK analyses

Reporting group values	Safety set	PK set	
Number of subjects	54	54	
Age categorical Units: Subjects			
Age 2 to <4 Years	20	20	
Age 7 to <18 Years	34	34	
Age continuous Units: years			
arithmetic mean	8.4	8.4	
standard deviation	± 4.68	± 4.68	
Gender categorical Units: Subjects			
Female	0	0	
Male	54	54	
Race Units: Subjects			
Asian	14	14	
Black or African American	1	1	
White	37	37	
Multiple	1	1	
Unknown	1	1	
Age at 1st symptoms Units: months			
arithmetic mean	31.7	31.7	
standard deviation	± 21.88	± 21.88	

End points

End points reporting groups

Reporting group title	Screening
Reporting group description: -	
Reporting group title	Age 2-<4 yrs - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 2-<4 yrs - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 2-<4 yrs - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 2-<4 yrs - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Reporting group description: -	
Subject analysis set title	Safety set
Subject analysis set type	Safety analysis

Subject analysis set description:

All subjects who receive at least one dose of study medication. Is the analysis set for all analyses, except PK analyses.

Subject analysis set title	PK set
Subject analysis set type	Sub-group analysis

Subject analysis set description:

All subjects who receive at least one dose of vamorolone study medication and have sufficient data for PK analysis. Is the analysis set for PK analyses

Primary: Overview of treatment-emergent adverse events (TEAEs)

End point title	Overview of treatment-emergent adverse events (TEAEs)[1]

End point description:

End point type	Primary

End point timeframe:

3-month treatment period

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: Number				
Total number of subjects with TEAEs	7	9	6	4
With drug related TEAEs	1	7	3	4
With severe TEAEs	0	0	0	1
With TEAEs leading to permanent discontinuation	0	0	0	0
With TEAEs leading to temporary interruption	0	0	0	0
With serious TEAEs	0	0	0	1

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg	Safety set	
Subject group type	Reporting group	Reporting group	Subject analysis set	
Number of subjects analysed	6	16	54	
Units: Number				
Total number of subjects with TEAEs	3	12	41	
With drug related TEAEs	1	8	24	
With severe TEAEs	0	1	2	
With TEAEs leading to permanent discontinuation	0	0	0	
With TEAEs leading to temporary interruption	0	1	1	
With serious TEAEs	0	1	2	

Statistical analyses

No statistical analyses for this end point

Primary: Change in Height (Absolute) From Baseline

End point title	Change in Height (Absolute) From Baseline[2]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[2] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: cm				
arithmetic mean (standard deviation)	1.98 (± 1.146)	2.35 (± 0.568)	0.55 (± 0.557)	2.29 (± 1.609)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	14		
Units: cm				
arithmetic mean (standard deviation)	0.60 (± 0.594)	1.27 (± 1.313)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Height (Percentile) From Baseline

End point title	Change in Height (Percentile) From Baseline[3]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[3] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: %				
arithmetic mean (standard deviation)	1.17 (± 11.538)	2.09 (± 5.634)	-3.89 (± 3.907)	2.02 (± 8.338)

End point values	Age 7-<18 yrs,	Age 7-<18 yrs,		

	corticosteroid treated - vamorolone 2 mg/kg	corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	14		
Units: %				
arithmetic mean (standard deviation)	-3.69 (± 3.408)	-0.30 (± 1.390)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Height (Z-score) From Baseline

End point title	Change in Height (Z-score) From Baseline[4]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[4] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: SD				
arithmetic mean (standard deviation)	0.04 (± 0.361)	0.14 (± 0.201)	-0.12 (± 0.111)	0.07 (± 0.253)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	14		
Units: SD				
arithmetic mean (standard deviation)	-0.13 (± 0.159)	0.00 (± 0.194)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Weight (Absolute) From Baseline

End point title	Change in Weight (Absolute) From Baseline[5]

End point description:

End point type	Primary

End point timeframe:

week 12

Notes:

[5] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: cm				
arithmetic mean (standard deviation)	0.43 (± 0.776)	0.65 (± 0.911)	-0.18 (± 3.259)	3.47 (± 3.133)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: cm				
arithmetic mean (standard deviation)	1.53 (± 1.188)	1.94 (± 2.598)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Weight (Percentile) From Baseline

End point title	Change in Weight (Percentile) From Baseline[6]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[6] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: %				
arithmetic mean (standard deviation)	-1.44 (± 12.835)	-1.97 (± 12.504)	-4.00 (± 11.672)	15.20 (± 2.083)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: %				
arithmetic mean (standard deviation)	2.12 (± 4.235)	4.22 (± 8.566)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Weight (Z-score) From Baseline

End point title	Change in Weight (Z-score) From Baseline[7]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[7] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: SD				
arithmetic mean (standard deviation)	-0.01 (±	0.05 (± 0.430)	-0.03 (±	0.55 (± 0.159)

	0.406)		0.438)	

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: SD				
arithmetic mean (standard deviation)	0.11 (± 0.147)	0.22 (± 0.353)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Body Mass Index (BMI) (Absolute) From Baseline

End point title	Change in Body Mass Index (BMI) (Absolute) From Baseline[8]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[8] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: kg/m2				
arithmetic mean (standard deviation)	-0.09 (± 0.933)	-0.04 (± 0.665)	0.05 (± 1.394)	1.53 (± 1.385)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	14		
Units: kg/m2				
arithmetic mean (standard deviation)	0.82 (± 0.784)	0.60 (± 1.471)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Body Mass Index (BMI) (Percentile) From Baseline

End point title	Change in Body Mass Index (BMI) (Percentile) From Baseline[9]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[9] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: %				
arithmetic mean (standard deviation)	-2.85 (± 24.623)	-1.61 (± 12.509)	-3.00 (± 15.781)	10.78 (± 7.312)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	14		
Units: %				
arithmetic mean (standard deviation)	6.22 (± 6.849)	4.44 (± 11.498)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Body Mass Index (BMI) (Z-score) From Baseline

End point title	Change in Body Mass Index (BMI) (Z-score) From Baseline[10]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[10] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: SD				
arithmetic mean (standard deviation)	-0.05 (± 0.764)	-0.02 (± 0.495)	0.27 (± 0.771)	1.10 (± 0.584)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	14		
Units: SD				
arithmetic mean (standard deviation)	0.29 (± 0.317)	0.29 (± 0.483)		

Statistical analyses

No statistical analyses for this end point

Primary: Change in Blood Pressure (BP) from Baseline

End point title	Change in Blood Pressure (BP) from Baseline[11]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[11] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	9	10	6	6
Units: mmHg				
arithmetic mean (standard deviation)				
Systolic BP	-2.33 (± 12.923)	-2.20 (± 11.233)	-2.50 (± 6.504)	5.00 (± 13.266)
Diastolic BP	-0.44 (± 8.248)	2.00 (± 8.179)	-3.17 (± 7.333)	5.80 (± 6.979)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	15		
Units: mmHg				
arithmetic mean (standard deviation)				
Systolic BP	2.50 (± 5.958)	0.07 (± 11.591)		
Diastolic BP	1.50 (± 6.950)	-2.40 (± 11.915)		

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With Treatment Emergent Cushingoid Features

End point title	Number of Participants With Treatment Emergent Cushingoid Features[12]

End point description:

End point type	Primary

End point timeframe:

Baseline to Week 12

Notes:

[12] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: Number	0	0	0	1

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: Number	0	0		

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With Clinically Significant Treatment-emergent Abnormal Clinical Laboratory Test Result

End point title	Number of Participants With Clinically Significant Treatment-emergent Abnormal Clinical Laboratory Test Result[13]

End point description:

End point type	Primary

End point timeframe:

Baseline to Week 12

Notes:

[13] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: Number				
Cortisol decreased	0	1	2	0
Blood glucose decreased	0	0	0	0
Blood insulin in creased	0	0	0	0
Blood TSH increased	0	0	0	0
Thyroxin free increased	0	0	0	0

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: Number				
Cortisol decreased	0	2		
Blood glucose decreased	0	1		
Blood insulin in creased	0	1		
Blood TSH increased	0	1		
Thyroxin free increased	0	1		

Statistical analyses

No statistical analyses for this end point

Primary: Categorical Analysis of QTcF

End point title	Categorical Analysis of QTcF[14]

End point description:

End point type	Primary

End point timeframe:

Week 12

Notes:

[14] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: Number				
=< 450 msec	10	10	6	5
> 450 msec	0	0	0	0

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		

Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	15		
Units: Number				
=< 450 msec	5	15		
> 450 msec	0	0		

Statistical analyses

No statistical analyses for this end point

Primary: Number of Subjects With Cataract

End point title	Number of Subjects With Cataract[15]

End point description:

End point type	Primary

End point timeframe:

Baseline and Week 12

Notes:

[15] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: Number				
No cataract at Baseline	9	9	6	6
No cataract at Week 12	0	4	6	5
Cataract at Baseline	0	0	0	0
Cataract at Week12	0	0	0	0

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: Number				
No cataract at Baseline	4	8		
No cataract at Week 12	4	7		
Cataract at Baseline	2	8		
Cataract at Week12	1	7		

Statistical analyses

No statistical analyses for this end point

Primary: Number of Subjects With Glaucoma

End point title	Number of Subjects With Glaucoma[16]

End point description:

End point type	Primary

End point timeframe:

Baseline and Week 12

Notes:

[16] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive statistics were planned and performed

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: Number				
No Glaucoma at Baseline	9	10	6	6
No Glaucoma at Week 12	9	4	6	5
Suspected Glaucoma at Baseline	0	0	0	0
Suspected Glaucoma at Week 12	0	0	0	0
Glaucoma at Baseline	0	0	0	0
Glaucoma at Week 12	0	0	0	0

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: Number				
No Glaucoma at Baseline	4	8		
No Glaucoma at Week 12	4	7		
Suspected Glaucoma at Baseline	0	1		
Suspected Glaucoma at Week 12	0	0		
Glaucoma at Baseline	0	0		
Glaucoma at Week 12	0	0		

Statistical analyses

No statistical analyses for this end point

Secondary: Pre-dose and Post-dose Plasma Concentration Measurements of Vamorolone at Day 1 and Week 2 - Age 7-<18 yrs

End point title	Pre-dose and Post-dose Plasma Concentration Measurements of Vamorolone at Day 1 and Week 2 - Age 7-<18 yrs

End point description:

End point type	Secondary

End point timeframe:

Day 1 and Week 2

End point values	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	6	6	6	16
Units: ng/mL				
arithmetic mean (standard deviation)				
Day 1 - predose	0 (± 0.00)	0 (± 0.00)	0 (± 0.00)	0 (± 0.00)
Day 1 - 1h	142.5 (± 225.74)	519.1 (± 437.26)	142.5 (± 225.74)	519.1 (± 437.26)
Day 1 - 2h	216.4 (± 181.19)	780.5 (± 457.91)	216.4 (± 181.19)	780.5 (± 457.91)
Day 1 - 4h	163.8 (± 103.74)	711.1 (± 353.60)	163.8 (± 103.74)	711.1 (± 353.60)
Day 1 - 6h	148.0 (± 109.33)	389.1 (± 307.57)	148.0 (± 109.33)	389.1 (± 307.57)
Day 1 - 8h	78.5 (± 41.66)	170.6 (± 183.67)	78.5 (± 41.66)	170.6 (± 183.67)
Week 2 - predose	0.7 (± 2.06)	2.9 (± 5.22)	0.7 (± 2.06)	2.9 (± 5.22)
Week 2 - 1h	192.0 (± 199.92)	463.7 (± 348.19)	192.0 (± 199.92)	463.7 (± 348.19)
Week 2 - 2h	217.8 (± 149.31)	686.3 (± 369.69)	217.8 (± 149.31)	686.3 (± 369.69)
Week 2 - 4h	207.6 (± 167.99)	603.0 (± 346.88)	207.6 (± 167.99)	603.0 (± 346.88)
Week 2 - 6h	158.9 (± 105.34)	300.2 (± 206.18)	158.9 (± 105.34)	300.2 (± 206.18)
Week 2 - 8h	55.8 (± 31.81)	174.7 (± 137.78)	55.8 (± 31.81)	174.7 (± 137.78)

Statistical analyses

No statistical analyses for this end point

Secondary: Pre-dose and Post-dose Plasma Concentration Measurements of Vamorolone at Day 1 and Week 2 - Age 2-<4 yrs

End point title	Pre-dose and Post-dose Plasma Concentration Measurements of Vamorolone at Day 1 and Week 2 - Age 2-<4 yrs

End point description:

End point type	Secondary

End point timeframe:

Day 1 and Week 2

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	10	10		
Units: ng / mL				
arithmetic mean (standard deviation)				
Day 1 - predose	0.0 (± 0.00)	0 (± 0.00)		
Day 1 - 1h	198.3 (± 196.67)	471.3 (± 333.61)		
Day 1 - 2h	236.7 (± 153.58)	769.3 (± 527.67)		
Day 1 - 6h	125.7 (± 84.83)	280.0 (± 249.99)		
Week 2 - predose	0 (± 0.00)	0 (± 0.00)		
Week 2 - 1h	346.0 (± 329.08)	409.8 (± 385.65)		
Week 2 - 2h	360.4 (± 222.93)	829.4 (± 740.83)		
Week 2 - 6h	134.5 (± 110.99)	272.0 (± 109.71)		

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Change From Baseline in Bayley-III Gross Motor Scale

End point title	Change From Baseline in Bayley-III Gross Motor Scale

End point description:

Test only applied to 2 to 4 year old subjects

End point type	Other pre-specified

End point timeframe:

Week 12

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	9	10		
Units: scale unit				
arithmetic mean (standard deviation)	0.44 (± 1.130)	2.50 (± 1.716)		

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Change From Baseline in Morning Cortisol Concentration

End point title	Change From Baseline in Morning Cortisol Concentration

End point description:

End point type	Other pre-specified

End point timeframe:

Week 12

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	8	6	6
Units: nmol/mL				
arithmetic mean (standard deviation)	-184.700 (± 138.2020)	-217.750 (± 103.9832)	-110.667 (± 85.8596)	-248.667 (± 186.9060)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	15		

Units: nmol/mL				
arithmetic mean (standard deviation)	12.000 (± 49.3356)	-34.933 (± 42.1485)		

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Change From Baseline in Bone Turnover Biomarkers (Serum Type 1 Collagen C-telopeptide [CTX1], Osteocalcin and Serum Aminoterminal Propeptide of Type I Collagen [P1NP],)

End point title	Change From Baseline in Bone Turnover Biomarkers (Serum Type 1 Collagen C-telopeptide [CTX1], Osteocalcin and Serum Aminoterminal Propeptide of Type I Collagen [P1NP],)

End point description:

End point type	Other pre-specified

End point timeframe:

Week 12

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	9	6	5
Units: ng/L, ug/L				
arithmetic mean (standard deviation)				
CTX1 (ng/L)	28.300 (± 387.1423)	138.000 (± 257.6640)	93.500 (± 235.7446)	101.800 (± 365.3542)
Osteocalcin (ug/L)	-3.340 (± 21.0550)	2.422 (± 10.6960)	14.817 (± 18.7328)	-1.120 (± 15.8528)
P1NP (ug/L)	-39.870 (± 188.7787)	14.478 (± 117.5704)	19.067 (± 78.6139)	-82.840 (± 178.0726)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: ng/L, ug/L				
arithmetic mean (standard deviation)				
CTX1 (ng/L)	631.833 (± 155.3621)	273.000 (± 335.4937)		

Osteocalcin (ug/L)	31.700 (± 11.6915)	16.546 (± 9.1486)		
P1NP (ug/L)	318.850 (± 99.0038)	149.671 (± 106.2874)		

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Change From Baseline in Insulin Resistance Biomarkers - Part 1 (Glucose, Hemoglobin A1c [HbA1c])

End point title	Change From Baseline in Insulin Resistance Biomarkers - Part 1 (Glucose, Hemoglobin A1c [HbA1c])

End point description:

End point type	Other pre-specified

End point timeframe:

Week 12

End point values	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	10	6	6
Units: mmol/L, fraction of 1				
arithmetic mean (standard deviation)				
Glucose (mmol/L)	-0.160 (± 0.2912)	-0.243 (± 0.6309)	-0.063 (± 0.3524)	-0.335 (± 0.3815)
HbA1c (fraction of 1)	0.001 (± 0.0026)	0.000 (± 0.0015)	0.000 (± 0.0016)	0.000 (± 0.0010)

End point values	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	16		
Units: mmol/L, fraction of 1				
arithmetic mean (standard deviation)				
Glucose (mmol/L)	0.195 (± 0.5727)	-0.254 (± 0.3909)		
HbA1c (fraction of 1)	0.000 (± 0.0011)	-0.001 (± 0.0012)		

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Change From Baseline in Insulin Resistance Biomarkers - Part 2 (Insulin)

End point title	Change From Baseline in Insulin Resistance Biomarkers - Part 2 (Insulin)

End point description:

End point type	Other pre-specified

End point timeframe:

Week 12

End point values	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	6	6	16
Units: pmol/L				
arithmetic mean (standard deviation)	6.333 (± 20.5183)	0.667 (± 33.0051)	46.333 (± 51.9256)	-26.333 (± 51.1493)

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: Until study completion (Week 12) [SAEs were reported until 30 days after last dose of study drug]	
Assessment type	Systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	25.0
Reporting groups	
Reporting group title	Age 2-<4 yrs - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 2-<4 yrs - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg
Reporting group description: -	
Reporting group title	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Reporting group description: -	

Serious adverse events	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Gastrointestinal disorders			
vomiting			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Rhabdomyolisis			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	1 / 16 (6.25%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Gastrointestinal disorders			
vomiting			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Rhabdomyolisis			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	Age 2-<4 yrs - vamorolone 2 mg/kg	Age 2-<4 yrs - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid untreated - vamorolone 2 mg/kg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	7 / 10 (70.00%)	9 / 10 (90.00%)	6 / 6 (100.00%)
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Energy increased			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Fatigue			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Pyrexia			

subjects affected / exposed	1 / 10 (10.00%)	1 / 10 (10.00%)	1 / 6 (16.67%)
occurrences (all)	2	1	1
Immune system disorders			
Hypersensitivity			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	2 / 10 (20.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	2	0	0
Epistaxis			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Oropharyngeal pain			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Rhinorrhoea			
subjects affected / exposed	1 / 10 (10.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	1	2	0
Psychiatric disorders			
Aggression			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Behaviour disorder			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Compulsive lip biting			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Disruptive mood dysregulation disorder			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Irritability			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Mood swings			

subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Investigations			
Blood glucose decreased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Blood insulin increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Blood thyroid stimulating hormone increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Thyroxine free increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Cortisol decreased			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	2 / 6 (33.33%)
occurrences (all)	0	1	2
Weight increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Injury, poisoning and procedural complications			
Arthropod bite			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Fall			
subjects affected / exposed	1 / 10 (10.00%)	2 / 10 (20.00%)	0 / 6 (0.00%)
occurrences (all)	1	2	0
Head injury			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Joint injury			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Skin abrasion			

subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Nervous system disorders			
Headache			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	1 / 6 (16.67%)
occurrences (all)	0	1	3
Hypoaesthesia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Lymphadenopathy			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Eye disorders			
Cataract			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Abdominal pain upper			
subjects affected / exposed	0 / 10 (0.00%)	2 / 10 (20.00%)	0 / 6 (0.00%)
occurrences (all)	0	2	0
Abnormal faeces			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Diarrhoea			
subjects affected / exposed	0 / 10 (0.00%)	3 / 10 (30.00%)	0 / 6 (0.00%)
occurrences (all)	0	3	0
Faeces discoloured			
subjects affected / exposed	1 / 10 (10.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	1	1	0
Nausea			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Toothache			

subjects affected / exposed	0 / 10 (0.00%)	2 / 10 (20.00%)	0 / 6 (0.00%)
occurrences (all)	0	2	0
Vomiting			
subjects affected / exposed	1 / 10 (10.00%)	1 / 10 (10.00%)	1 / 6 (16.67%)
occurrences (all)	1	1	1
Skin and subcutaneous tissue disorders			
Miliaria			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Rash			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Urticaria			
subjects affected / exposed	1 / 10 (10.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Endocrine disorders			
Adrenal insufficiency			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Adrenal suppression			
subjects affected / exposed	0 / 10 (0.00%)	5 / 10 (50.00%)	0 / 6 (0.00%)
occurrences (all)	0	5	0
Cushingoid			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Back pain			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Musculoskeletal stiffness			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Myalgia			

subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Pain in extremity			
subjects affected / exposed	0 / 10 (0.00%)	2 / 10 (20.00%)	0 / 6 (0.00%)
occurrences (all)	0	2	0
Infections and infestations			
COVID-19			
subjects affected / exposed	1 / 10 (10.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Gastroenteritis viral			
subjects affected / exposed	0 / 10 (0.00%)	1 / 10 (10.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Nasopharyngitis			
subjects affected / exposed	4 / 10 (40.00%)	3 / 10 (30.00%)	2 / 6 (33.33%)
occurrences (all)	8	3	2
Upper respiratory tract infection			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Metabolism and nutrition disorders			
Increased appetite			
subjects affected / exposed	0 / 10 (0.00%)	0 / 10 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0

Non-serious adverse events	Age 7-<18 yrs, corticosteroid untreated - vamorolone 6 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 2 mg/kg	Age 7-<18 yrs, corticosteroid treated - vamorolone 6 mg/kg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	4 / 6 (66.67%)	3 / 6 (50.00%)	12 / 16 (75.00%)
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	0 / 6 (0.00%)	2 / 6 (33.33%)	0 / 16 (0.00%)
occurrences (all)	0	2	0
Energy increased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Fatigue			

subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Pyrexia			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Immune system disorders			
Hypersensitivity			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	0	1	0
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Epistaxis			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Oropharyngeal pain			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Rhinorrhoea			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	0	1	0
Psychiatric disorders			
Aggression			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Behaviour disorder			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Compulsive lip biting			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Disruptive mood dysregulation disorder			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	2 / 16 (12.50%)
occurrences (all)	0	0	2
Irritability			

subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Mood swings			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Investigations			
Blood glucose decreased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Blood insulin increased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Blood thyroid stimulating hormone increased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Thyroxine free increased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Cortisol decreased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	2 / 16 (12.50%)
occurrences (all)	0	0	2
Weight increased			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Injury, poisoning and procedural complications			
Arthropod bite			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	0	1	0
Fall			
subjects affected / exposed	1 / 6 (16.67%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	1	2	0
Head injury			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Joint injury			

subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	0	1	0
Skin abrasion			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	0	1	0
Nervous system disorders			
Headache			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	4 / 16 (25.00%)
occurrences (all)	0	0	5
Hypoaesthesia			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	0	1	0
Blood and lymphatic system disorders			
Lymphadenopathy			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Eye disorders			
Cataract			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Abdominal pain upper			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	3
Abnormal faeces			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Diarrhoea			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Faeces discoloured			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Nausea			

subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Toothache			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Vomiting			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	2 / 16 (12.50%)
occurrences (all)	2	0	2
Skin and subcutaneous tissue disorders			
Miliaria			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Rash			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	0	1	0
Urticaria			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Endocrine disorders			
Adrenal insufficiency			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Adrenal suppression			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	1	0	1
Cushingoid			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Back pain			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	2 / 16 (12.50%)
occurrences (all)	0	1	2
Musculoskeletal stiffness			

subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Myalgia			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 16 (0.00%)
occurrences (all)	0	1	0
Pain in extremity			
subjects affected / exposed	0 / 6 (0.00%)	2 / 6 (33.33%)	0 / 16 (0.00%)
occurrences (all)	0	2	0
Infections and infestations			
COVID-19			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Gastroenteritis viral			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	0	0	0
Nasopharyngitis			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	2 / 16 (12.50%)
occurrences (all)	0	1	2
Upper respiratory tract infection			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Metabolism and nutrition disorders			
Increased appetite			
subjects affected / exposed	2 / 6 (33.33%)	0 / 6 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
14 April 2022	VBP15-006-A1 (Version 1.1) Reasons for Protocol Amendment #1: 1. To clarify vamorolone is a 4.0% wt/vol suspension and provide details of vamorolone packaging 2. To clarify that age of enrollment will be collected and date of birth will not 3. To clarify standing height will be measured in all participants who can stand independently 4. To remove glutamate dehydrogenase (GLDH) from laboratory evaluations 5. To clarify vamorolone administration will take place at the study site during the Week 6 visit 6. To specify subjects should be fasted ≥6 hours prior to all study visits when blood will be drawn
09 February 2023	VBP15-006-A2 (Version 1.0) Reasons for Protocol Amendment #2: The following changes have been included in this amendment and will be implemented following the standard approval process: • To make editorial modifications regarding transfer of Sponsor from ReveraGen BioPharma, Inc. to Santhera Pharmaceutical (Switzerland) Ltd. • To update section 1 following Investigator's Brochure revision (version 12) • To update the reference list • To update Dosing Recommendation for children weighing 50 kg or more • To describe the procedure of the dose tapering after discontinuation of vamorolone • To include ambulatory status recording as part of physical examination at Baseline • To define a new age subgroup (12 to <18 years) in which 10 additional glucocorticoid-treated subjects will be recruited, to the vamorolone daily dose of 6 mg/kg and to add exploration of the gonadal and thyroid axis biomarkers in this subgroup. To update the total number of participating subjects accordingly. • To clarify that date of birth may be collected where local regulations allow.
11 April 2023	VBP15-006-A3 (Version 1.0) Reasons for Protocol Amendment #3: PPD, the central laboratory was providing an EDTA-1ML K2 (Plastic) tube for collection of specific samples. However the manufacturer of this item has informed PPD that they are discontinuing the manufacture of this item globally, effective immediately. As such and since there is no equivalent tube with 1 mL volume, PPD needs to replace it with an alternate tube EDTA-1.2 mL S-Monovette K2 (Plastic). As a consequence, the volumes of blood collected at the study visits need to be revised. The following changes have been included in this amendment and will be implemented following the standard approval process: • Replace the 1 mL EDTA blood sampling tube by the 1.2 mL and revise the sampling plan (HbA1c is analyzed from the hematology sample)

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported