Clinical trial results:
A Phase IIIb, open-label, single-arm, single-dose, multicenter study to evaluate the safety, tolerability and efficacy of gene replacement therapy with intravenous OAV101 (AVXS-101) in pediatric patients with spinal muscular atrophy (SMA)

Summary

EudraCT number	2020-005995-37
Trial protocol	FR DE BE PT IT
Global end of trial date	13 June 2023

Results information

Result version number	v1
This version publication date	24 December 2023
First version publication date	24 December 2023

Trial information

Trial identification

Sponsor protocol code	COAV101A12306

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04851873
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Novartis Pharma AG
Sponsor organisation address	Novartis Campus, Basel, Switzerland,
Public contact	Clinical Disclosure Office, Novartis Pharma AG, 41 613241111, Novartis.email@Novartis.com
Scientific contact	Clinical Disclosure Office, Novartis Pharma AG, 41 613241111, Novartis.email@Novartis.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMEA-002168-PIP01-17
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	13 June 2023
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	13 June 2023
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective was to assess the safety and tolerability of intravenous (IV) OAV101 over a 12-month period in participants with SMA weighing ≥8.5 kg to ≤ 21 kg.

Protection of trial subjects:

The study was in compliance with the ethical principles derived from the Declaration of Helsinki and the International Conference on Harmonization (ICH) Good Clinical Practice (GCP) guidelines. All the local regulatory requirements pertinent to safety of trial subjects were also followed during the conduct of the trial.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	08 September 2021
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Australia: 3
Country: Number of subjects enrolled	Belgium: 2
Country: Number of subjects enrolled	Canada: 4
Country: Number of subjects enrolled	France: 2
Country: Number of subjects enrolled	United Kingdom: 3
Country: Number of subjects enrolled	Italy: 2
Country: Number of subjects enrolled	Portugal: 3
Country: Number of subjects enrolled	Taiwan: 3
Country: Number of subjects enrolled	United States: 2
Worldwide total number of subjects	24
EEA total number of subjects	9

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)	1
Children (2-11 years)	23
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Participants took part in 13 investigative sites across 9 countries.

Pre-assignment

Screening details:

The screening period began after signature of the study informed consent. The study included a screening period of up to 45 days. On Day -1, participants were admitted to the hospital for pre-treatment baseline procedures. On Day 1, participants received the study treatment.

Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes
Arm title	OAV101 1.1e14 vg/kg 8.5-13 kg

Arm description:

8.5-13 kg

Arm type	Experimental
Investigational medicinal product name	Onasemnogene abeparvovec
Investigational medicinal product code	OAV101
Other name	AVXS-101
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

OAV101 1.1e14 vg/kg

Arm title	OAV101 1.1e14 vg/kg >13-17 kg

Arm description:

>13-17 kg

Arm type	Experimental
Investigational medicinal product name	Onasemnogene abeparvovec
Investigational medicinal product code	OAV101
Other name	AVXS-101
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

OAV101 1.1e14 vg/kg

Arm title	OAV101 1.1e14 vg/kg >17-21 kg

Arm description:

>17-21 kg

Arm type	Experimental
Investigational medicinal product name	Onasemnogene abeparvovec
Investigational medicinal product code	OAV101
Other name	AVXS-101
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:
OAV101 1.1e14 vg/kg

Number of subjects in period 1	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg >13-17 kg	OAV101 1.1e14 vg/kg >17-21 kg
Started	7	8	9
Completed	7	8	9

Baseline characteristics

Reporting groups	
Reporting group title	OAV101 1.1e14 vg/kg 8.5-13 kg
Reporting group description: 8.5-13 kg	
Reporting group title	OAV101 1.1e14 vg/kg >13-17 kg
Reporting group description: >13-17 kg	
Reporting group title	OAV101 1.1e14 vg/kg >17-21 kg
Reporting group description: >17-21 kg	

Reporting group values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg >13-17 kg	OAV101 1.1e14 vg/kg >17-21 kg
Number of subjects	7	8	9
Age Categorical Units: Participants			
0 < 28 days	0	0	0
28 days - < 2 years	1	0	0
2 years - < 12 years	6	8	9
12 years - <18 years	0	0	0
>= 18 years	0	0	0
Age Continuous Units: years			
arithmetic mean	3.027	4.519	6.137
standard deviation	± 1.1458	± 1.1769	± 1.6018
Sex: Female, Male Units: Participants			
Female	3	5	4
Male	4	3	5
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native	0	0	0
Asian	1	5	3
Native Hawaiian or Other Pacific Islander	0	0	0
Black or African American	0	0	0
White	6	1	6
More than one race	0	0	0
Unknown or Not Reported	0	2	0

Reporting group values	Total		
Number of subjects	24		
Age Categorical Units: Participants			
0 < 28 days	0		
28 days - < 2 years	1		
2 years - < 12 years	23		
12 years - <18 years	0		

>= 18 years	0		

Age Continuous Units: years arithmetic mean standard deviation	- 		
Sex: Female, Male Units: Participants			
Female	12		
Male	12		
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native	0		
Asian	9		
Native Hawaiian or Other Pacific Islander	0		
Black or African American	0		
White	13		
More than one race	0		
Unknown or Not Reported	2		

End points

End points reporting groups

Reporting group title	OAV101 1.1e14 vg/kg 8.5-13 kg
Reporting group description: 8.5-13 kg	
Reporting group title	OAV101 1.1e14 vg/kg >13-17 kg
Reporting group description: >13-17 kg	
Reporting group title	OAV101 1.1e14 vg/kg >17-21 kg
Reporting group description: >17-21 kg	
Subject analysis set title	OAV101 1.1e14 vg/kg Overall
Subject analysis set type	Full analysis
Subject analysis set description: Overall	
Subject analysis set title	OAV101 1.1e14 vg/kg >13-17kg
Subject analysis set type	Full analysis
Subject analysis set description: >13-17kg	
Subject analysis set title	OAV101 1.1e14 vg/kg >17-21kg
Subject analysis set type	Full analysis
Subject analysis set description: >17-21kg	
Subject analysis set title	OAV101 1.1e14 vg/kg Overall
Subject analysis set type	Full analysis
Subject analysis set description: Overall	
Subject analysis set title	OAV101 1.1e14 vg/kg 8.5-13 kg Overall
Subject analysis set type	Full analysis
Subject analysis set description: Overall	

Primary: Number of participants with treatment emergent Adverse Events (AEs) and Serious Adverse Events (SAEs) by weight bracket

End point title	Number of participants with treatment emergent Adverse Events (AEs) and Serious Adverse Events (SAEs) by weight bracket[1]

End point description:

An AE is any untoward medical occurrence (e.g. any unfavorable and unintended sign [including abnormal laboratory findings], symptom or disease) in a clinical investigation participant after providing written informed consent for participation in the study.

End point type	Primary

End point timeframe:

Up to Month 12

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: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg >13-17 kg	OAV101 1.1e14 vg/kg >17-21 kg	OAV101 1.1e14 vg/kg Overall
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	7	8	9	24
Units: Participants				
Any treatment-emergent adverse events (AEs)	7	8	9	24
Any treatment-emergent AEs related to OAV101	7	8	9	24
Any severe treatment-emergent AEs	1	4	3	8
Any serious treatment-emergent AEs	3	7	5	15
Serious treatment-emergent AEs related to OAV101	1	4	2	7
Treatment-emergent AEs leading to study disc.	0	0	0	0
Treatment-emergent AEs leading to death	0	0	0	0
Treatment-emergent AEs of special interest	7	7	9	23

Statistical analyses

No statistical analyses for this end point

Primary: Number of participants with important identified and important potential risks (Adverse Events of Special Interest (AESI)) by risk name and weight bracket

End point title	Number of participants with important identified and important potential risks (Adverse Events of Special Interest (AESI)) by risk name and weight bracket[2]

End point description:

Important identified and important potential risks included the following AESIs: Hepatotoxicity, Thrombocytopenia, Cardiac adverse events, Dorsal root ganglia toxicity and Thrombotic microangiopathy.

These were assessed by the investigator.

End point type	Primary

End point timeframe:

Up to Month 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: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg >13-17 kg	OAV101 1.1e14 vg/kg >17-21 kg	OAV101 1.1e14 vg/kg Overall
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	7	8	9	24
Units: Participants				
Hepatotoxicity	6	5	9	20
Transient thrombocytopenia	4	6	7	17
Cardiac adverse events	0	2	1	3
Thrombotic microangiopathy	0	0	0	0
Dorsal root ganglia cell inflammation	0	0	0	0

Statistical analyses

No statistical analyses for this end point

Primary: Summary of participants meeting criteria for potentially clinically significant vital sign values by weight bracket - systolic and diastolic blood pressure

End point title	Summary of participants meeting criteria for potentially clinically significant vital sign values by weight bracket - systolic and diastolic blood pressure[3][4]

End point description:

Change from baseline in vital signs measurements - systolic and diastolic blood pressure (mmHg).

Systolic Blood Pressure-Low:<=5th percentile of the age(Any Age), High:>=90th percentile of the age, gender, and height group (<18 yrs).

Diastolic Blood Pressure-High:>=90th percentile of the age, gender, and height group(<18 yrs).

End point type	Primary

End point timeframe:

12 months

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: This is an open label, single dose study - statistical analysis does not apply.

[4] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg >13-17kg	OAV101 1.1e14 vg/kg >17-21kg
Subject group type	Reporting group	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	24	8	9
Units: Participants				
Systolic Blood Pressure (mmHg) Low	1	2	0	1
Systolic Blood Pressure (mmHg) High	7	23	8	8
Diastolic Blood Pressure (mmHg) High	7	22	7	8

Statistical analyses

No statistical analyses for this end point

Primary: Change from baseline in vital signs measurements - Systolic Blood Pressure (mmHg)

End point title	Change from baseline in vital signs measurements - Systolic Blood Pressure (mmHg)[5][6]

End point description:

End point type	Primary

End point timeframe:

Baseline, Days 2 and 3, Weeks 1, 2, 3, 4, 6, 8, 10, 13, 26, 39 and 52

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: This is an open label, single dose study - statistical analysis does not apply.

[6] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg >13-17kg	OAV101 1.1e14 vg/kg >17-21kg
Subject group type	Reporting group	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	24	8	9
Units: mmHg				
arithmetic mean (standard deviation)				
Change from baseline at Day 2	5.3 (± 12.65)	1.3 (± 11.75)	-1.3 (± 13.01)	0.4 (± 10.36)
Change from baseline at Day 3	-2.3 (± 15.01)	-1.4 (± 11.62)	-5.6 (± 11.16)	3.0 (± 8.38)
Change from baseline at Week 1	13.4 (± 10.53)	2.2 (± 15.18)	-7.4 (± 15.59)	2.0 (± 12.84)
Change from baseline at Week 2	11.7 (± 20.33)	2.8 (± 16.45)	-3.8 (± 17.60)	1.8 (± 9.15)
Change from baseline at Week 3 (n=6,8,9,23)	15.8 (± 8.04)	2.7 (± 12.33)	-3.5 (± 12.87)	-0.7 (± 7.25)
Change from baseline at Week 4 (n=6,8,8,22)	12.3 (± 16.50)	4.5 (± 15.05)	-3.5 (± 15.89)	6.8 (± 10.02)
Change from baseline at Week 6 (n=7,8,8,23)	8.1 (± 12.92)	3.4 (± 18.13)	-2.8 (± 24.15)	5.4 (± 15.39)
Change from baseline at Week 8 (n=7,7,9,23)	3.9 (± 15.49)	2.3 (± 13.69)	-0.7 (± 14.57)	3.6 (± 12.83)
Change from baseline at Week 10	10.0 (± 16.99)	2.8 (± 14.32)	-5.6 (± 13.19)	4.6 (± 10.06)
Change from baseline at Week 13	6.1 (± 10.78)	0.8 (± 13.28)	-4.5 (± 17.50)	1.4 (± 9.93)
Change from baseline at Week 26	-5.7 (± 9.05)	-8.5 (± 13.46)	-12.9 (± 15.34)	-6.9 (± 14.99)
Change from baseline at Week 39 (n=7,7,9,23)	16.4 (± 29.70)	0.3 (± 21.98)	-10.3 (± 13.59)	-3.9 (± 3.56)
Change from baseline at Week 52	5.0 (± 21.60)	-1.1 (± 15.67)	-7.8 (± 13.54)	0.1 (± 10.81)

Statistical analyses

No statistical analyses for this end point

Primary: Change from baseline in vital signs measurements - Diastolic Blood Pressure (mmHg)

End point title	Change from baseline in vital signs measurements - Diastolic Blood Pressure (mmHg)[7][8]

End point description:

End point type	Primary

End point timeframe:

Baseline, Days 2 and 3, Weeks 1, 2, 3, 4, 6, 8, 10, 13, 26, 39 and 52

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: This is an open label, single dose study - statistical analysis does not apply.

[8] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg >13-17kg	OAV101 1.1e14 vg/kg >17-21kg
Subject group type	Reporting group	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	24	8	9
Units: mmHg				
arithmetic mean (standard deviation)				
Change from baseline at Day 2 (n=6,8,9,23)	1.7 (± 12.42)	1.3 (± 12.71)	4.8 (± 13.22)	-2.1 (± 13.04)
Change from baseline at Day 3 (n=6,8,9,23)	-3.7 (± 12.29)	-1.1 (± 11.22)	-5.1 (± 13.91)	4.2 (± 5.45)
Change from baseline at Week 1	7.4 (± 20.07)	4.2 (± 15.13)	1.8 (± 13.56)	3.8 (± 13.41)
Change from baseline at Week 2	7.1 (± 16.07)	6.2 (± 12.05)	7.3 (± 11.30)	4.4 (± 10.33)
Change from baseline at Week 3 (n=6,8,9,23)	12.0 (± 15.09)	4.6 (± 13.28)	-1.5 (± 14.01)	5.0 (± 9.60)
Change from baseline at Week 4 (n=6,8,8,22)	3.8 (± 19.27)	2.1 (± 12.12)	1.8 (± 9.97)	1.3 (± 8.53)
Change from baseline at Week 6 (n=6,8,8,22)	10.3 (± 10.91)	5.0 (± 13.77)	-0.5 (± 17.87)	6.5 (± 10.14)
Change from baseline at Week 8 (n=6,7,9,22)	10.5 (± 14.57)	6.2 (± 14.18)	3.0 (± 17.68)	5.9 (± 11.72)
Change from baseline at Week 10 (n=6,8,9,23)	6.7 (± 15.55)	2.9 (± 12.01)	-2.0 (± 8.88)	4.8 (± 11.74)
Change from baseline at Week 13 (n=6,8,9,23)	9.7 (± 15.37)	2.7 (± 13.20)	0.4 (± 13.54)	0.1 (± 11.03)
Change from baseline at Week 26	-0.7 (± 9.05)	-3.7 (± 9.41)	-9.1 (± 10.26)	-1.2 (± 7.55)
Change from baseline at Week 39 (n=7,7,9,23)	2.4 (± 15.73)	-0.8 (± 12.44)	-4.4 (± 13.01)	-0.4 (± 9.62)
Change from baseline at Week 52	0.3 (± 11.74)	0.0 (± 9.60)	-2.0 (± 11.89)	1.7 (± 5.52)

Statistical analyses

No statistical analyses for this end point

Primary: Change from baseline in vital signs measurements - Respiratory Rate (breaths/min)

End point title	Change from baseline in vital signs measurements - Respiratory Rate (breaths/min)[9][10]

End point description:

Change from baseline in vital signs measurements - oxygen saturation level (%).

Oxygen saturation is the fraction of oxygen-saturated hemoglobin relative to total hemoglobin (unsaturated+saturated) in the blood and then multiplied by 100.

End point type	Primary

End point timeframe:

Baseline, Days 2 and 3, Weeks 1, 2, 3, 4, 6, 8, 10, 13, 26, 39 and 52

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: This is an open label, single dose study - statistical analysis does not apply.

[10] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg >13-17kg	OAV101 1.1e14 vg/kg >17-21kg
Subject group type	Reporting group	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	24	8	9
Units: breaths/min)				
arithmetic mean (standard deviation)				
Change from baseline at Day 2	-1.1 (± 3.08)	-0.6 (± 3.66)	-1.6 (± 3.74)	0.8 (± 3.96)
Change from baseline at Day 3	-0.1 (± 5.87)	0.1 (± 4.66)	-0.1 (± 4.52)	0.4 (± 4.30)
Change from baseline at Week 1	2.7 (± 6.78)	1.3 (± 5.45)	-0.1 (± 4.22)	1.6 (± 5.61)
Change from baseline at Week 2	2.4 (± 5.44)	1.0 (± 4.81)	-0.8 (± 5.01)	1.4 (± 4.16)
Change from baseline at Week 3 (n=6,8,9,23)	5.5 (± 8.96)	1.5 (± 6.63)	-1.8 (± 5.09)	1.8 (± 5.02)
Change from baseline at Week 4 (n=7,8,8,23)	2.6 (± 4.54)	1.7 (± 5.31)	1.4 (± 7.35)	1.4 (± 4.00)
Change from baseline at Week 6 (n=7,8,8,23)	5.1 (± 9.56)	1.4 (± 7.19)	-0.6 (± 4.75)	0.3 (± 6.43)
Change from baseline at Week 8 (n=7,7,9,23)	3.3 (± 7.45)	0.8 (± 5.52)	-0.6 (± 4.47)	0.0 (± 4.42)
Change from baseline at Week 10	3.3 (± 6.58)	0.5 (± 4.99)	-1.0 (± 4.31)	-0.3 (± 3.61)
Change from baseline at Week 13	1.7 (± 6.75)	0.1 (± 4.44)	-0.5 (± 3.51)	-0.6 (± 2.92)
Change from baseline at Week 26	4.1 (± 8.69)	1.7 (± 5.71)	0.8 (± 3.69)	0.7 (± 4.24)
Change from baseline at Week 39 (n=7,7,9,23)	1.4 (± 5.03)	-0.3 (± 4.61)	-1.7 (± 4.35)	-0.6 (± 4.56)
Change from baseline at Week 52	1.7 (± 7.34)	-0.5 (± 5.18)	-0.5 (± 4.66)	-2.2 (± 3.19)

Statistical analyses

No statistical analyses for this end point

Primary: Change from baseline in vital signs measurements - Pulse Rate (beats/min)

End point title	Change from baseline in vital signs measurements - Pulse Rate (beats/min)[11][12]

End point description:

Change from baseline in vital signs measurements - oxygen saturation level (%).

Oxygen saturation is the fraction of oxygen-saturated hemoglobin relative to total hemoglobin (unsaturated+saturated) in the blood and then multiplied by 100.

End point type	Primary

End point timeframe:

Baseline, Days 2 and 3, Weeks 1, 2, 3, 4, 6, 8, 10, 13, 26, 39 and 52

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: This is an open label, single dose study - statistical analysis does not apply.

[12] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg >13-17kg	OAV101 1.1e14 vg/kg >17-21kg
Subject group type	Reporting group	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	24	8	9
Units: beats/min				
arithmetic mean (standard deviation)				
Change from baseline at Day 2	3.3 (± 18.54)	9.2 (± 19.35)	6.3 (± 4.43)	16.3 (± 26.54)
Change from baseline at Day 3	-1.4 (± 19.29)	2.4 (± 16.36)	-3.1 (± 14.21)	10.2 (± 14.19)
Change from baseline at Week 1	1.4 (± 14.27)	-4.3 (± 17.19)	-9.6 (± 18.04)	-3.9 (± 18.84)
Change from baseline at Week 2	-13.4 (± 21.25)	-5.1 (± 17.39)	-8.8 (± 15.21)	4.7 (± 12.12)
Change from baseline at Week 3 (n=6,8,9,23)	21.0 (± 21.83)	9.3 (± 18.60)	-3.8 (± 12.46)	13.1 (± 15.12)
Change from baseline at Week 4 (n=7,8,8,23)	-4.7 (± 17.27)	1.8 (± 19.95)	-3.5 (± 22.82)	12.8 (± 16.23)
Change from baseline at Week 6 (n=7,8,8,23)	12.9 (± 20.70)	7.0 (± 16.58)	0.0 (± 9.59)	8.8 (± 17.64)
Change from baseline at Week 8 (n=7,7,9,23)	-3.6 (± 15.75)	-0.5 (± 18.83)	-6.3 (± 22.69)	6.4 (± 17.63)
Change from baseline at Week 10	-3.9 (± 25.14)	1.1 (± 19.08)	-4.3 (± 16.57)	9.7 (± 14.13)
Change from baseline at Week 13	-6.4 (± 12.23)	2.2 (± 16.28)	2.6 (± 15.65)	8.4 (± 18.06)
Change from baseline at Week 26	-6.9 (± 12.85)	-2.7 (± 15.12)	-6.3 (± 17.00)	3.8 (± 14.34)
Change from baseline at Week 39 (n=7,7,9,23)	-4.7 (± 21.85)	-2.6 (± 17.54)	-7.7 (± 13.23)	3.1 (± 17.12)
Change from baseline at Week 52	-8.9 (± 10.32)	-6.4 (± 16.35)	-16.4 (± 14.38)	4.4 (± 16.46)

Statistical analyses

No statistical analyses for this end point

Primary: Change from baseline in vital signs measurements - Temperature (C)

End point title	Change from baseline in vital signs measurements - Temperature (C)[13][14]

End point description:

End point type	Primary

End point timeframe:

Baseline, Days 2 and 3, Weeks 1, 2, 3, 4, 6, 8, 10, 13, 26, 39 and 52

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: This is an open label, single dose study - statistical analysis does not apply.

[14] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg >13-17kg	OAV101 1.1e14 vg/kg >17-21kg
Subject group type	Reporting group	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	24	8	9
Units: Degrees Celsius				
arithmetic mean (standard deviation)				
Change from baseline at Day 2	0.07 (± 0.502)	0.01 (± 0.722)	-0.11 (± 0.714)	0.07 (± 0.918)
Change from baseline at Day 3	0.11 (± 0.261)	-0.08 (± 0.541)	-0.48 (± 0.719)	0.12 (± 0.319)
Change from baseline at Week 1	-0.07 (± 0.386)	-0.17 (± 0.528)	-0.44 (± 0.563)	0.00 (± 0.548)
Change from baseline at Week 2	0.30 (± 0.592)	-0.08 (± 0.618)	-0.41 (± 0.649)	-0.09 (± 0.478)
Change from baseline at Week 3 (n=6,8,9,23)	0.32 (± 0.556)	0.01 (± 0.474)	-0.24 (± 0.403)	0.02 (± 0.387)
Change from baseline at Week 4 (n=7,8,8,23)	0.16 (± 0.673)	-0.04 (± 0.645)	-0.31 (± 0.775)	0.05 (± 0.431)
Change from baseline at Week 6 (n=7,8,8,23)	0.26 (± 0.458)	-0.01 (± 0.535)	-0.18 (± 0.599)	-0.09 (± 0.500)
Change from baseline at Week 8 (n=7,7,9,23)	0.13 (± 0.547)	-0.02 (± 0.521)	-0.21 (± 0.654)	0.01 (± 0.386)
Change from baseline at Week 10	0.34 (± 0.351)	-0.05 (± 0.635)	-0.45 (± 0.727)	0.01 (± 0.553)
Change from baseline at Week 13	0.13 (± 0.399)	0.05 (± 0.525)	-0.06 (± 0.701)	0.09 (± 0.473)
Change from baseline at Week 26	0.31 (± 0.453)	0.05 (± 0.460)	-0.15 (± 0.532)	0.03 (± 0.324)
Change from baseline at Week 39 (n=7,7,9,23)	0.33 (± 0.350)	0.03 (± 0.518)	-0.10 (± 0.622)	-0.11 (± 0.491)
Change from baseline at Week 52	0.63 (± 0.836)	-0.03 (± 0.814)	-0.53 (± 0.819)	-0.11 (± 0.401)

Statistical analyses

No statistical analyses for this end point

Primary: Summary of participants meeting criteria for potentially clinically significant vital sign values by weight bracket - temperature

End point title	Summary of participants meeting criteria for potentially clinically significant vital sign values by weight bracket - temperature[15][16]

End point description:

Change from baseline in vital signs measurements - temperature (degrees Celsius)

Temperature-Low:<=35ºC(Any Age),High:>=38.4ºC(<18 yrs).

End point type	Primary

End point timeframe:

12 months

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: This is an open label, single dose study - statistical analysis does not apply.

[16] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg >13-17kg	OAV101 1.1e14 vg/kg >17-21kg
Subject group type	Reporting group	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	24	8	9
Units: Participants				
Temperature (ºC) Low	0	0	0	0
Temperature (ºC) High	1	2	0	1

Statistical analyses

No statistical analyses for this end point

Primary: Change from baseline in vital signs measurements - oxygen saturation level

End point title	Change from baseline in vital signs measurements - oxygen saturation level[17][18]

End point description:

Change from baseline in vital signs measurements - oxygen saturation level (%).

Oxygen saturation is the fraction of oxygen-saturated hemoglobin relative to total hemoglobin (unsaturated+saturated) in the blood and then multiplied by 100.

End point type	Primary

End point timeframe:

Baseline, Days 2 and 3, Weeks 1, 2, 3, 4, 6, 8, 10, 13, 26, 39 and 52

Notes:

[17] - 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: This is an open label, single dose study - statistical analysis does not apply.

[18] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: This is an open label, single dose study - statistical analysis does not apply.

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg >13-17kg	OAV101 1.1e14 vg/kg >17-21kg
Subject group type	Reporting group	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	24	8	9
Units: % Oxygen Saturated				
arithmetic mean (standard deviation)				
Change from baseline at Day 2	0.0 (± 0.82)	-0.3 (± 0.99)	-0.3 (± 1.04)	-0.4 (± 1.13)
Change from baseline at Day 3	0.3 (± 1.80)	-0.5 (± 1.41)	-0.1 (± 0.83)	-1.3 (± 1.12)
Change from baseline at Week 1	0.1 (± 1.07)	-0.4 (± 1.47)	-0.4 (± 2.00)	-0.8 (± 1.20)
Change from baseline at Week 2	-0.7 (± 1.89)	-0.8 (± 1.79)	-1.0 (± 1.51)	-0.8 (± 2.11)

Change from baseline at Week 3 (n=6,8,9,23)	-1.0 (± 1.90)	-0.8 (± 1.38)	-0.3 (± 1.28)	-1.1 (± 1.05)
Change from baseline at Week 4 (n=7,8,8,23)	0.0 (± 1.91)	-0.4 (± 1.38)	-0.5 (± 1.31)	-0.8 (± 0.89)
Change from baseline at Week 6 (n=7,8,8,23)	-0.1 (± 1.21)	-0.6 (± 1.31)	-1.0 (± 1.51)	-0.6 (± 1.19)
Change from baseline at Week 8 (n=7,7,9,23)	-0.7 (± 1.89)	-0.4 (± 1.31)	0.0 (± 1.15)	-0.4 (± 0.88)
Change from baseline at Week 10	0.3 (± 1.89)	-0.5 (± 1.72)	-1.0 (± 1.93)	-0.7 (± 1.32)
Change from baseline at Week 13	0.1 (± 1.57)	-0.3 (± 1.43)	-0.5 (± 1.07)	-0.4 (± 1.67)
Change from baseline at Week 26	-0.6 (± 1.51)	-0.3 (± 1.60)	-0.1 (± 1.36)	-0.2 (± 1.99)
Change from baseline at Week 39 (n=7,7,8,22)	-1.0 (± 1.41)	-0.4 (± 1.22)	0.1 (± 0.90)	-0.4 (± 1.19)
Change from baseline at Week 52	-1.0 (± 2.00)	-0.5 (± 1.64)	-0.6 (± 1.51)	-0.1 (± 1.54)

Statistical analyses

No statistical analyses for this end point

Secondary: Achievement of development motor milestones according to the modified and combined WHO-MGRS and Bayley scale of Infant and Toddler Development.

End point title	Achievement of development motor milestones according to the modified and combined WHO-MGRS and Bayley scale of Infant and Toddler Development.

End point description:

The World Health Organization-Multicentre Growth Reference Study (WHO-MGRS) and Bayley scale of Infant and Toddler Development was modified and combined into a single scale expressly for this study, to measure developmental motor milestones. These were assessed via the milestone checklist, formed of 10 yes/no questions with optional video documentation. The developmental milestones are: head control, sitting with support, sitting without support, sitting without support for 30 seconds, hands-and-knees crawling, pulls to stand, standing with assistance, walking with assistance, standing alone and walking alone. A yes response indicates that the patient reached a particular development milestone.

wk = Week
w/out = without

End point type	Secondary

End point timeframe:

Baseline, Week 26 and Week 52

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg >13-17 kg	OAV101 1.1e14 vg/kg >17-21 kg	OAV101 1.1e14 vg/kg Overall
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	7	8	9	24
Units: Participants				
Baseline Head control (Bayley GM #4)	7	8	9	24
Baseline Sits with support (Bayley GM #19)	7	8	9	24
Baseline Sitting without support (WHO MGRS)	6	7	8	21
Baseline Sits w/out support 30 s Bayley GM #26	6	7	8	21

Baseline Hands-and-knees crawling (WHO MGRS)	1	3	6	10
Baseline Pulls to stand (Bayley GM #35)	1	3	5	9
Baseline Standing with assistance (WHO MGRS)	0	2	5	7
Baseline Walking with assistance (WHO MGRS)	0	2	5	7
Baseline Standing alone (WHO MGRS)	0	2	4	6
Baseline Walking alone (WHO MGRS)	0	2	4	6
Week 26 Head control (Bayley GM #4)	7	8	9	24
Week 26 Sits with support (Bayley GM #19)	7	8	9	24
Week 26 Sitting without support (WHO MGRS)	7	5	9	21
Week 26 Sits without support 30 s Bayley GM #26	7	6	8	20
Week 26 Hands-and-knees crawling (WHO MGRS)	2	3	6	11
Week 26 Pulls to stand (Bayley GM #35)	2	3	5	10
Week 26 Standing with assistance (WHO MGRS)	1	3	5	9
Week 26 Walking with assistance (WHO MGRS)	1	3	4	8
Week 26 Standing alone (WHO MGRS)	0	3	4	7
Week 26 Walking alone (WHO MGRS)	0	2	4	6
Week 52 Head control (Bayley GM #4)	7	8	9	24
Week 52 Sits with support (Bayley GM #19)	7	7	9	23
Week 52 Sitting without support (WHO MGRS)	7	7	8	22
Week 52 Sits without support 30 s Bayley GM #26	7	6	8	21
Week 52 Hands-and-knees crawling (WHO MGRS)	2	3	6	11
Wk 52 Pulls to stand (Bayley GM #35) (n=7,8,8,23)	2	3	5	10
Week 52 Standing with assistance (WHO MGRS)	2	3	5	10
Week 52 Walking with assistance (WHO MGRS)	1	3	4	8
Week 52 Standing alone (WHO MGRS)	0	3	4	7
Week 52 Walking alone (WHO MGRS)	0	2	4	6

Statistical analyses

No statistical analyses for this end point

Secondary: Change from baseline in Hammersmith Functional Motor Scale - Expanded (HFMSE), as appropriate according to participant age

End point title	Change from baseline in Hammersmith Functional Motor Scale - Expanded (HFMSE), as appropriate according to participant age

End point description:

The HFMSE was devised for use in children with SMA to give objective information on motor ability and clinical progression. The HFMSE is formed of 33 assessments. Each motor skill item is scored on a 3 point Likert scale from 0 (no response) to 2 (full response), with a total score range of 0 to 66. A higher

score indicates a higher level of ability.

End point type	Secondary

End point timeframe:

Baseline, Week 4, Week 13, Week 26, Week 39 and Week 52

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg >13-17 kg	OAV101 1.1e14 vg/kg >17-21 kg	OAV101 1.1e14 vg/kg Overall
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	5	6	9	20
Units: HFMSE total score on a scale				
arithmetic mean (standard deviation)				
Change from baseline at Week 4 (n=4,6,9,19)	3.8 (± 2.99)	2.3 (± 2.66)	3.1 (± 3.10)	3.0 (± 2.83)
Change from baseline at Week 13 (n=4,6,9,19)	1.3 (± 7.89)	4.5 (± 3.99)	3.9 (± 3.98)	3.5 (± 4.83)
Change from baseline at Week 26 (n=5,6,9,20)	3.4 (± 5.18)	4.3 (± 5.05)	3.1 (± 4.14)	3.6 (± 4.45)
Change from baseline at Week 39 (n=5,5,9,19)	2.6 (± 6.11)	-0.6 (± 4.72)	4.3 (± 3.71)	2.6 (± 4.87)
Change from baseline at Week 52 (n=5,6,7,18)	3.0 (± 5.24)	3.7 (± 5.75)	4.3 (± 4.07)	3.7 (± 4.73)

Statistical analyses

No statistical analyses for this end point

Secondary: Change from baseline in Revised Upper Limb Module (RULM), as appropriate according to participant age.

End point title	Change from baseline in Revised Upper Limb Module (RULM), as appropriate according to participant age.

End point description:

The RULM assesses motor performance in the upper limbs from childhood through adulthood in ambulatory and non-ambulatory individuals with SMA. 'The scale consists of an entry item to establish functional levels and 19 items covering distal to proximal movements. The entry item is a modified version of the Brooke scale, including activities ranging from no functional use of hands (score 0) to full bilateral shoulder abduction (score 6). The entry item does not contribute to the total score but serves as a functional classification of overall upper limb functional ability. Of the remaining 19 items, 18 are scored on a 3 point scoring system and 1 item is scored on a 2 point scoring system. The test is performed unilaterally using the limb preferred by the participant. The total score ranges from 0, if all the items cannot be performed, to 37, if all the activities are achieved fully without any compensation. ' Higher scores indicate higher levels of motor ability.

End point type	Secondary

End point timeframe:

Baseline, Week 4, Week 13, Week 26, Week 39 and Week 52

End point values	OAV101 1.1e14 vg/kg 8.5-13 kg	OAV101 1.1e14 vg/kg >13-17 kg	OAV101 1.1e14 vg/kg >17-21 kg	OAV101 1.1e14 vg/kg 8.5-13 kg
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	4	6	8	18
Units: RULM total score on a scale				
arithmetic mean (standard deviation)				
Change from baseline at Week 4	2.8 (± 2.36)	1.2 (± 1.33)	1.0 (± 1.93)	1.4 (± 1.89)
Change from baseline at Week 13	4.3 (± 1.50)	0.8 (± 2.32)	2.0 (± 2.56)	2.1 (± 2.52)
Change from baseline at Week 26	5.3 (± 2.50)	1.3 (± 1.21)	1.6 (± 2.88)	2.3 (± 2.74)
Change from baseline at Week 39 (n=4,5,8,17)	7.3 (± 2.22)	-0.6 (± 1.34)	2.0 (± 4.34)	2.5 (± 4.29)
Change from baseline at Week 52 (n=3,6,8,17)	6.0 (± 3.46)	0.3 (± 1.63)	1.8 (± 4.71)	2.0 (± 4.02)

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: Adverse events are reported from the single dose of study treatment until end of study, up to maximum duration of 12 months	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	26.0

Reporting groups	
Reporting group title	OAV101 1.1e14 vg/kg 8.5 to 13 kg
Reporting group description: 8.5 to 13 kg	
Reporting group title	OAV101 1.1e14 vg/kg Overall
Reporting group description: Overall	
Reporting group title	OAV101 1.1e14 vg/kg Greater than 17 to 21 kg
Reporting group description: Greater than 17 to 21 kg	
Reporting group title	OAV101 1.1e14 vg/kg Greater than 13 to 17 kg
Reporting group description: Greater than 13 to 17 kg	

Serious adverse events	OAV101 1.1e14 vg/kg 8.5 to 13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg Greater than 17 to 21 kg
Total subjects affected by serious adverse events			
subjects affected / exposed	3 / 7 (42.86%)	15 / 24 (62.50%)	5 / 9 (55.56%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Investigations			
Liver function test abnormal			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Liver function test increased			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	1 / 1	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			

Femur fracture			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Radius fracture			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Thrombocytopenia			
subjects affected / exposed	0 / 7 (0.00%)	3 / 24 (12.50%)	1 / 9 (11.11%)
occurrences causally related to treatment / all	0 / 0	4 / 4	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Bicytopenia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Anaemia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences causally related to treatment / all	0 / 0	1 / 1	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Asthenia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Constipation			

subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Vomiting			
subjects affected / exposed	0 / 7 (0.00%)	3 / 24 (12.50%)	1 / 9 (11.11%)
occurrences causally related to treatment / all	0 / 0	2 / 4	1 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Hepatotoxicity			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences causally related to treatment / all	0 / 0	1 / 1	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hypertransaminasaemia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences causally related to treatment / all	0 / 0	1 / 2	1 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatic cytolysis			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Urinary retention			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Hip deformity			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
COVID-19			

subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	2 / 9 (22.22%)
occurrences causally related to treatment / all	0 / 0	0 / 2	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
COVID-19 pneumonia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis viral			
subjects affected / exposed	1 / 7 (14.29%)	2 / 24 (8.33%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 3	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	2 / 9 (22.22%)
occurrences causally related to treatment / all	0 / 0	0 / 2	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory tract infection			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Subglottic laryngitis			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Varicella			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			
Hypoglycaemia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Dehydration			

subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	OAV101 1.1e14 vg/kg Greater than 13 to 17 kg		
Total subjects affected by serious adverse events			
subjects affected / exposed	7 / 8 (87.50%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Investigations			
Liver function test abnormal			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Liver function test increased			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Femur fracture			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Radius fracture			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Blood and lymphatic system disorders			
Thrombocytopenia			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences causally related to treatment / all	3 / 3		
deaths causally related to treatment / all	0 / 0		
Bicytopenia			

subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Anaemia			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Asthenia			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Constipation			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Vomiting			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences causally related to treatment / all	1 / 2		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Hepatotoxicity			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Hypertransaminasaemia			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		

Hepatic cytolysis			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Urinary retention			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Hip deformity			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
COVID-19			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
COVID-19 pneumonia			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastroenteritis viral			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Respiratory tract infection			

subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Subglottic laryngitis			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Varicella			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Metabolism and nutrition disorders			
Hypoglycaemia			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Dehydration			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	OAV101 1.1e14 vg/kg 8.5 to 13 kg	OAV101 1.1e14 vg/kg Overall	OAV101 1.1e14 vg/kg Greater than 17 to 21 kg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	7 / 7 (100.00%)	24 / 24 (100.00%)	9 / 9 (100.00%)
General disorders and administration site conditions			
Malaise			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Pyrexia			
subjects affected / exposed	4 / 7 (57.14%)	10 / 24 (41.67%)	5 / 9 (55.56%)
occurrences (all)	10	18	5
Immune system disorders			

Hypersensitivity			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Respiratory, thoracic and mediastinal disorders			
Nocturnal dyspnoea			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Nasal congestion			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Epistaxis			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	2	0
Cough			
subjects affected / exposed	3 / 7 (42.86%)	3 / 24 (12.50%)	0 / 9 (0.00%)
occurrences (all)	5	5	0
Upper respiratory tract congestion			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	5	5
Sneezing			
subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	0 / 9 (0.00%)
occurrences (all)	0	2	0
Oropharyngeal pain			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Respiratory tract congestion			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Psychiatric disorders			
Irritability			
subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	2 / 9 (22.22%)
occurrences (all)	0	2	2
Insomnia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Hallucination			

subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	1 / 7 (14.29%)	2 / 24 (8.33%)	1 / 9 (11.11%)
occurrences (all)	2	3	1
Aspartate aminotransferase increased			
subjects affected / exposed	1 / 7 (14.29%)	2 / 24 (8.33%)	1 / 9 (11.11%)
occurrences (all)	2	3	1
Blood lactate dehydrogenase increased			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Blood potassium abnormal			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Blood urea increased			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Electrocardiogram T wave inversion			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Platelet count abnormal			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Liver function test increased			
subjects affected / exposed	1 / 7 (14.29%)	2 / 24 (8.33%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Liver function test abnormal			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Hepatic enzyme increased			
subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	0 / 9 (0.00%)
occurrences (all)	0	2	0
Haemoglobin decreased			

subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Platelet count decreased			
subjects affected / exposed	3 / 7 (42.86%)	7 / 24 (29.17%)	2 / 9 (22.22%)
occurrences (all)	3	7	2
Transaminases increased			
subjects affected / exposed	3 / 7 (42.86%)	6 / 24 (25.00%)	1 / 9 (11.11%)
occurrences (all)	3	7	1
Weight increased			
subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	1 / 9 (11.11%)
occurrences (all)	0	2	1
Prothrombin time prolonged			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Joint dislocation			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Procedural nausea			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Procedural pain			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Stoma site erythema			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Torus fracture			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Cardiac disorders			

Bradycardia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Tachycardia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Pericardial effusion			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Nervous system disorders			
Dizziness			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Headache			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Lethargy			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Tremor			
subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	0 / 9 (0.00%)
occurrences (all)	0	2	0
Blood and lymphatic system disorders			
Thrombocytopenia			
subjects affected / exposed	1 / 7 (14.29%)	5 / 24 (20.83%)	2 / 9 (22.22%)
occurrences (all)	1	5	2
Anaemia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Eye disorders			
Blepharitis			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Eye irritation			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Periorbital swelling			

subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	2 / 7 (28.57%)	2 / 24 (8.33%)	0 / 9 (0.00%)
occurrences (all)	2	2	0
Abdominal pain upper			
subjects affected / exposed	0 / 7 (0.00%)	4 / 24 (16.67%)	2 / 9 (22.22%)
occurrences (all)	0	5	2
Constipation			
subjects affected / exposed	1 / 7 (14.29%)	3 / 24 (12.50%)	0 / 9 (0.00%)
occurrences (all)	1	5	0
Diarrhoea			
subjects affected / exposed	2 / 7 (28.57%)	3 / 24 (12.50%)	0 / 9 (0.00%)
occurrences (all)	3	4	0
Faecaloma			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Gastritis			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Gastrooesophageal reflux disease			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Leukoplakia oral			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Nausea			
subjects affected / exposed	3 / 7 (42.86%)	8 / 24 (33.33%)	3 / 9 (33.33%)
occurrences (all)	3	8	3
Upper gastrointestinal haemorrhage			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Vomiting			
subjects affected / exposed	5 / 7 (71.43%)	17 / 24 (70.83%)	7 / 9 (77.78%)
occurrences (all)	6	25	10

Hepatobiliary disorders			
Hypertransaminasaemia			
subjects affected / exposed	1 / 7 (14.29%)	7 / 24 (29.17%)	5 / 9 (55.56%)
occurrences (all)	1	11	7
Skin and subcutaneous tissue disorders			
Eczema infantile			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Dermatitis			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Rash erythematous			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Rash			
subjects affected / exposed	1 / 7 (14.29%)	2 / 24 (8.33%)	0 / 9 (0.00%)
occurrences (all)	1	2	0
Hypertrichosis			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Renal and urinary disorders			
Pollakiuria			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Endocrine disorders			
Cushingoid			
subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	1 / 9 (11.11%)
occurrences (all)	0	2	1
Cushing's syndrome			
subjects affected / exposed	1 / 7 (14.29%)	3 / 24 (12.50%)	2 / 9 (22.22%)
occurrences (all)	1	3	2
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	0 / 7 (0.00%)	3 / 24 (12.50%)	3 / 9 (33.33%)
occurrences (all)	0	3	3
Pain in extremity			

subjects affected / exposed	0 / 7 (0.00%)	3 / 24 (12.50%)	3 / 9 (33.33%)
occurrences (all)	0	4	4
Osteopenia			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Muscular weakness			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	2	0
Back pain			
subjects affected / exposed	0 / 7 (0.00%)	2 / 24 (8.33%)	1 / 9 (11.11%)
occurrences (all)	0	2	1
Infections and infestations			
Rhinitis			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
COVID-19			
subjects affected / exposed	1 / 7 (14.29%)	7 / 24 (29.17%)	2 / 9 (22.22%)
occurrences (all)	1	7	2
Conjunctivitis			
subjects affected / exposed	1 / 7 (14.29%)	2 / 24 (8.33%)	0 / 9 (0.00%)
occurrences (all)	1	2	0
Gastroenteritis			
subjects affected / exposed	1 / 7 (14.29%)	3 / 24 (12.50%)	2 / 9 (22.22%)
occurrences (all)	1	5	4
Infectious mononucleosis			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Influenza			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Nasopharyngitis			
subjects affected / exposed	2 / 7 (28.57%)	5 / 24 (20.83%)	1 / 9 (11.11%)
occurrences (all)	2	5	1
Otitis media			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1

Pneumonia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0
Respiratory tract infection			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Upper respiratory tract infection			
subjects affected / exposed	4 / 7 (57.14%)	8 / 24 (33.33%)	2 / 9 (22.22%)
occurrences (all)	5	11	4
Urinary tract infection			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Viral skin infection			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	1 / 7 (14.29%)	2 / 24 (8.33%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Dehydration			
subjects affected / exposed	1 / 7 (14.29%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	1	1	0
Vitamin D deficiency			
subjects affected / exposed	0 / 7 (0.00%)	1 / 24 (4.17%)	0 / 9 (0.00%)
occurrences (all)	0	1	0

Non-serious adverse events	OAV101 1.1e14 vg/kg Greater than 13 to 17 kg		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	8 / 8 (100.00%)		
General disorders and administration site conditions			
Malaise			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Pyrexia			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	3		

Immune system disorders			
Hypersensitivity			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Respiratory, thoracic and mediastinal disorders			
Nocturnal dyspnoea			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Nasal congestion			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Epistaxis			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	2		
Cough			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Upper respiratory tract congestion			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Sneezing			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences (all)	2		
Oropharyngeal pain			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Respiratory tract congestion			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Psychiatric disorders			
Irritability			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Insomnia			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		

Hallucination			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Aspartate aminotransferase increased			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Blood lactate dehydrogenase increased			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Blood potassium abnormal			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Blood urea increased			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Electrocardiogram T wave inversion			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Platelet count abnormal			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Liver function test increased			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Liver function test abnormal			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Hepatic enzyme increased			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences (all)	2		
Haemoglobin decreased			

subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Platelet count decreased			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences (all)	2		
Transaminases increased			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences (all)	3		
Weight increased			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Prothrombin time prolonged			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Joint dislocation			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Procedural nausea			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Procedural pain			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Stoma site erythema			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Torus fracture			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Cardiac disorders			

Bradycardia subjects affected / exposed occurrences (all)	1 / 8 (12.50%) 1		
Tachycardia subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Pericardial effusion subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Nervous system disorders Dizziness subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Headache subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Lethargy subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Tremor subjects affected / exposed occurrences (all)	2 / 8 (25.00%) 2		
Blood and lymphatic system disorders Thrombocytopenia subjects affected / exposed occurrences (all)	2 / 8 (25.00%) 2		
Anaemia subjects affected / exposed occurrences (all)	1 / 8 (12.50%) 1		
Eye disorders Blepharitis subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Eye irritation subjects affected / exposed occurrences (all)	1 / 8 (12.50%) 1		
Periorbital swelling			

subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Abdominal pain upper			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences (all)	3		
Constipation			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences (all)	4		
Diarrhoea			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Faecaloma			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Gastritis			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Gastrooesophageal reflux disease			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Leukoplakia oral			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Nausea			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences (all)	2		
Upper gastrointestinal haemorrhage			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Vomiting			
subjects affected / exposed	5 / 8 (62.50%)		
occurrences (all)	9		

Hepatobiliary disorders Hypertransaminasaemia subjects affected / exposed occurrences (all)	 1 / 8 (12.50%) 3		
Skin and subcutaneous tissue disorders Eczema infantile subjects affected / exposed occurrences (all) Dermatitis subjects affected / exposed occurrences (all) Rash erythematous subjects affected / exposed occurrences (all) Rash subjects affected / exposed occurrences (all) Hypertrichosis subjects affected / exposed occurrences (all)	 0 / 8 (0.00%) 0 0 / 8 (0.00%) 0 0 / 8 (0.00%) 0 1 / 8 (12.50%) 1 1 / 8 (12.50%) 1		
Renal and urinary disorders Pollakiuria subjects affected / exposed occurrences (all)	 1 / 8 (12.50%) 1		
Endocrine disorders Cushingoid subjects affected / exposed occurrences (all) Cushing's syndrome subjects affected / exposed occurrences (all)	 1 / 8 (12.50%) 1 0 / 8 (0.00%) 0		
Musculoskeletal and connective tissue disorders Arthralgia subjects affected / exposed occurrences (all) Pain in extremity	 0 / 8 (0.00%) 0 		

subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Osteopenia			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Muscular weakness			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	2		
Back pain			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Infections and infestations			
Rhinitis			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
COVID-19			
subjects affected / exposed	4 / 8 (50.00%)		
occurrences (all)	4		
Conjunctivitis			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Gastroenteritis			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		
Infectious mononucleosis			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Influenza			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Nasopharyngitis			
subjects affected / exposed	2 / 8 (25.00%)		
occurrences (all)	2		
Otitis media			
subjects affected / exposed	0 / 8 (0.00%)		
occurrences (all)	0		

Pneumonia subjects affected / exposed occurrences (all)	1 / 8 (12.50%) 1		
Respiratory tract infection subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Upper respiratory tract infection subjects affected / exposed occurrences (all)	2 / 8 (25.00%) 2		
Urinary tract infection subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Viral skin infection subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Metabolism and nutrition disorders Decreased appetite subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Dehydration subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0		
Vitamin D deficiency subjects affected / exposed occurrences (all)	1 / 8 (12.50%) 1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
28 February 2022	The main purpose of the amendment was to remove virus serology form time points past screening in the Schedule of Assessments. Other changes included adding complement panel, optional pharmacogenetics and ACEND assessment as exploratory endpoints. In addition, protocol was edited for consistency and clarification purposes.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported