



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) | EMA-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: \leq 5th percentile of the age(Any Age), High: \geq 90th percentile of the age, gender, and height group (<18 yrs).

Diastolic Blood Pressure-High: \geq 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)

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|---|--|
| 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)

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|-----------------|--|
| 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: ≤35°C(Any Age),High: ≥38.4°C(<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 (°C) Low | 0 | 0 | 0 | 0 |
| Temperature (°C) 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 occurrences (all) | 1 / 7 (14.29%) 1 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Respiratory, thoracic and mediastinal disorders | | | |
| Nocturnal dyspnoea subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Nasal congestion subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Epistaxis subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 2 | 0 / 9 (0.00%) 0 |
| Cough subjects affected / exposed occurrences (all) | 3 / 7 (42.86%) 5 | 3 / 24 (12.50%) 5 | 0 / 9 (0.00%) 0 |
| Upper respiratory tract congestion subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 5 | 1 / 9 (11.11%) 5 |
| Sneezing subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 2 / 24 (8.33%) 2 | 0 / 9 (0.00%) 0 |
| Oropharyngeal pain subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Respiratory tract congestion subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Psychiatric disorders | | | |
| Irritability subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 2 / 24 (8.33%) 2 | 2 / 9 (22.22%) 2 |
| Insomnia subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 1 / 9 (11.11%) 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 occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Tachycardia subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 1 / 9 (11.11%) 1 |
| Pericardial effusion subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Nervous system disorders Dizziness subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 1 / 9 (11.11%) 1 |
| Headache subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 1 / 9 (11.11%) 1 |
| Lethargy subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Tremor subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 2 / 24 (8.33%) 2 | 0 / 9 (0.00%) 0 |
| Blood and lymphatic system disorders Thrombocytopenia subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 5 / 24 (20.83%) 5 | 2 / 9 (22.22%) 2 |
| Anaemia subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Eye disorders Blepharitis subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 1 / 9 (11.11%) 1 |
| Eye irritation subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Periorbital swelling | | | |

| | | | |
|--|---------------------|---------------------|--------------------|
| subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 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 occurrences (all) | 0 / 7 (0.00%) 0 | 3 / 24 (12.50%) 4 | 3 / 9 (33.33%) 4 |
| Osteopenia subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Muscular weakness subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 2 | 0 / 9 (0.00%) 0 |
| Back pain subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 2 / 24 (8.33%) 2 | 1 / 9 (11.11%) 1 |
| Infections and infestations | | | |
| Rhinitis subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| COVID-19 subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 7 / 24 (29.17%) 7 | 2 / 9 (22.22%) 2 |
| Conjunctivitis subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 2 / 24 (8.33%) 2 | 0 / 9 (0.00%) 0 |
| Gastroenteritis subjects affected / exposed occurrences (all) | 1 / 7 (14.29%) 1 | 3 / 24 (12.50%) 5 | 2 / 9 (22.22%) 4 |
| Infectious mononucleosis subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Influenza subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 0 / 9 (0.00%) 0 |
| Nasopharyngitis subjects affected / exposed occurrences (all) | 2 / 7 (28.57%) 2 | 5 / 24 (20.83%) 5 | 1 / 9 (11.11%) 1 |
| Otitis media subjects affected / exposed occurrences (all) | 0 / 7 (0.00%) 0 | 1 / 24 (4.17%) 1 | 1 / 9 (11.11%) 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 occurrences (all) | 0 / 8 (0.00%) 0 | | |
| Investigations | | | |
| Alanine aminotransferase increased subjects affected / exposed occurrences (all) | 0 / 8 (0.00%) 0 | | |
| Aspartate aminotransferase increased subjects affected / exposed occurrences (all) | 0 / 8 (0.00%) 0 | | |
| Blood lactate dehydrogenase increased subjects affected / exposed occurrences (all) | 0 / 8 (0.00%) 0 | | |
| Blood potassium abnormal subjects affected / exposed occurrences (all) | 1 / 8 (12.50%) 1 | | |
| Blood urea increased subjects affected / exposed occurrences (all) | 0 / 8 (0.00%) 0 | | |
| Electrocardiogram T wave inversion subjects affected / exposed occurrences (all) | 1 / 8 (12.50%) 1 | | |
| Platelet count abnormal subjects affected / exposed occurrences (all) | 0 / 8 (0.00%) 0 | | |
| Liver function test increased subjects affected / exposed occurrences (all) | 0 / 8 (0.00%) 0 | | |
| Liver function test abnormal subjects affected / exposed occurrences (all) | 1 / 8 (12.50%) 1 | | |
| Hepatic enzyme increased subjects affected / exposed occurrences (all) | 2 / 8 (25.00%) 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) Headache subjects affected / exposed occurrences (all) Lethargy subjects affected / exposed occurrences (all) Tremor subjects affected / exposed occurrences (all) | 0 / 8 (0.00%) 0 0 / 8 (0.00%) 0 0 / 8 (0.00%) 0 2 / 8 (25.00%) 2 | | |
| Blood and lymphatic system disorders Thrombocytopenia subjects affected / exposed occurrences (all) Anaemia subjects affected / exposed occurrences (all) | 2 / 8 (25.00%) 2 1 / 8 (12.50%) 1 | | |
| Eye disorders Blepharitis subjects affected / exposed occurrences (all) Eye irritation subjects affected / exposed occurrences (all) Periorbital swelling | 0 / 8 (0.00%) 0 1 / 8 (12.50%) 1 | | |

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

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