



## Clinical trial results:

### A Phase 2 Open-Label Multicenter Study to Evaluate the Efficacy, Safety, and Pharmacokinetics of Nedosiran in Pediatric Patients from Birth to 11 Years of Age with Primary Hyperoxaluria and Relatively Intact Renal Function

#### Summary

|                          |                  |
|--------------------------|------------------|
| EudraCT number           | 2021-001083-16   |
| Trial protocol           | DE ES PL IT      |
| Global end of trial date | 05 February 2025 |

#### Results information

|                                |                |
|--------------------------------|----------------|
| Result version number          | v1 (current)   |
| This version publication date  | 22 August 2025 |
| First version publication date | 22 August 2025 |

#### Trial information

##### Trial identification

|                       |              |
|-----------------------|--------------|
| Sponsor protocol code | DCR-PHXC-203 |
|-----------------------|--------------|

##### Additional study identifiers

|                                    |             |
|------------------------------------|-------------|
| ISRCTN number                      | -           |
| ClinicalTrials.gov id (NCT number) | NCT05001269 |
| WHO universal trial number (UTN)   | -           |

Notes:

#### Sponsors

|                              |   |
|------------------------------|---|
| Sponsor organisation name    | Dicerna Pharmaceuticals, Inc., a Novo Nordisk company   |
| Sponsor organisation address | Novo Nordisk, Novo Allé, Bagsvaerd, Denmark, 2880   |
| Public contact               | Clinical Reporting Office (2834), Dicerna Pharmaceuticals, Inc., a Novo Nordisk company, clinicaltrials@novonordisk.com |
| Scientific contact           | Clinical Reporting Office (2834), Dicerna Pharmaceuticals, Inc., a Novo Nordisk company, clinicaltrials@novonordisk.com |

Notes:

#### Paediatric regulatory details

|  |                     |
|--|---------------------|
| Is trial part of an agreed paediatric investigation plan (PIP)       | Yes                 |
| EMA paediatric investigation plan number(s)                          | EMA-002493-PIP01-18 |
| 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                       | 15 May 2025      |
| Is this the analysis of the primary completion data? | No               |
| Global end of trial reached?                         | Yes              |
| Global end of trial date                             | 05 February 2025 |
| Was the trial ended prematurely?                     | No               |

Notes:

## General information about the trial

Main objective of the trial:

Main objective of the study trial is to assess the efficacy of nedosiran in neonates, infants, and children with PH and relatively intact renal function based upon eGFR and serum creatinine

Protection of trial subjects:

This study will be conducted in accordance with the protocol and with the consensus ethical principles derived from international guidelines including the Declaration of Helsinki and CIOMS International Ethical Guidelines (CIOMS VI, 2005); applicable International Conference on Harmonisation (ICH) Good Clinical practices (GCP) guidelines, and applicable laws and regulations, including privacy laws.

With regard to paediatric participants, the ICH Harmonised Tripartite Guideline: Clinical Investigation of Medicinal Products in the Paediatric Population (E11, 20 July 2000) and the European Commission Ethical Considerations for Clinical Trials on Medicinal Products Conducted with Minors (18 September 2017) were considered during the design of the trial, and blood volume minimized where possible.

Background therapy: -

Evidence for comparator: -

|   |                  |
|---|------------------|
| Actual start date of recruitment                          | 22 February 2022 |
| 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 | United States: 5        |
| Country: Number of subjects enrolled | Canada: 1               |
| Country: Number of subjects enrolled | Spain: 1                |
| Country: Number of subjects enrolled | Germany: 4              |
| Country: Number of subjects enrolled | United Kingdom: 1       |
| Country: Number of subjects enrolled | Japan: 1                |
| Country: Number of subjects enrolled | Lebanon: 9              |
| Country: Number of subjects enrolled | Türkiye: 3              |
| Country: Number of subjects enrolled | United Arab Emirates: 2 |
| Worldwide total number of subjects   | 27                      |
| EEA total number of subjects         | 5                       |

Notes:

| <b>Subjects enrolled per age group</b>    |    |
|---|----|
| In utero                                  | 0  |
| Preterm newborn - gestational age < 37 wk | 0  |
| Newborns (0-27 days)                      | 0  |
| Infants and toddlers (28 days-23 months)  | 5  |
| Children (2-11 years)                     | 22 |
| 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:

The study was conducted at 13 sites in 9 countries.

### Pre-assignment

Screening details:

This is Phase 2, open-label, single-arm uncontrolled study in paediatric subjects with genetically confirmed primary hyperoxaluria (PH), with relatively intact renal function based upon estimated glomerular filtration rate (eGFR) and serum creatinine. A total of 27 subjects were enrolled in the study and all of them completed the study.

### Period 1

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

### Arms

|                              |                        |
|------------------------------|------------------------|
| Are arms mutually exclusive? | Yes                    |
| <b>Arm title</b>             | Children 0 to <2 years |

Arm description:

All subjects that included neonates (0 to less than [ $<$ ] 2 years) with primary hyperoxaluria (PH) and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 milligrams per kilogram [mg/kg], not to exceed 170 milligrams [mg]), subcutaneously from Day 1 though Month 6.

|  |                     |
|--|---------------------|
| Arm type                               | Experimental        |
| Investigational medicinal product name | Nedosiran           |
| Investigational medicinal product code | DCR-PHXC            |
| Other name                             |                     |
| Pharmaceutical forms                   | Sterile concentrate |
| Routes of administration               | Subcutaneous use    |

Dosage and administration details:

Subject were administered a sterile formulation of drug substance (nedosiran sodium) in water for injection, intended for subcutaneous administration. Total monthly dose (3.5 mg/kg, not to exceed 170 mg) was calculated based upon body weight on Day 1 of the study that remained constant throughout the study.

|                  |                        |
|------------------|------------------------|
| <b>Arm title</b> | Children 2 to <6 years |
|------------------|------------------------|

Arm description:

All subjects that included infants (2 to <6 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.

|  |                     |
|--|---------------------|
| Arm type                               | Experimental        |
| Investigational medicinal product name | Nedosiran           |
| Investigational medicinal product code | DCR-PHXC            |
| Other name                             |                     |
| Pharmaceutical forms                   | Sterile concentrate |
| Routes of administration               | Subcutaneous use    |

Dosage and administration details:

Subject were administered a sterile formulation of drug substance (nedosiran sodium) in water for injection, intended for subcutaneous administration. Total monthly dose (3.5 mg/kg, not to exceed 170 mg) was calculated based upon body weight on Day 1 of the study that remained constant throughout the study.

|                  |                        |
|------------------|------------------------|
| <b>Arm title</b> | Children 6 to 11 years |
|------------------|------------------------|

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**Arm description:**

All subjects that included children (6 to 11 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.

|  |                     |
|--|---------------------|
| Arm type                               | Experimental        |
| Investigational medicinal product name | Nedosiran           |
| Investigational medicinal product code | DCR-PHXC            |
| Other name                             |                     |
| Pharmaceutical forms                   | Sterile concentrate |
| Routes of administration               | Subcutaneous use    |

**Dosage and administration details:**

Subject were administered a sterile formulation of drug substance (nedosiran sodium) in water for injection, intended for subcutaneous administration. Total monthly dose (3.5 mg/kg, not to exceed 170 mg) was calculated based upon body weight on Day 1 of the study that remained constant throughout the study.

| <b>Number of subjects in period 1</b> | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |
|---------------------------------------|------------------------|------------------------|------------------------|
| Started                               | 5                      | 13                     | 9                      |
| Modified Intent-To-Treat (MITT)       | 3 <sup>[1]</sup>       | 9 <sup>[2]</sup>       | 8 <sup>[3]</sup>       |
| Safety                                | 5                      | 13                     | 9                      |
| Completed                             | 5                      | 13                     | 9                      |

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**Notes:**

[1] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: This milestone reports MITT population with 3 subjects reported under 0 to <2 years age group.

[2] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: This milestone reports MITT population with 9 subjects reported under 2 to <6 years age group.

[3] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: This milestone reports MITT population with 3 subjects reported under 6 to 11 years age group.

## Baseline characteristics

### Reporting groups

|  |                        |
|--|------------------------|
| Reporting group title  | Children 0 to <2 years |
| Reporting group description:<br>All subjects that included neonates (0 to less than [<] 2 years) with primary hyperoxaluria (PH) and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 milligrams per kilogram [mg/kg], not to exceed 170 milligrams [mg]), subcutaneously from Day 1 though Month 6. |                        |
| Reporting group title  | Children 2 to <6 years |
| Reporting group description:<br>All subjects that included infants (2 to <6 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.  |                        |
| Reporting group title  | Children 6 to 11 years |
| Reporting group description:<br>All subjects that included children (6 to 11 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.   |                        |

| Reporting group values                             | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |
|--|------------------------|------------------------|------------------------|
| Number of subjects                                 | 5                      | 13                     | 9                      |
| Age Categorical<br>Units: Subjects                 |                        |                        |                        |
| In utero   | 0                      | 0                      | 0                      |
| Preterm newborn infants (gestational age < 37 wks) | 0                      | 0                      | 0                      |
| Newborns (0-27 days)                               | 0                      | 0                      | 0                      |
| Infants and toddlers (28 days-23 months)           | 5                      | 0                      | 0                      |
| Children (2-11 years)                              | 0                      | 13                     | 9                      |
| Adolescents (12-17 years)                          | 0                      | 0                      | 0                      |
| Adults (18-64 years)                               | 0                      | 0                      | 0                      |
| From 65-84 years                                   | 0                      | 0                      | 0                      |
| 85 years and over                                  | 0                      | 0                      | 0                      |
| Age Continuous<br>Units: years                     |                        |                        |                        |
| arithmetic mean                                    | 1.30                   | 3.54                   | 7.78                   |
| standard deviation                                 | ± 0.529                | ± 1.198                | ± 1.093                |
| Gender Categorical<br>Units: Subjects              |                        |                        |                        |
| Female   | 1                      | 6                      | 4                      |
| Male   | 4                      | 7                      | 5                      |

| Reporting group values                             | Total |  |  |
|--|-------|--|--|
| Number of subjects                                 | 27    |  |  |
| Age Categorical<br>Units: Subjects                 |       |  |  |
| In utero   | 0     |  |  |
| Preterm newborn infants (gestational age < 37 wks) | 0     |  |  |

|  |    |  |  |
|--|----|--|--|
| Newborns (0-27 days)                     | 0  |  |  |
| Infants and toddlers (28 days-23 months) | 5  |  |  |
| Children (2-11 years)                    | 22 |  |  |
| Adolescents (12-17 years)                | 0  |  |  |
| Adults (18-64 years)                     | 0  |  |  |
| From 65-84 years                         | 0  |  |  |
| 85 years and over                        | 0  |  |  |
| Age Continuous                           |    |  |  |
| Units: years                             |    |  |  |
| arithmetic mean                          |    |  |  |
| standard deviation                       | -  |  |  |
| Gender Categorical                       |    |  |  |
| Units: Subjects                          |    |  |  |
| Female                                   | 11 |  |  |
| Male                                     | 16 |  |  |

## End points

### End points reporting groups

|  |                        |
|--|------------------------|
| Reporting group title  | Children 0 to <2 years |
| Reporting group description:<br>All subjects that included neonates (0 to less than [ $<$ ] 2 years) with primary hyperoxaluria (PH) and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 milligrams per kilogram [mg/kg], not to exceed 170 milligrams [mg]), subcutaneously from Day 1 though Month 6. |                        |
| Reporting group title  | Children 2 to <6 years |
| Reporting group description:<br>All subjects that included infants (2 to <6 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.  |                        |
| Reporting group title  | Children 6 to 11 years |
| Reporting group description:<br>All subjects that included children (6 to 11 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.   |                        |

### Primary: Percent change from Baseline to Month 6 in spot urinary oxalate-to-creatinine ratio in PH1, PH2, or PH3 subject subgroups

|   |  |
|---|--|
| End point title   | Percent change from Baseline to Month 6 in spot urinary oxalate-to-creatinine ratio in PH1, PH2, or PH3 subject subgroups <sup>[1]</sup> |
| End point description:<br>This endpoint reported percent change from baseline to Month 6 in spot urinary oxalate-to-creatinine ratio in paediatric subjects (birth to 11 years of age) with genetically confirmed primary hyperoxaluria type 1 (PH1), primary hyperoxaluria type 2 (PH2), or primary hyperoxaluria type 3 (PH3) subgroups. The MITT Population included all subjects who received at least 1 dose of study intervention and have at least 1 post-baseline spot urinary oxalate to creatinine ratio. Here 'Number of subject analysed' signified 'Overall number of subjects analysed' and 'n' signified 'Number Analysed' that is number of subjects with available data for particular timepoint, for the respective arms; and "99999" signifies that data for these categories were not evaluable as the number of subject for the respective category was 0. |  |
| End point type  | Primary  |
| End point timeframe:<br>Baseline (Week 0), Month 6  |  |
| Notes:<br>[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.<br>Justification: No statistical hypotheses confirmatory testing was planned in this study. The endpoint is descriptive in nature.  |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: Percent change                |                        |                        |                        |  |
| arithmetic mean (standard deviation) |                        |                        |                        |  |
| PH1 (n=3,9,8)                        | -74.06 ( $\pm$ 13.193) | -68.34 ( $\pm$ 8.574)  | -61.44 ( $\pm$ 24.565) |  |
| PH2 (n=0,4,1)                        | 99999 ( $\pm$ 99999)   | -17.98 ( $\pm$ 37.847) | -16.10 ( $\pm$ 0)      |  |
| PH3 (n=2,0,0)                        | -41.43 ( $\pm$ 13.633) | 99999 ( $\pm$ 99999)   | 99999 ( $\pm$ 99999)   |  |



## Statistical analyses

No statistical analyses for this end point

### Primary: Absolute change from Baseline to Month 6 in spot urinary oxalate-to-creatinine ratio in PH1, PH2, or PH3 subject subgroups

|                 |   |
|-----------------|---|
| End point title | Absolute change from Baseline to Month 6 in spot urinary oxalate-to-creatinine ratio in PH1, PH2, or PH3 subject subgroups <sup>[2]</sup> |
|-----------------|---|

End point description:

This endpoint reported absolute change from baseline to Month 6 in spot urinary oxalate-to-creatinine ratio in paediatric subjects (birth to 11 years of age) with genetically confirmed PH1, PH2, or PH3 subgroups. The MITT Population included all subjects who received at least 1 dose of study intervention and have at least 1 post-baseline spot urinary oxalate to creatinine ratio. Here 'Number of subject analysed' signified 'Overall number of subjects analysed' and 'n' signified 'Number Analysed' that is number of subjects with available data for particular timepoint, for the respective arms; and "99999" signifies that data for these categories were not evaluable as the number of subject for the respective category was 0.

|                |         |
|----------------|---------|
| End point type | Primary |
|----------------|---------|

End point timeframe:

Baseline (Week 0), Month 6

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: No statistical hypotheses confirmatory testing was planned in this study. The endpoint is descriptive in nature.

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: micromole/millimole           |                        |                        |                        |  |
| arithmetic mean (standard deviation) |                        |                        |                        |  |
| PH1 (n=3,9,8)                        | -563.972 (± 378.3308)  | -316.344 (± 150.6332)  | -189.106 (± 120.5939)  |  |
| PH2 (n=0,4,1)                        | 99999 (± 99999)        | -64.458 (± 92.5196)    | -27.583 (± 0)          |  |
| PH3 (n=2,0,0)                        | -135.283 (± 17.1591)   | 99999 (± 99999)        | 99999 (± 99999)        |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Number of Treatment Emergent Adverse Events and Serious Adverse Events-Nature

|                 |   |
|-----------------|---|
| End point title | Number of Treatment Emergent Adverse Events and Serious Adverse Events-Nature |
|-----------------|---|

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**End point description:**

This endpoint reported nature of TEAEs and SAEs. An AE is any untoward medical occurrence in clinical study subject, temporally associated with the use of study drug, whether or not considered related to the study drug. An SAE is any untoward medical occurrence that, at any dose results in death, is life-threatening, requires inpatient hospitalisation or prolongation of existing hospitalisation, results in persistent disability/incapacity, is a congenital anomaly/birth defect, and medical events. An AE is treatment emergent if they have an onset or worsen in severity after a subject receives the study drug. TEAEs are considered as leading to discontinuation if the action taken is marked as "drug withdrawn" on the case report form. TEAEs of special interest include injection site reactions, muscle pain and weakness, and kidney stone events. Safety population included all subjects who received at least 1 dose of study intervention.

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|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

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**End point timeframe:**

From baseline (Week 0) up to Month 6

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| End point values                          | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|---|------------------------|------------------------|------------------------|--|
| Subject group type                        | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed               | 5                      | 13                     | 9                      |  |
| Units: Events                             |                        |                        |                        |  |
| Treatment Related TEAE                    | 2                      | 7                      | 4                      |  |
| TEAE Leading to Treatment Interruption    | 1                      | 0                      | 0                      |  |
| TEAE Leading to Treatment Discontinuation | 0                      | 0                      | 0                      |  |
| Serious Treatment Related TEAE            | 0                      | 0                      | 0                      |  |
| Fatal TEAE                                | 0                      | 0                      | 0                      |  |
| TEAE of Special Interest                  | 7                      | 8                      | 8                      |  |

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**Statistical analyses**

No statistical analyses for this end point

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**Secondary: Number of Treatment Emergent Adverse Events (TEAEs) and Serious Adverse Events (SAEs)**

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|                 |   |
|-----------------|---|
| End point title | Number of Treatment Emergent Adverse Events (TEAEs) and Serious Adverse Events (SAEs) |
|-----------------|---|

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**End point description:**

This endpoint reported number of incidents of TEAEs and SAEs. An adverse event (AE) is any untoward medical occurrence in a patient or clinical study subject, temporally associated with the use of study intervention, whether or not considered related to the study intervention. An SAE is defined as any untoward medical occurrence that, at any dose results in death, is life-threatening, requires inpatient hospitalization or prolongation of existing hospitalization, results in persistent disability/incapacity, is a congenital anomaly/birth defect, and medical events. An AE will be defined as treatment emergent if they have an onset or worsen in severity after a subject receives the study intervention. Safety population included all subjects who received at least 1 dose of study intervention.

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|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

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**End point timeframe:**

From baseline (Week 0) up to Month 6

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| End point values            | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|-----------------------------|------------------------|------------------------|------------------------|--|
| Subject group type          | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed | 5                      | 13                     | 9                      |  |
| Units: Events               |                        |                        |                        |  |
| TEAEs                       | 37                     | 66                     | 38                     |  |
| SAEs                        | 1                      | 4                      | 2                      |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in 12-lead Electrocardiogram (ECG)- ECG Mean Heart Rate

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in 12-lead Electrocardiogram (ECG)- ECG Mean Heart Rate |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in ECG mean heart rate. Safety population included all subjects who received at least 1 dose of study intervention.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: beats/minute                  |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -11.6 (± 32.34)        | -4.2 (± 15.73)         | 6.2 (± 16.98)          |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in 12-lead ECG- RR Interval

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in 12-lead ECG- RR Interval |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in RR Interval. Safety population included all subjects who received at least 1 dose of study intervention.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: seconds                       |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.1 (± 0.16)           | 0.0 (± 0.10)           | -0.1 (± 0.12)          |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in 12-lead ECG-QTcF Interval, Aggregate

|  |  |
|--|--|
| End point title  | Change from Baseline in 12-lead ECG-QTcF Interval, Aggregate |
| End point description:<br>This endpoint reported change from baseline to Month 6 in aggregate QTcF Interval. Safety population included all subjects who received at least 1 dose of study intervention. |  |
| End point type   | Secondary  |
| End point timeframe:<br>Baseline (Week 0), Month 6   |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: milliseconds                  |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 21.0 (± 17.68)         | 4.5 (± 14.88)          | 1.8 (± 13.29)          |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in 12-lead ECG-PR Interval, Aggregate

|  |  |
|--|--|
| End point title  | Change from Baseline in 12-lead ECG-PR Interval, Aggregate |
| End point description:<br>This endpoint reported change from baseline to Month 6 in aggregate PR Interval. Safety population included all subjects who received at least 1 dose of study intervention. |  |
| End point type   | Secondary  |
| End point timeframe:<br>Baseline (Week 0), Month 6   |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: milliseconds                  |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 3.6 (± 8.56)           | 1.8 (± 6.39)           | -1.0 (± 8.75)          |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in 12-lead ECG-QT Interval, Aggregate

|  |  |
|--|--|
| End point title  | Change from Baseline in 12-lead ECG-QT Interval, Aggregate |
| End point description:   |  |
| This endpoint reported change from baseline to Month 6 in aggregate QT Interval. Safety population included all subjects who received at least 1 dose of study intervention. |  |
| End point type   | Secondary  |
| End point timeframe:   |  |
| Baseline (Week 0), Month 6   |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: milliseconds                  |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 28.0 (± 30.60)         | 8.2 (± 20.46)          | -6.7 (± 23.69)         |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in 12-lead ECG-QRS Duration, Aggregate

|   |   |
|---|---|
| End point title   | Change from Baseline in 12-lead ECG-QRS Duration, Aggregate |
| End point description:  |   |
| This endpoint reported change from baseline to Month 6 in aggregate QRS Interval. Safety population included all subjects who received at least 1 dose of study intervention. |   |
| End point type  | Secondary   |
| End point timeframe:  |   |
| Baseline (Week 0), Month 6  |   |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: milliseconds                  |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 3.2 (± 5.45)           | 0.5 (± 6.44)           | 1.7 (± 3.24)           |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in vital sign assessment- Height

|  |   |
|--|---|
| End point title  | Change from Baseline in vital sign assessment- Height |
| End point description:<br>This endpoint reported change from baseline to Month 6 in subject's heights. Safety population included all subjects who received at least 1 dose of study intervention. |   |
| End point type   | Secondary   |
| End point timeframe:<br>Baseline (Week 0), Month 6   |   |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: centimetres                   |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 6.24 (± 2.220)         | 4.01 (± 1.237)         | 2.76 (± 1.548)         |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in vital sign assessment-Weight

|  |  |
|--|--|
| End point title  | Change from Baseline in vital sign assessment-Weight |
| End point description:<br>This endpoint reported change from baseline to Month 6 in subject's weights. Safety population included all subjects who received at least 1 dose of study intervention. |  |
| End point type   | Secondary  |
| End point timeframe:<br>Baseline (Week 0), Month 6   |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: kilograms (kg)                |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 1.130 (± 0.3033)       | 1.488 (± 1.2636)       | 2.411 (± 1.4575)       |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in vital sign assessment-Body Mass Index

|  |   |
|--|---|
| End point title  | Change from Baseline in vital sign assessment-Body Mass Index |
| End point description:<br>This endpoint reported change from baseline to Month 6 in subject's BMI. Safety population included all subjects who received at least 1 dose of study intervention. |   |
| End point type   | Secondary   |
| End point timeframe:<br>Baseline (Week 0), Month 6   |   |

| End point values                                       | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--|------------------------|------------------------|------------------------|--|
| Subject group type                                     | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed                            | 5                      | 13                     | 9                      |  |
| Units: Kilograms per metre square (kg/m <sup>2</sup> ) |                        |                        |                        |  |
| arithmetic mean (standard deviation)                   | -0.668 (± 0.8318)      | 0.152 (± 0.9265)       | 0.608 (± 0.9316)       |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Subjects with Significant Findings- Physical Examination

|   |  |
|---|--|
| End point title   | Change from Baseline in Subjects with Significant Findings- Physical Examination |
| End point description:<br>This endpoint reported change from baseline to Month 6 in number of subjects with significant findings (physical examination). Change from baseline was calculated using formula: value at current time point – baseline value. Safety population included all subjects who received at least 1 dose of study intervention. |  |

|                            |           |
|----------------------------|-----------|
| End point type             | Secondary |
| End point timeframe:       |           |
| Baseline (Week 0), Month 6 |           |

| End point values            | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|-----------------------------|------------------------|------------------------|------------------------|--|
| Subject group type          | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed | 5                      | 13                     | 9                      |  |
| Units: subjects             |                        |                        |                        |  |
| number (not applicable)     | 0                      | 0                      | -1                     |  |

### Statistical analyses

No statistical analyses for this end point

#### Secondary: Change from Baseline in vital sign assessment-Heart Rate

|   |  |
|---|--|
| End point title   | Change from Baseline in vital sign assessment-Heart Rate |
| End point description:  |  |
| This endpoint reported change from baseline to Month 6 in subject's heart rate. Safety population included all subjects who received at least 1 dose of study intervention. |  |
| End point type  | Secondary  |
| End point timeframe:  |  |
| Baseline (Week 0), Month 6  |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: beats/minute                  |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 6.2 (± 14.13)          | -1.5 (± 12.52)         | 4.7 (± 16.96)          |  |

### Statistical analyses

No statistical analyses for this end point

#### Secondary: Change from Baseline in vital sign assessment-Oral Body Temperature

|  |   |
|--|---|
| End point title  | Change from Baseline in vital sign assessment-Oral Body Temperature |
| End point description:   |   |
| This endpoint reported change from baseline to Month 6 in subject's oral body temperature. Safety population included all subjects who received at least 1 dose of study intervention. |   |
| End point type   | Secondary   |



End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: degree celcius (°C)           |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.36 (± 0.555)         | -0.01 (± 0.441)        | -0.00 (± 0.339)        |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in vital sign assessment-Respiratory Rate

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in vital sign assessment-Respiratory Rate |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in subject's respiratory rate. Safety population included all subjects who received at least 1 dose of study intervention.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: breaths/minute                |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -1.4 (± 6.77)          | -1.8 (± 4.17)          | 0.8 (± 2.44)           |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Erythrocytes

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology assessment: Erythrocytes |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in subject's erythrocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                            |           |
|----------------------------|-----------|
| End point type             | Secondary |
| End point timeframe:       |           |
| Baseline (Week 0), Month 6 |           |

| End point values  | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|---|------------------------|------------------------|------------------------|--|
| Subject group type                                      | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed                             | 4                      | 11                     | 8                      |  |
| Units: Tera cells per litre (10 <sup>12</sup> cells /L) |                        |                        |                        |  |
| arithmetic mean (standard deviation)                    | 0.195 (± 0.3375)       | 0.173 (± 0.2765)       | 0.055 (± 0.2076)       |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Haemoglobin

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Haematology assessment: Haemoglobin |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in subject's haemoglobin. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                            |           |
|----------------------------|-----------|
| End point type             | Secondary |
| End point timeframe:       |           |
| Baseline (Week 0), Month 6 |           |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: Grams per decilitre (g/dL)    |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.575 (± 0.7632)       | 0.155 (± 0.8092)       | 0.250 (± 0.7051)       |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in vital sign assessment-Systolic Blood Pressure and Diastolic Blood Pressure

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in vital sign assessment-Systolic Blood Pressure and Diastolic Blood Pressure |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in subject's systolic and diastolic blood pressure. Safety population included all subjects who received at least 1 dose of study intervention.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: Millimetres of Mercury (mmHg) |                        |                        |                        |  |
| arithmetic mean (standard deviation) |                        |                        |                        |  |
| Systolic Blood Pressure              | 6.8 (± 22.20)          | -6.8 (± 17.21)         | 2.8 (± 7.95)           |  |
| Diastolic Blood Pressure             | 7.6 (± 17.84)          | 1.1 (± 13.62)          | 2.8 (± 4.27)           |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Haematocrit

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Haematology assessment: Haematocrit |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in subject's haematocrit. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: Ratio of Haematocrit          |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.01 (± 0.025)         | 0.01 (± 0.019)         | 0.00 (± 0.023)         |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Erythrocytes Mean

## Corpuscular Volume

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology assessment: Erythrocytes Mean Corpuscular Volume |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in subject's erythrocytes mean corpuscular volume. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: Femtolitre (fL)               |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 1.70 (± 3.818)         | -1.25 (± 2.393)        | -0.05 (± 2.530)        |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in Haematology assessment: Reticulocytes

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Haematology assessment: Reticulocytes |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in subject's reticulocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: Percentage of Reticulocytes   |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.15 (± 0.208)         | 0.14 (± 0.781)         | -0.08 (± 0.443)        |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology Assessment: Erythrocytes Mean Corpuscular Haemoglobin Concentration

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology Assessment:<br>Erythrocytes Mean Corpuscular Haemoglobin Concentration |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in subject's erythrocytes mean corpuscular haemoglobin concentration. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: g/dL                          |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.42 (± 1.754)        | -0.09 (± 1.691)        | 0.19 (± 0.783)         |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Erythrocytes Mean Corpuscular Haemoglobin

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology assessment:<br>Erythrocytes Mean Corpuscular Haemoglobin |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in subject's erythrocytes mean corpuscular haemoglobin. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: picograms                     |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.25 ( $\pm$ 0.624)    | -0.48 ( $\pm$ 1.244)   | 0.18 ( $\pm$ 0.738)    |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Platelets

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Haematology assessment: Platelets |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in subject's platelets. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                               | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--|------------------------|------------------------|------------------------|--|
| Subject group type                             | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed                    | 4                      | 11                     | 7                      |  |
| Units: Giga cells per litre ( $10^9$ cells /L) |                        |                        |                        |  |
| arithmetic mean (standard deviation)           | -1.8 ( $\pm$ 40.87)    | -18.6 ( $\pm$ 62.25)   | -27.7 ( $\pm$ 102.53)  |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Leukocytes

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology assessment: Leukocytes |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in subject's leukocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: 10 <sup>9</sup> cells /L      |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -1.460 (± 2.1985)      | 0.383 (± 2.3556)       | -0.625 (± 2.2732)      |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Lymphocytes

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Haematology assessment: Lymphocytes |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in subject's lymphocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: 10 <sup>9</sup> cells /L      |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -1.808 (± 2.3547)      | 0.429 (± 1.1896)       | -0.577 (± 0.9109)      |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Monocytes

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Haematology assessment: Monocytes |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in subject's monocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: 10 <sup>9</sup> cells /L      |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.013 (± 0.2295)      | -0.024 (± 0.1306)      | -0.038 (± 0.1288)      |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Mean Platelet Volume

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology assessment: Mean Platelet Volume |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in subject's mean platelet volume. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 7                      |  |
| Units: fL                            |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.05 (± 0.507)         | -0.10 (± 0.610)        | 0.03 (± 0.553)         |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Eosinophils

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Haematology assessment: Eosinophils |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in subject's eosinophils. Safety population



included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                            |           |
|----------------------------|-----------|
| End point type             | Secondary |
| End point timeframe:       |           |
| Baseline (Week 0), Month 6 |           |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: 10 <sup>9</sup> cells /L      |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.078 (± 0.1441)       | 0.029 (± 0.1495)       | 0.106 (± 0.3064)       |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Neutrophils

|   |   |
|---|---|
| End point title   | Change from Baseline in Haematology assessment: Neutrophils |
| End point description:  |   |
| This endpoint reported change from baseline to Month 6 in subject's neutrophils. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms. |   |
| End point type  | Secondary   |
| End point timeframe:  |   |
| Baseline (Week 0), Month 6  |   |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: 10 <sup>9</sup> cells /L      |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.165 (± 2.1021)      | -0.073 (± 1.4642)      | -0.106 (± 1.3720)      |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Basophils

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Haematology assessment: Basophils |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in subject's basophils. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: 10 <sup>9</sup> cells /L      |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.035 (± 0.0545)       | -0.008 (± 0.0606)      | -0.014 (± 0.0320)      |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Monocytes/Leukocytes

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology assessment: Monocytes/Leukocytes |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in the ratio of monocytes/leukocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                       | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--|------------------------|------------------------|------------------------|--|
| Subject group type                     | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed            | 4                      | 11                     | 8                      |  |
| Units: Ratio of Lymphocytes/Leukocytes |                        |                        |                        |  |
| arithmetic mean (standard deviation)   | 0.70 (± 2.534)         | -0.45 (± 1.109)        | -0.14 (± 1.255)        |  |

### Statistical analyses

No statistical analyses for this end point

**Secondary: Change from Baseline in Haematology assessment: Eosinophils/Leukocytes**

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology assessment: Eosinophils/Leukocytes |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in the ratio of eosinophils/leukocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                       | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--|------------------------|------------------------|------------------------|--|
| Subject group type                     | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed            | 4                      | 11                     | 8                      |  |
| Units: Ratio of Eosinophils/Leukocytes |                        |                        |                        |  |
| arithmetic mean (standard deviation)   | 1.33 ( $\pm$ 1.531)    | 0.22 ( $\pm$ 1.829)    | 1.29 ( $\pm$ 3.061)    |  |

**Statistical analyses**

No statistical analyses for this end point

**Secondary: Change from Baseline in Haematology assessment: Lymphocytes/Leukocytes**

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Haematology assessment: Lymphocytes/Leukocytes |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in the ratio of lymphocytes/leukocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                       | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--|------------------------|------------------------|------------------------|--|
| Subject group type                     | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed            | 4                      | 11                     | 8                      |  |
| Units: Ratio of Lymphocytes/Leukocytes |                        |                        |                        |  |
| arithmetic mean (standard deviation)   | -10.25 ( $\pm$ 16.801) | 2.20 ( $\pm$ 9.901)    | -2.51 ( $\pm$ 13.862)  |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Neutrophils/Leukocytes

|   |  |
|---|--|
| End point title   | Change from Baseline in Haematology assessment: Neutrophils/Leukocytes |
| End point description:<br>This endpoint reported change from baseline to Month 6 in the ratio of neutrophils/leukocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms. |  |
| End point type  | Secondary  |
| End point timeframe:<br>Baseline (Week 0), Month 6  |  |

| End point values                       | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--|------------------------|------------------------|------------------------|--|
| Subject group type                     | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed            | 4                      | 11                     | 8                      |  |
| Units: Ratio of Neutrophils/Leukocytes |                        |                        |                        |  |
| arithmetic mean (standard deviation)   | 7.63 ( $\pm$ 14.717)   | -2.31 ( $\pm$ 9.655)   | 1.50 ( $\pm$ 12.398)   |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Haematology assessment: Basophils/Leukocytes

|   |  |
|---|--|
| End point title   | Change from Baseline in Haematology assessment: Basophils/Leukocytes |
| End point description:<br>This endpoint reported change from baseline to Month 6 in the ratio of basophils/leukocytes. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms. |  |
| End point type  | Secondary  |
| End point timeframe:<br>Baseline (Week 0), Month 6  |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 11                     | 8                      |  |
| Units: Ratio of Basophils/Leukocytes |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.55 ( $\pm$ 0.777)    | -0.01 ( $\pm$ 0.517)   | -0.09 ( $\pm$ 0.606)   |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in Clinical Chemistry Parameter: Alanine Aminotransferase

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Alanine Aminotransferase |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in alanine aminotransferase. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: Units per litre (U/L)         |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -1.8 ( $\pm$ 4.44)     | 14.9 ( $\pm$ 24.68)    | 5.1 ( $\pm$ 4.61)      |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in Clinical Chemistry Parameter: Aspartate Aminotransferase

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Aspartate Aminotransferase |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in aspartate aminotransferase. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the

respective arms.

|                            |           |
|----------------------------|-----------|
| End point type             | Secondary |
| End point timeframe:       |           |
| Baseline (Week 0), Month 6 |           |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: U/L                           |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -4.0 (± 11.94)         | 6.4 (± 13.83)          | 7.1 (± 6.94)           |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Lactate Dehydrogenase

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Lactate Dehydrogenase |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in lactate dehydrogenase. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                            |           |
|----------------------------|-----------|
| End point type             | Secondary |
| End point timeframe:       |           |
| Baseline (Week 0), Month 6 |           |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 4                      | 12                     | 7                      |  |
| Units: U/L                           |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -29.3 (± 17.29)        | 19.5 (± 67.20)         | 1.6 (± 23.44)          |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Glutamate Dehydrogenase

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Clinical Chemistry Parameter: |
|-----------------|---|

## End point description:

This endpoint reported change from baseline to Month 6 in glutamate dehydrogenase. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

## End point timeframe:

|                            |
|----------------------------|
| Baseline (Week 0), Month 6 |
|----------------------------|

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 12                     | 8                      |  |
| Units: U/L                           |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.00 (± 0.000)         | 0.28 (± 0.601)         | 0.06 (± 0.316)         |  |

## Statistical analyses

No statistical analyses for this end point

**Secondary: Change from Baseline in Clinical Chemistry Parameter: Gamma Glutamyl Transferase**

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Gamma Glutamyl Transferase |
|-----------------|--|

## End point description:

This endpoint reported change from baseline to Month 6 in gamma glutamyl transferase. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

## End point timeframe:

|                            |
|----------------------------|
| Baseline (Week 0), Month 6 |
|----------------------------|

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: U/L                           |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.4 (± 4.45)          | 1.4 (± 1.89)           | 1.6 (± 0.92)           |  |

## Statistical analyses

No statistical analyses for this end point

**Secondary: Change from Baseline in Clinical Chemistry Parameter: Bilirubin and Direct Bilirubin**

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Bilirubin and Direct Bilirubin |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in bilirubin and direct bilirubin. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subject analysed' signified 'Overall number of subjects analysed' and 'n' signified 'Number Analyzed' that is number of subjects with available data for particular timepoint, for the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: micromole per litre (umol/L)  |                        |                        |                        |  |
| arithmetic mean (standard deviation) |                        |                        |                        |  |
| Bilirubin (n=5,13,8)                 | -0.46 (± 1.389)        | 1.62 (± 3.467)         | 1.23 (± 2.355)         |  |
| Direct Bilirubin (n=4,12,7)          | -0.07 (± 0.096)        | 0.20 (± 1.224)         | -0.03 (± 0.588)        |  |

**Statistical analyses**

No statistical analyses for this end point

**Secondary: Change from Baseline in Clinical Chemistry Parameter: Alkaline Phosphatase**

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Alkaline Phosphatase |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in alkaline phosphatase. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: U/L                           |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -6.6 (± 19.65)         | 14.6 (± 37.61)         | -8.4 (± 14.88)         |  |



## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Protein

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Protein |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in protein. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: Grams per litre (g/L)         |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.8 (± 6.76)          | 0.6 (± 3.71)           | -2.0 (± 5.55)          |  |

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Sodium

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Sodium |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in sodium. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: millimole per litre (mmol/L)  |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.4 (± 2.51)          | -0.6 (± 2.79)          | -0.2 (± 2.91)          |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in Clinical Chemistry Parameter: Creatine Kinase

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Creatine Kinase |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in creatine kinase. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: U/L                           |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -45.6 (± 58.24)        | 9.5 (± 37.12)          | 5.0 (± 20.34)          |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in Clinical Chemistry Parameter: Albumin

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Albumin |
|-----------------|---|

End point description:

This endpoint reported change from baseline to Month 6 in albumin. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 9                      |  |
| Units: g/L                           |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.2 (± 2.17)          | 1.2 (± 2.79)           | -0.2 (± 2.64)          |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Creatinine

|  |  |
|--|--|
| End point title  | Change from Baseline in Clinical Chemistry Parameter: Creatinine |
| End point description:<br>This endpoint reported change from baseline to Month 6 in creatinine. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms. |  |
| End point type   | Secondary  |
| End point timeframe:<br>Baseline (Week 0), Month 6   |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: umol/L                        |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.66 (± 5.062)         | 1.10 (± 6.327)         | 1.26 (± 1.965)         |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Chloride

|  |  |
|--|--|
| End point title  | Change from Baseline in Clinical Chemistry Parameter: Chloride |
| End point description:<br>This endpoint reported change from baseline to Month 6 in chloride. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms. |  |
| End point type   | Secondary  |
| End point timeframe:<br>Baseline (Week 0), Month 6   |  |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: mmol/L                        |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -1.0 (± 1.22)          | 0.4 (± 2.93)           | -0.9 (± 2.64)          |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Potassium

|   |   |
|---|---|
| End point title   | Change from Baseline in Clinical Chemistry Parameter: Potassium |
| End point description:<br>This endpoint reported change from baseline to Month 6 in potassium. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms. |   |
| End point type  | Secondary   |
| End point timeframe:<br>Baseline (Week 0), Month 6  |   |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: mmol/L                        |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.42 (± 0.356)        | -0.11 (± 0.571)        | -0.15 (± 0.411)        |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Blood Urea Nitrogen

|   |   |
|---|---|
| End point title   | Change from Baseline in Clinical Chemistry Parameter: Blood Urea Nitrogen |
| End point description:<br>This endpoint reported change from baseline to Month 6 in blood urea nitrogen. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms. |   |
| End point type  | Secondary   |

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 13                     | 8                      |  |
| Units: mmol/L                        |                        |                        |                        |  |
| arithmetic mean (standard deviation) | 0.56 ( $\pm$ 2.016)    | 0.06 ( $\pm$ 1.999)    | -0.11 ( $\pm$ 1.082)   |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Cystatin C

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Cystatin C |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in cystatin C. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subjects analysed' signified number of subject with available data for particular timepoint, in the respective arms.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 12                     | 9                      |  |
| Units: milligrams per litre (mg/L)   |                        |                        |                        |  |
| arithmetic mean (standard deviation) | -0.120 ( $\pm$ 0.1595) | -0.026 ( $\pm$ 0.1962) | 0.024 ( $\pm$ 0.1283)  |  |

### Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline in Clinical Chemistry Parameter: Plasma Oxalate

|                 |  |
|-----------------|--|
| End point title | Change from Baseline in Clinical Chemistry Parameter: Plasma Oxalate |
|-----------------|--|

End point description:

This endpoint reported change from baseline to Month 6 in plasma oxalate. Safety population included all subjects who received at least 1 dose of study intervention. Here 'Number of subject analysed'

signifies 'Overall number of subjects analysed'; 'n' signifies 'Number Analyzed' that is number of subjects with available data for particular timepoint, for the respective arms; and "99999" signifies that data for these categories were not evaluable as the number of subject for the respective category was 0.

|                            |           |
|----------------------------|-----------|
| End point type             | Secondary |
| End point timeframe:       |           |
| Baseline (Week 0), Month 6 |           |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 5                      | 8                      | 5                      |  |
| Units: umol/L                        |                        |                        |                        |  |
| arithmetic mean (standard deviation) |                        |                        |                        |  |
| PH1 (n=3,6,5)                        | -9.000 (± 10.5830)     | -5.667 (± 2.6583)      | -4.400 (± 4.3932)      |  |
| PH2 (n=0,2,0)                        | 99999 (± 99999)        | -2.500 (± 0.7071)      | 99999 (± 99999)        |  |
| PH3 (n=2,0,0)                        | -2.000 (± 1.4142)      | 99999 (± 99999)        | 99999 (± 99999)        |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Plasma PK Parameters: Area Under the Concentration-time Curve Calculated to the Last Observable concentration at time t (AUCt)

|                 |  |
|-----------------|--|
| End point title | Plasma PK Parameters: Area Under the Concentration-time Curve Calculated to the Last Observable concentration at time t (AUCt) |
|-----------------|--|

End point description:

This endpoint was expected to report AUCt which is defined as area under the concentration-time curve calculated to the last observable concentration at time t. However, in this study, a non-compartmental PK analysis was not planned or executed due to the limited PK samples collected in paediatric subjects per protocol, hence these PK parameters were not estimated. The PK population included all subjects who had received at least 1 dose of study intervention (without major dosing violations) and had at least 1 evaluable postdose PK assessment.

|   |           |
|---|-----------|
| End point type  | Secondary |
| End point timeframe:  |           |
| Day 1: Postdose 0- to 4-hour and 4- to 24-hour, Days 2, 30, and 90: post dose, Day 150: predose and Day 150: postdose: 0- to 4-hour and 4- to 24-hour |           |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 0 <sup>[3]</sup>       | 0 <sup>[4]</sup>       | 0 <sup>[5]</sup>       |  |
| Units: hour*nanograms per millilitre |                        |                        |                        |  |
| arithmetic mean (standard deviation) | ()                     | ()                     | ()                     |  |

Notes:

[3] - AUCt was not estimable due to limited sample collection.

[4] - AUCt was not estimable due to limited sample collection.

[5] - AUCt was not estimable due to limited sample collection.

## Statistical analyses

No statistical analyses for this end point

## Secondary: Plasma Pharmacokinetic (PK) Parameter: Maximum Observed Concentration (Cmax)

|                 |  |
|-----------------|--|
| End point title | Plasma Pharmacokinetic (PK) Parameter: Maximum Observed Concentration (Cmax) |
|-----------------|--|

End point description:

This endpoint was expected to report Cmax which is defined as maximum observed concentration. However, in this study, a non-compartmental PK analysis was not planned or executed due to the limited PK samples collected in paediatric subjects per protocol, hence these PK parameters were not estimated. The PK population included all subjects who had received at least 1 dose of study intervention (without major dosing violations) and had at least 1 evaluable postdose PK assessment.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Day 1: Postdose 0- to 4-hour and 4- to 24-hour, Days 2, 30, and 90: post dose, Day 150: predose and Day 150: postdose: 0- to 4-hour and 4- to 24-hour

| End point values                        | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|---|------------------------|------------------------|------------------------|--|
| Subject group type                      | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed             | 0 <sup>[6]</sup>       | 0 <sup>[7]</sup>       | 0 <sup>[8]</sup>       |  |
| Units: nanograms per millilitre (ng/mL) |                        |                        |                        |  |
| arithmetic mean (standard deviation)    | ()                     | ()                     | ()                     |  |

Notes:

[6] - Cmax was not estimable due to limited sample collection.

[7] - Cmax was not estimable due to limited sample collection.

[8] - Cmax was not estimable due to limited sample collection.

## Statistical analyses

No statistical analyses for this end point

## Secondary: Plasma PK Parameters: Area Under the Concentration-time Curve From Time Zero to Infinity (AUC<sub>∞</sub>)

|                 |  |
|-----------------|--|
| End point title | Plasma PK Parameters: Area Under the Concentration-time Curve From Time Zero to Infinity (AUC <sub>∞</sub> ) |
|-----------------|--|

End point description:

This endpoint was expected to report AUC<sub>∞</sub> which is defined as area under the concentration-time curve from time zero to infinity. However, in this study, a non-compartmental PK analysis was not planned or executed due to the limited PK samples collected in paediatric subjects per protocol, hence these PK

parameters were not estimated. The PK population included all subjects who had received at least 1 dose of study intervention (without major dosing violations) and had at least 1 evaluable postdose PK assessment.

|   |           |
|---|-----------|
| End point type  | Secondary |
| End point timeframe:  |           |
| Day 1: Postdose 0- to 4-hour and 4- to 24-hour, Days 2, 30, and 90: post dose, Day 150: predose and Day 150: postdose: 0- to 4-hour and 4- to 24-hour |           |

| End point values                     | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--------------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type                   | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed          | 0 <sup>[9]</sup>       | 0 <sup>[10]</sup>      | 0 <sup>[11]</sup>      |  |
| Units: hour*nanograms per millilitre |                        |                        |                        |  |
| arithmetic mean (standard deviation) | ()                     | ()                     | ()                     |  |

Notes:

[9] - AUC<sub>∞</sub> was not estimable due to limited sample collection.

[10] - AUC<sub>∞</sub> was not estimable due to limited sample collection.

[11] - AUC<sub>∞</sub> was not estimable due to limited sample collection.

## Statistical analyses

No statistical analyses for this end point

## Secondary: Percentage of Subjects with Spot Urinary Oxalate-to-Creatinine Ratio ≤Upper Limit of Normal (ULN) or ≤1.5\*ULN at any time point through Month 6 in PH1, PH2, or PH3 Subject Subgroups

|                 |   |
|-----------------|---|
| End point title | Percentage of Subjects with Spot Urinary Oxalate-to-Creatinine Ratio ≤Upper Limit of Normal (ULN) or ≤1.5*ULN at any time point through Month 6 in PH1, PH2, or PH3 Subject Subgroups |
|-----------------|---|

End point description:

This endpoint reported percentage of subjects from baseline to Month 6 Oxalate-to-creatinine Ratio less than and equal to (≤) upper limit of normal (ULN) or ≤1.5\*ULN at any time point through Month 6 in paediatric subjects (birth to 11 years of age) with genetically confirmed PH1, PH2, or PH3 subgroups. The MITT Population included all subjects who received at least 1 dose of study intervention and have at least 1 post-baseline spot urinary oxalate to creatinine ratio. Here 'Number of subject analysed' signifies 'Overall number of subjects analysed'; 'n' signifies 'Number Analyzed' that is number of subjects with available data for particular timepoint, for the respective arms; and "99999" signifies that data for these categories were not evaluable as the number of subject for the respective category was 0.

|  |           |
|--|-----------|
| End point type                         | Secondary |
| End point timeframe:                   |           |
| From baseline (week 0) through Month 6 |           |

| End point values              | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|-------------------------------|------------------------|------------------------|------------------------|--|
| Subject group type            | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed   | 5                      | 13                     | 9                      |  |
| Units: Percentage of subjects |                        |                        |                        |  |
| number (not applicable)       |                        |                        |                        |  |
| PH1: ≤ 1.0 * ULN (n=3,9,8)    | 66.7                   | 44.4                   | 62.5                   |  |
| PH1: ≤ 1.5 * ULN (n=3,9,8)    | 100                    | 88.9                   | 100                    |  |



|   |       |       |       |  |
|---|-------|-------|-------|--|
| PH2: $\leq 1.0 \times \text{ULN}$ (n=0,4,1) | 99999 | 50.0  | 0     |  |
| PH2: $\leq 1.5 \times \text{ULN}$ (n=0,4,1) | 99999 | 75.0  | 100   |  |
| PH3: $\leq 1.0 \times \text{ULN}$ (n=2,0,0) | 50.0  | 99999 | 99999 |  |
| PH3: $\leq 1.5 \times \text{ULN}$ (n=2,0,0) | 100   | 99999 | 99999 |  |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline in eGFR at Month 6 (only in subjects $\geq 12$ Months of age at Screening) in PH1, PH2, or PH3 subject subgroups

|                 |   |
|-----------------|---|
| End point title | Change from Baseline in eGFR at Month 6 (only in subjects $\geq 12$ Months of age at Screening) in PH1, PH2, or PH3 subject subgroups |
|-----------------|---|

End point description:

This endpoint reported Change from Baseline in GFR estimated Cystatin C at Month 6 (only in subjects  $\geq 12$  Months of age at Screening) in PH1, PH2, or PH3 subject subgroups. MITT Population included all subjects who received at least 1 dose of study intervention and have at least 1 post-baseline spot urinary oxalate to creatinine ratio. The eGFR was calculated using multivariate Schwartz equation using formula:  $\text{eGFR} = 39.8 \times [\text{ht}/\text{Scr}]^{0.456} [1.8/\text{cysC}]^{0.418} [30/\text{BUN}]^{0.0791.076^{\text{male}} [\text{ht}/1.4]^{0.179}}$  where ht (height) = metres, Scr (serum creatinine) = milligrams per decilitre (mg/dL), cysC (cystatin C) = mg/L, and BUN (blood urea nitrogen) = mg/dL. Here 'Number of subject analysed' signifies 'Overall number of subjects analysed'; 'n' signifies 'Number Analysed' i.e, number of subjects with available data for particular timepoint, for the respective arms; and "99999" signifies that data for these categories were not evaluable as number of subject for respective category was 0.

|                |           |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (Week 0), Month 6

| End point values                               | Children 0 to <2 years | Children 2 to <6 years | Children 6 to 11 years |  |
|--|------------------------|------------------------|------------------------|--|
| Subject group type                             | Reporting group        | Reporting group        | Reporting group        |  |
| Number of subjects analysed                    | 5                      | 12                     | 9                      |  |
| Units: millilitre/minute/standard surface area |                        |                        |                        |  |
| arithmetic mean (standard deviation)           |                        |                        |                        |  |
| PH1: (n=3,8,8)                                 | 0.0 ( $\pm 17.06$ )    | 0.3 ( $\pm 10.02$ )    | -0.8 ( $\pm 7.21$ )    |  |
| PH2: (n=0,4,1)                                 | 99999 ( $\pm 99999$ )  | 3.5 ( $\pm 13.48$ )    | -7.0 ( $\pm 0$ )       |  |
| PH3: (n=2,0,0)                                 | 15.0 ( $\pm 9.90$ )    | 99999 ( $\pm 99999$ )  | 99999 ( $\pm 99999$ )  |  |

## Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

Baseline (Week 0) to Month 6

Adverse event reporting additional description:

AE: Any untoward medical occurrence in subject, temporally associated with use of study drug, whether or not considered related to study drug. TEAE: Any AE if they have an onset or worsen in severity after a subject receives the study. Safety population: All subjects who received at least 1 dose of study drug.

|                 |            |
|-----------------|------------|
| Assessment type | Systematic |
|-----------------|------------|

### Dictionary used

|                 |        |
|-----------------|--------|
| Dictionary name | MedDRA |
|-----------------|--------|

|                    |    |
|--------------------|----|
| Dictionary version | 28 |
|--------------------|----|

### Reporting groups

|                       |                        |
|-----------------------|------------------------|
| Reporting group title | Children 0 to <2 years |
|-----------------------|------------------------|

Reporting group description:

All subjects that included neonates (0 to <2 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.

|                       |         |
|-----------------------|---------|
| Reporting group title | Overall |
|-----------------------|---------|

Reporting group description: -

|                       |                        |
|-----------------------|------------------------|
| Reporting group title | Children 6 to 11 years |
|-----------------------|------------------------|

Reporting group description:

All subjects that included children (6 to 11 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.

|                       |                        |
|-----------------------|------------------------|
| Reporting group title | Children 2 to <6 years |
|-----------------------|------------------------|

Reporting group description:

All subjects that included infants (2 to <6 years) with PH and relatively intact renal function based upon eGFR and serum creatinine, received monthly dose of nedosiran (3.5 mg/kg, not to exceed 170 mg), subcutaneously from Day 1 though Month 6.

| Serious adverse events                            | Children 0 to <2 years | Overall         | Children 6 to 11 years |
|---|------------------------|-----------------|------------------------|
| Total subjects affected by serious adverse events |                        |                 |                        |
| subjects affected / exposed                       | 1 / 5 (20.00%)         | 5 / 27 (18.52%) | 1 / 9 (11.11%)         |
| number of deaths (all causes)                     | 0                      | 0               | 0                      |
| number of deaths resulting from adverse events    | 0                      | 0               | 0                      |
| Renal and urinary disorders                       |                        |                 |                        |
| Nephrolithiasis                                   |                        |                 |                        |
| subjects affected / exposed                       | 0 / 5 (0.00%)          | 2 / 27 (7.41%)  | 1 / 9 (11.11%)         |
| occurrences causally related to treatment / all   | 0 / 0                  | 0 / 3           | 0 / 1                  |
| deaths causally related to treatment / all        | 0 / 0                  | 0 / 0           | 0 / 0                  |
| Pyelocaliectasis                                  |                        |                 |                        |

|   |                |                |                |
|---|----------------|----------------|----------------|
| subjects affected / exposed                     | 0 / 5 (0.00%)  | 1 / 27 (3.70%) | 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          |
| <b>Infections and infestations</b>              |                |                |                |
| Gastroenteritis                                 |                |                |                |
| subjects affected / exposed                     | 0 / 5 (0.00%)  | 1 / 27 (3.70%) | 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          |
| Pneumonia                                       |                |                |                |
| subjects affected / exposed                     | 0 / 5 (0.00%)  | 1 / 27 (3.70%) | 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          |
| Upper respiratory tract infection               |                |                |                |
| subjects affected / exposed                     | 1 / 5 (20.00%) | 1 / 27 (3.70%) | 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          |

|   |                        |  |  |
|---|------------------------|--|--|
| <b>Serious adverse events</b>                     | Children 2 to <6 years |  |  |
| Total subjects affected by serious adverse events |                        |  |  |
| subjects affected / exposed                       | 3 / 13 (23.08%)        |  |  |
| number of deaths (all causes)                     | 0                      |  |  |
| number of deaths resulting from adverse events    | 0                      |  |  |
| <b>Renal and urinary disorders</b>                |                        |  |  |
| Nephrolithiasis                                   |                        |  |  |
| subjects affected / exposed                       | 1 / 13 (7.69%)         |  |  |
| occurrences causally related to treatment / all   | 0 / 2                  |  |  |
| deaths causally related to treatment / all        | 0 / 0                  |  |  |
| Pyelocaliectasis                                  |                        |  |  |
| subjects affected / exposed                       | 1 / 13 (7.69%)         |  |  |
| occurrences causally related to treatment / all   | 0 / 1                  |  |  |
| deaths causally related to treatment / all        | 0 / 0                  |  |  |
| <b>Infections and infestations</b>                |                        |  |  |
| Gastroenteritis                                   |                        |  |  |

|   |                |  |  |
|---|----------------|--|--|
| subjects affected / exposed                     | 0 / 13 (0.00%) |  |  |
| occurrences causally related to treatment / all | 0 / 0          |  |  |
| deaths causally related to treatment / all      | 0 / 0          |  |  |
| Pneumonia                                       |                |  |  |
| subjects affected / exposed                     | 1 / 13 (7.69%) |  |  |
| occurrences causally related to treatment / all | 0 / 1          |  |  |
| deaths causally related to treatment / all      | 0 / 0          |  |  |
| Upper respiratory tract infection               |                |  |  |
| subjects affected / exposed                     | 0 / 13 (0.00%) |  |  |
| occurrences causally related to treatment / all | 0 / 0          |  |  |
| deaths causally related to treatment / all      | 0 / 0          |  |  |

Frequency threshold for reporting non-serious adverse events: 5 %

| <b>Non-serious adverse events</b>                     | Children 0 to <2 years | Overall          | Children 6 to 11 years |
|---|------------------------|------------------|------------------------|
| Total subjects affected by non-serious adverse events |                        |                  |                        |
| subjects affected / exposed                           | 5 / 5 (100.00%)        | 23 / 27 (85.19%) | 7 / 9 (77.78%)         |
| General disorders and administration site conditions  |                        |                  |                        |
| Injection site pain                                   |                        |                  |                        |
| subjects affected / exposed                           | 1 / 5 (20.00%)         | 5 / 27 (18.52%)  | 2 / 9 (22.22%)         |
| occurrences (all)                                     | 1                      | 6                | 3                      |
| Pyrexia   |                        |                  |                        |
| subjects affected / exposed                           | 4 / 5 (80.00%)         | 6 / 27 (22.22%)  | 2 / 9 (22.22%)         |
| occurrences (all)                                     | 4                      | 9                | 5                      |
| Injection site induration                             |                        |                  |                        |
| subjects affected / exposed                           | 0 / 5 (0.00%)          | 1 / 27 (3.70%)   | 0 / 9 (0.00%)          |
| occurrences (all)                                     | 0                      | 1                | 0                      |
| Reproductive system and breast disorders              |                        |                  |                        |
| Balanoposthitis                                       |                        |                  |                        |
| subjects affected / exposed                           | 1 / 5 (20.00%)         | 1 / 27 (3.70%)   | 0 / 9 (0.00%)          |
| occurrences (all)                                     | 1                      | 1                | 0                      |
| Respiratory, thoracic and mediastinal disorders       |                        |                  |                        |
| Cough   |                        |                  |                        |

|  |                |                 |                |
|--|----------------|-----------------|----------------|
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 4 / 27 (14.81%) | 0 / 9 (0.00%)  |
| occurrences (all)                              | 0              | 5               | 0              |
| Epistaxis                                      |                |                 |                |
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 0              | 1               | 0              |
| Rhinorrhoea                                    |                |                 |                |
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 0              | 1               | 0              |
| Tonsillar hypertrophy                          |                |                 |                |
| subjects affected / exposed                    | 1 / 5 (20.00%) | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 1              | 1               | 0              |
| Psychiatric disorders                          |                |                 |                |
| Enuresis                                       |                |                 |                |
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 0              | 1               | 0              |
| Irritability                                   |                |                 |                |
| subjects affected / exposed                    | 1 / 5 (20.00%) | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 1              | 1               | 0              |
| Tic  |                |                 |                |
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 0              | 1               | 0              |
| Investigations                                 |                |                 |                |
| Cystatin C increased                           |                |                 |                |
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 1 / 9 (11.11%) |
| occurrences (all)                              | 0              | 2               | 2              |
| Alanine aminotransferase increased             |                |                 |                |
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 0              | 1               | 0              |
| Hepatic enzyme increased                       |                |                 |                |
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 0              | 1               | 0              |
| Urinary sediment                               |                |                 |                |
| subjects affected / exposed                    | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                              | 0              | 1               | 0              |
| Injury, poisoning and procedural complications |                |                 |                |

|  |                     |                      |                     |
|--|---------------------|----------------------|---------------------|
| Procedural pain<br>subjects affected / exposed<br>occurrences (all)                                    | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Immunisation reaction<br>subjects affected / exposed<br>occurrences (all)                              | 1 / 5 (20.00%)<br>1 | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Skin laceration<br>subjects affected / exposed<br>occurrences (all)                                    | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Nervous system disorders<br>Dizziness<br>subjects affected / exposed<br>occurrences (all)              | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Headache<br>subjects affected / exposed<br>occurrences (all)   | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 1 / 9 (11.11%)<br>1 |
| Blood and lymphatic system disorders<br>Anaemia<br>subjects affected / exposed<br>occurrences (all)    | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 1 / 9 (11.11%)<br>1 |
| Thrombocytopenia<br>subjects affected / exposed<br>occurrences (all)                                   | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Ear and labyrinth disorders<br>Ear pain<br>subjects affected / exposed<br>occurrences (all)            | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Gastrointestinal disorders<br>Abdominal pain upper<br>subjects affected / exposed<br>occurrences (all) | 1 / 5 (20.00%)<br>5 | 2 / 27 (7.41%)<br>6  | 0 / 9 (0.00%)<br>0  |
| Diarrhoea<br>subjects affected / exposed<br>occurrences (all)  | 1 / 5 (20.00%)<br>1 | 4 / 27 (14.81%)<br>5 | 1 / 9 (11.11%)<br>1 |
| Constipation<br>subjects affected / exposed<br>occurrences (all)                                       | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |

|  |                     |                      |                     |
|--|---------------------|----------------------|---------------------|
| Vomiting<br>subjects affected / exposed<br>occurrences (all)         | 2 / 5 (40.00%)<br>2 | 2 / 27 (7.41%)<br>2  | 0 / 9 (0.00%)<br>0  |
| Odynophagia<br>subjects affected / exposed<br>occurrences (all)      | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Nausea<br>subjects affected / exposed<br>occurrences (all)           | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 1 / 9 (11.11%)<br>1 |
| Skin and subcutaneous tissue disorders                               |                     |                      |                     |
| Rash vesicular<br>subjects affected / exposed<br>occurrences (all)   | 1 / 5 (20.00%)<br>1 | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Rash<br>subjects affected / exposed<br>occurrences (all)             | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>2  | 0 / 9 (0.00%)<br>0  |
| Macule<br>subjects affected / exposed<br>occurrences (all)           | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Renal and urinary disorders  |                     |                      |                     |
| Calculus urinary<br>subjects affected / exposed<br>occurrences (all) | 0 / 5 (0.00%)<br>0  | 3 / 27 (11.11%)<br>6 | 2 / 9 (22.22%)<br>4 |
| Haematuria<br>subjects affected / exposed<br>occurrences (all)       | 0 / 5 (0.00%)<br>0  | 2 / 27 (7.41%)<br>2  | 1 / 9 (11.11%)<br>1 |
| Dysuria<br>subjects affected / exposed<br>occurrences (all)          | 0 / 5 (0.00%)<br>0  | 2 / 27 (7.41%)<br>2  | 2 / 9 (22.22%)<br>2 |
| Renal colic<br>subjects affected / exposed<br>occurrences (all)      | 1 / 5 (20.00%)<br>1 | 3 / 27 (11.11%)<br>3 | 2 / 9 (22.22%)<br>2 |
| Nephrolithiasis<br>subjects affected / exposed<br>occurrences (all)  | 2 / 5 (40.00%)<br>6 | 3 / 27 (11.11%)<br>7 | 1 / 9 (11.11%)<br>1 |
| Glycosuria   |                     |                      |                     |

|  |                     |                      |                     |
|--|---------------------|----------------------|---------------------|
| subjects affected / exposed<br>occurrences (all)   | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Renal disorder<br>subjects affected / exposed<br>occurrences (all)   | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Urinary incontinence<br>subjects affected / exposed<br>occurrences (all)   | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| Musculoskeletal and connective tissue disorders<br>Pain in extremity<br>subjects affected / exposed<br>occurrences (all) | 0 / 5 (0.00%)<br>0  | 2 / 27 (7.41%)<br>2  | 0 / 9 (0.00%)<br>0  |
| Flank pain<br>subjects affected / exposed<br>occurrences (all)   | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 1 / 9 (11.11%)<br>1 |
| Infections and infestations<br>Conjunctivitis<br>subjects affected / exposed<br>occurrences (all)                        | 0 / 5 (0.00%)<br>0  | 2 / 27 (7.41%)<br>2  | 0 / 9 (0.00%)<br>0  |
| Influenza<br>subjects affected / exposed<br>occurrences (all)  | 2 / 5 (40.00%)<br>2 | 3 / 27 (11.11%)<br>3 | 0 / 9 (0.00%)<br>0  |
| Respiratory tract infection<br>subjects affected / exposed<br>occurrences (all)  | 0 / 5 (0.00%)<br>0  | 2 / 27 (7.41%)<br>2  | 0 / 9 (0.00%)<br>0  |
| Nasopharyngitis<br>subjects affected / exposed<br>occurrences (all)  | 2 / 5 (40.00%)<br>4 | 4 / 27 (14.81%)<br>8 | 0 / 9 (0.00%)<br>0  |
| Upper respiratory tract infection<br>subjects affected / exposed<br>occurrences (all)                                    | 0 / 5 (0.00%)<br>0  | 3 / 27 (11.11%)<br>8 | 0 / 9 (0.00%)<br>0  |
| Croup infectious<br>subjects affected / exposed<br>occurrences (all)   | 0 / 5 (0.00%)<br>0  | 1 / 27 (3.70%)<br>1  | 0 / 9 (0.00%)<br>0  |
| COVID-19   |                     |                      |                     |



|                                     |                |                 |                |
|-------------------------------------|----------------|-----------------|----------------|
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                   | 0              | 1               | 0              |
| Urinary tract infection             |                |                 |                |
| subjects affected / exposed         | 1 / 5 (20.00%) | 3 / 27 (11.11%) | 1 / 9 (11.11%) |
| occurrences (all)                   | 1              | 4               | 2              |
| Escherichia urinary tract infection |                |                 |                |
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 1 / 9 (11.11%) |
| occurrences (all)                   | 0              | 4               | 4              |
| Gastroenteritis                     |                |                 |                |
| subjects affected / exposed         | 1 / 5 (20.00%) | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                   | 2              | 2               | 0              |
| Scarlet fever                       |                |                 |                |
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 1 / 9 (11.11%) |
| occurrences (all)                   | 0              | 1               | 1              |
| Otitis media acute                  |                |                 |                |
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                   | 0              | 1               | 0              |
| Hepatitis A                         |                |                 |                |
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 1 / 9 (11.11%) |
| occurrences (all)                   | 0              | 1               | 1              |
| Impetigo                            |                |                 |                |
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                   | 0              | 1               | 0              |
| Urinary tract infection pseudomonal |                |                 |                |
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 1 / 9 (11.11%) |
| occurrences (all)                   | 0              | 1               | 1              |
| Tonsillitis                         |                |                 |                |
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 1 / 9 (11.11%) |
| occurrences (all)                   | 0              | 2               | 2              |
| Metabolism and nutrition disorders  |                |                 |                |
| Iron deficiency                     |                |                 |                |
| subjects affected / exposed         | 1 / 5 (20.00%) | 2 / 27 (7.41%)  | 0 / 9 (0.00%)  |
| occurrences (all)                   | 1              | 3               | 0              |
| Obesity                             |                |                 |                |
| subjects affected / exposed         | 0 / 5 (0.00%)  | 1 / 27 (3.70%)  | 0 / 9 (0.00%)  |
| occurrences (all)                   | 0              | 1               | 0              |

|  |                     |                     |                    |
|--|---------------------|---------------------|--------------------|
| Vitamin D deficiency<br>subjects affected / exposed<br>occurrences (all) | 1 / 5 (20.00%)<br>1 | 1 / 27 (3.70%)<br>1 | 0 / 9 (0.00%)<br>0 |
|--|---------------------|---------------------|--------------------|

|   |   |  |  |
|---|---|--|--|
| <b>Non-serious adverse events</b>   | Children 2 to <6 years  |  |  |
| Total subjects affected by non-serious adverse events<br>subjects affected / exposed  | 11 / 13 (84.62%)  |  |  |
| General disorders and administration site conditions<br>Injection site pain<br>subjects affected / exposed<br>occurrences (all)<br><br>Pyrexia<br>subjects affected / exposed<br>occurrences (all)<br><br>Injection site induration<br>subjects affected / exposed<br>occurrences (all)   | 2 / 13 (15.38%)<br>2<br><br>0 / 13 (0.00%)<br>0<br><br>1 / 13 (7.69%)<br>1                            |  |  |
| Reproductive system and breast disorders<br>Balanoposthitis<br>subjects affected / exposed<br>occurrences (all)   | 0 / 13 (0.00%)<br>0   |  |  |
| Respiratory, thoracic and mediastinal disorders<br>Cough<br>subjects affected / exposed<br>occurrences (all)<br><br>Epistaxis<br>subjects affected / exposed<br>occurrences (all)<br><br>Rhinorrhoea<br>subjects affected / exposed<br>occurrences (all)<br><br>Tonsillar hypertrophy<br>subjects affected / exposed<br>occurrences (all) | 4 / 13 (30.77%)<br>5<br><br>1 / 13 (7.69%)<br>1<br><br>1 / 13 (7.69%)<br>1<br><br>0 / 13 (0.00%)<br>0 |  |  |
| Psychiatric disorders   |   |  |  |

|   |                     |  |  |
|---|---------------------|--|--|
| Enuresis<br>subjects affected / exposed<br>occurrences (all)  | 1 / 13 (7.69%)<br>1 |  |  |
| Irritability<br>subjects affected / exposed<br>occurrences (all)  | 0 / 13 (0.00%)<br>0 |  |  |
| Tic<br>subjects affected / exposed<br>occurrences (all)   | 1 / 13 (7.69%)<br>1 |  |  |
| Investigations<br>Cystatin C increased<br>subjects affected / exposed<br>occurrences (all)                            | 0 / 13 (0.00%)<br>0 |  |  |
| Alanine aminotransferase increased<br>subjects affected / exposed<br>occurrences (all)                                | 1 / 13 (7.69%)<br>1 |  |  |
| Hepatic enzyme increased<br>subjects affected / exposed<br>occurrences (all)  | 1 / 13 (7.69%)<br>1 |  |  |
| Urinary sediment<br>subjects affected / exposed<br>occurrences (all)  | 1 / 13 (7.69%)<br>1 |  |  |
| Injury, poisoning and procedural complications<br>Procedural pain<br>subjects affected / exposed<br>occurrences (all) | 1 / 13 (7.69%)<br>1 |  |  |
| Immunisation reaction<br>subjects affected / exposed<br>occurrences (all)   | 0 / 13 (0.00%)<br>0 |  |  |
| Skin laceration<br>subjects affected / exposed<br>occurrences (all)   | 1 / 13 (7.69%)<br>1 |  |  |
| Nervous system disorders<br>Dizziness<br>subjects affected / exposed<br>occurrences (all)                             | 1 / 13 (7.69%)<br>1 |  |  |

|  |   |  |  |
|--|---|--|--|
| Headache<br>subjects affected / exposed<br>occurrences (all)   | 0 / 13 (0.00%)<br>0   |  |  |
| Blood and lymphatic system disorders<br>Anaemia<br>subjects affected / exposed<br>occurrences (all)<br><br>Thrombocytopenia<br>subjects affected / exposed<br>occurrences (all)  | 0 / 13 (0.00%)<br>0<br><br>1 / 13 (7.69%)<br>1  |  |  |
| Ear and labyrinth disorders<br>Ear pain<br>subjects affected / exposed<br>occurrences (all)  | 1 / 13 (7.69%)<br>1   |  |  |
| Gastrointestinal disorders<br>Abdominal pain upper<br>subjects affected / exposed<br>occurrences (all)<br><br>Diarrhoea<br>subjects affected / exposed<br>occurrences (all)<br><br>Constipation<br>subjects affected / exposed<br>occurrences (all)<br><br>Vomiting<br>subjects affected / exposed<br>occurrences (all)<br><br>Odynophagia<br>subjects affected / exposed<br>occurrences (all)<br><br>Nausea<br>subjects affected / exposed<br>occurrences (all) | 1 / 13 (7.69%)<br>1<br><br>2 / 13 (15.38%)<br>3<br><br>1 / 13 (7.69%)<br>1<br><br>0 / 13 (0.00%)<br>0<br><br>1 / 13 (7.69%)<br>1<br><br>0 / 13 (0.00%)<br>0 |  |  |
| Skin and subcutaneous tissue disorders<br>Rash vesicular<br>subjects affected / exposed<br>occurrences (all)   | 0 / 13 (0.00%)<br>0   |  |  |

|   |                 |  |  |
|---|-----------------|--|--|
| Rash  |                 |  |  |
| subjects affected / exposed                     | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                               | 2               |  |  |
| Macule  |                 |  |  |
| subjects affected / exposed                     | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                               | 1               |  |  |
| Renal and urinary disorders                     |                 |  |  |
| Calculus urinary                                |                 |  |  |
| subjects affected / exposed                     | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                               | 2               |  |  |
| Haematuria                                      |                 |  |  |
| subjects affected / exposed                     | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                               | 1               |  |  |
| Dysuria   |                 |  |  |
| subjects affected / exposed                     | 0 / 13 (0.00%)  |  |  |
| occurrences (all)                               | 0               |  |  |
| Renal colic                                     |                 |  |  |
| subjects affected / exposed                     | 0 / 13 (0.00%)  |  |  |
| occurrences (all)                               | 0               |  |  |
| Nephrolithiasis                                 |                 |  |  |
| subjects affected / exposed                     | 0 / 13 (0.00%)  |  |  |
| occurrences (all)                               | 0               |  |  |
| Glycosuria                                      |                 |  |  |
| subjects affected / exposed                     | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                               | 1               |  |  |
| Renal disorder                                  |                 |  |  |
| subjects affected / exposed                     | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                               | 1               |  |  |
| Urinary incontinence                            |                 |  |  |
| subjects affected / exposed                     | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                               | 1               |  |  |
| Musculoskeletal and connective tissue disorders |                 |  |  |
| Pain in extremity                               |                 |  |  |
| subjects affected / exposed                     | 2 / 13 (15.38%) |  |  |
| occurrences (all)                               | 2               |  |  |
| Flank pain                                      |                 |  |  |

|                                     |                 |  |  |
|-------------------------------------|-----------------|--|--|
| subjects affected / exposed         | 0 / 13 (0.00%)  |  |  |
| occurrences (all)                   | 0               |  |  |
| Infections and infestations         |                 |  |  |
| Conjunctivitis                      |                 |  |  |
| subjects affected / exposed         | 2 / 13 (15.38%) |  |  |
| occurrences (all)                   | 2               |  |  |
| Influenza                           |                 |  |  |
| subjects affected / exposed         | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                   | 1               |  |  |
| Respiratory tract infection         |                 |  |  |
| subjects affected / exposed         | 2 / 13 (15.38%) |  |  |
| occurrences (all)                   | 2               |  |  |
| Nasopharyngitis                     |                 |  |  |
| subjects affected / exposed         | 2 / 13 (15.38%) |  |  |
| occurrences (all)                   | 4               |  |  |
| Upper respiratory tract infection   |                 |  |  |
| subjects affected / exposed         | 3 / 13 (23.08%) |  |  |
| occurrences (all)                   | 8               |  |  |
| Croup infectious                    |                 |  |  |
| subjects affected / exposed         | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                   | 1               |  |  |
| COVID-19                            |                 |  |  |
| subjects affected / exposed         | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                   | 1               |  |  |
| Urinary tract infection             |                 |  |  |
| subjects affected / exposed         | 1 / 13 (7.69%)  |  |  |
| occurrences (all)                   | 1               |  |  |
| Escherichia urinary tract infection |                 |  |  |
| subjects affected / exposed         | 0 / 13 (0.00%)  |  |  |
| occurrences (all)                   | 0               |  |  |
| Gastroenteritis                     |                 |  |  |
| subjects affected / exposed         | 0 / 13 (0.00%)  |  |  |
| occurrences (all)                   | 0               |  |  |
| Scarlet fever                       |                 |  |  |
| subjects affected / exposed         | 0 / 13 (0.00%)  |  |  |
| occurrences (all)                   | 0               |  |  |

|   |                     |  |  |
|---|---------------------|--|--|
| Otitis media acute<br>subjects affected / exposed<br>occurrences (all)                  | 1 / 13 (7.69%)<br>1 |  |  |
| Hepatitis A<br>subjects affected / exposed<br>occurrences (all)                         | 0 / 13 (0.00%)<br>0 |  |  |
| Impetigo<br>subjects affected / exposed<br>occurrences (all)                            | 1 / 13 (7.69%)<br>1 |  |  |
| Urinary tract infection pseudomonal<br>subjects affected / exposed<br>occurrences (all) | 0 / 13 (0.00%)<br>0 |  |  |
| Tonsillitis<br>subjects affected / exposed<br>occurrences (all)                         | 0 / 13 (0.00%)<br>0 |  |  |
| Metabolism and nutrition disorders  |                     |  |  |
| Iron deficiency<br>subjects affected / exposed<br>occurrences (all)                     | 1 / 13 (7.69%)<br>2 |  |  |
| Obesity<br>subjects affected / exposed<br>occurrences (all)                             | 1 / 13 (7.69%)<br>1 |  |  |
| Vitamin D deficiency<br>subjects affected / exposed<br>occurrences (all)                | 0 / 13 (0.00%)<br>0 |  |  |

## More information

### Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

| Date             | Amendment  |
|------------------|--|
| 21 December 2021 | <p>Amendment 1, version 2.0:<br/>The 15-Apr-2021 (Original Protocol, version 1.0) version of the protocol was amended to expand the population to include 6- to 11-year-old subjects. Other principal changes included changing the primary objective from safety to efficacy, adding pregnancy and contraception requirements, adding a maximum dose of 170 mg, updating the number of planned subjects from 15 to 25, and clarifying that the efficacy endpoints were presented by PH1, PH2, or PH3 subgroups.</p> <p>1) Expanded population to include 6- to 11-year-old subjects. 2) Updated the primary endpoint to efficacy based upon spot urinary oxalate-to-creatinine ratio; safety endpoint was moved to secondary. 3) Added a maximum dose of 170 mg. 4) Clarified for subjects <math>\geq 6</math> months of age at Screening, the dose was to remain constant throughout the study (i.e., the dose administered on Day 1 will be the dose administered at all following visits regardless of any change in body weight). 5) Updated number of planned subjects from 15 to 25. 6) Clarified that the secondary efficacy endpoint related to normalisation of spot urinary oxalate-to-creatinine ratio includes subjects with spot urinary oxalate-to-creatinine ratio <math>\leq</math> the ULN or <math>\leq 1.5 \times</math> ULN at any time point. 7) Clarified that the efficacy endpoints will be presented by PH1, PH2, or PH3 subgroups. 8) Other study related and administrative changes.</p> |
| 01 November 2022 | <p>Amendment 2, version 3.0:<br/>The 21-Dec-2021 version of the protocol was amended to expand the population to include <math>&lt;2</math>-year-old subjects.</p> <p>1) Expanded population to include 0- to 2-year-old subjects. 2) Corrected errors in the Schedule of assessment relating to recording of fluid intake. 3) Other administrative updates.</p>   |
| 23 February 2023 | <p>Amendment 3, version 4.0:<br/>1) Removed the 10 kg minimum body weight from the inclusion criteria. 2) Added text regarding maximum blood volume collection allowed based on body weight and to direct the Investigator to the Laboratory Manual for the blood draw priority list. 3) Updated study duration from 18 to 24 months. 4) Added text to allow for interval weight gain dose adjustments in concomitant therapy regimens. 5) Updated text to clarify the range of 4 to 7 days for reporting of fluid intake. 6) Added additional text denoting differences in response between participants with PH1 and PH2 in study. 7) Added text regarding spot urine collection in infants. 8) Added text to allow for interval weight gain dose adjustments in concomitant therapy regimens. 9) Other administrative updates</p>   |

Notes:

### Interruptions (globally)

Were there any global interruptions to the trial? No

### Limitations and caveats

None reported