



Clinical trial results:

An Open-Label, Inpatient Dose-Escalation Study to Evaluate the Safety, Tolerability, Immunogenicity, and Biological Activity of ATYR1940 in Patients With Early Onset and Other Pediatric Onset Facioscapulohumeral Muscular Dystrophy

Summary

| | |
|--------------------------|------------------|
| EudraCT number | 2014-003346-27 |
| Trial protocol | IT |
| Global end of trial date | 14 February 2017 |

Results information

| | |
|--------------------------------|--------------|
| Result version number | v1 (current) |
| This version publication date | 20 May 2018 |
| First version publication date | 20 May 2018 |

Trial information

Trial identification

| | |
|-----------------------|----------------|
| Sponsor protocol code | ATYR1940-C-003 |
|-----------------------|----------------|

Additional study identifiers

| | |
|------------------------------------|--------------------|
| ISRCTN number | - |
| ClinicalTrials.gov id (NCT number) | - |
| WHO universal trial number (UTN) | - |
| Other trial identifiers | IND number: 122045 |

Notes:

Sponsors

| | |
|------------------------------|--|
| Sponsor organisation name | aTyr Pharma, Inc. |
| Sponsor organisation address | 3545 John Hopkins Court, Suite #250, San Diego, CA, United States, 92121 |
| Public contact | Clinical Trial Operations, Voisin Consulting, clinicaltrialinformation@voisinconsulting.com |
| Scientific contact | Clinical Trial Operations, Voisin Consulting, clinicaltrialinformation@voisinconsulting.com |

Notes:

Paediatric regulatory details

| | |
|--|----|
| Is trial part of an agreed paediatric investigation plan (PIP) | No |
| 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? | No |

Notes:

Results analysis stage

| | |
|--|------------------|
| Analysis stage | Final |
| Date of interim/final analysis | 13 July 2017 |
| Is this the analysis of the primary completion data? | Yes |
| Primary completion date | 14 February 2017 |
| Global end of trial reached? | Yes |
| Global end of trial date | 14 February 2017 |
| Was the trial ended prematurely? | No |

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety, tolerability, and immunogenicity of the intravenous (IV) administration of ATYR1940, at doses of 0.3, 1.0, and 3.0 mg/kg, to patients with early onset FSHD

Protection of trial subjects:

The study process, benefits and risks of participating in the study were explained to each subject. In addition, if the study drug needed to be stopped for safety, the doctor, his/her staff along with the medical monitor, were to continue to monitor participant's health and determine what treatment should be given (if any) until the symptoms or findings had resolved or until a satisfactory conclusion was reached.

Background therapy: -

Evidence for comparator: -

| | |
|---|-------------------|
| Actual start date of recruitment | 01 September 2015 |
| 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 | France: 1 |
| Country: Number of subjects enrolled | Italy: 2 |
| Country: Number of subjects enrolled | United States: 5 |
| Worldwide total number of subjects | 8 |
| EEA total number of subjects | 3 |

Notes:

Subjects enrolled per age group

| | |
|---|---|
| In utero | 0 |
| Preterm newborn - gestational age < 37 wk | 0 |
| Newborns (0-27 days) | 0 |
| Infants and toddlers (28 days-23 months) | 0 |
| Children (2-11 years) | 0 |
| Adolescents (12-17 years) | 3 |
| Adults (18-64 years) | 5 |

| | |
|---------------------|---|
| From 65 to 84 years | 0 |
| 85 years and over | 0 |

Subject disposition

Recruitment

Recruitment details:

Per the original study design enrollment was to be performed in 2 stages. Stage 1 was conducted and completed and the data are described herein. The sponsor elected not to conduct Stage 2.

The key patient disease characteristics included an established genetically confirmed diagnosis of FSHD with FSHD signs or symptoms presenting < 10 years.

Pre-assignment

Screening details:

The study actually had 3 periods distinct periods - screening, treatment (13 weeks including 1 dose of placebo at Week 1) and follow-up (12 weeks).

Pre-assignment period milestones

| | |
|------------------------------|------------------|
| Number of subjects started | 9 ^[1] |
| Number of subjects completed | 8 |

Pre-assignment subject non-completion reasons

| | |
|----------------------------|-------------------|
| Reason: Number of subjects | Screen failure: 1 |
|----------------------------|-------------------|

Notes:

[1] - The number of subjects reported to have started the pre-assignment period are not the same as the worldwide number enrolled in the trial. It is expected that these numbers will be the same.

Justification: The worldwide number corresponds to the number of patients randomized (8) and not to the number of patients screened (9).

Period 1

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

Arms

| | |
|-----------|----------|
| Arm title | ATYR1940 |
|-----------|----------|

Arm description:

Enrollment into the study was to be conducted in 2 stages, based on patient age. In Stage 1, up to 8 patients between the ages of 16 and 25 years with early onset FSHD who met study entry criteria were enrolled. Stage 2 of enrollment, which was planned to include patients with early onset FSHD between the ages of 12 and 15 years, was to be initiated following an amendment to the study, based on consideration of safety data of ATYR1940 gathered in Stage 1 of this study, along with clinical safety data obtained in other studies of ATYR1940. The Sponsor elected not to conduct Stage 2.

| | |
|--|---------------------------------------|
| Arm type | Experimental |
| Investigational medicinal product name | ATYR1940 |
| Investigational medicinal product code | |
| Other name | |
| Pharmaceutical forms | Concentrate for solution for infusion |
| Routes of administration | Intravenous use |

Dosage and administration details:

Three dose levels of ATYR1940 were to be evaluated using inpatient dose escalation: 0.3, 1.0, and 3.0 mg/kg. Following a single, 90-minute IV placebo (normal saline Week 1) infusion, ATYR1940 was to be administered as a 90-minute IV infusion once a week for 12 weeks, starting at a dose of 0.3 mg/kg, with the potential for inpatient dose escalation over the dosing period.

During the 13-week treatment period, patients visited the clinic weekly for dosing and assessments of safety, immunogenicity, and PD activity. Patients also returned to the clinic 1, 4, and 12 weeks after the last dose of ATYR1940 for assessment of safety, immunogenicity, and biological and PD activity. The maximum duration of patient participation in the study was 28 weeks.

| Number of subjects in period 1 | ATYR1940 |
|---------------------------------------|----------|
| Started | 8 |
| Completed | 7 |
| Not completed | 1 |
| Lost to follow-up | 1 |

Baseline characteristics

Reporting groups

| | |
|-----------------------|----------|
| Reporting group title | ATYR1940 |
|-----------------------|----------|

Reporting group description:

Enrollment into the study was to be conducted in 2 stages, based on patient age. In Stage 1, up to 8 patients between the ages of 16 and 25 years with early onset FSHD who met study entry criteria were enrolled. Stage 2 of enrollment, which was planned to include patients with early onset FSHD between the ages of 12 and 15 years, was to be initiated following an amendment to the study, based on consideration of safety data of ATYR1940 gathered in Stage 1 of this study, along with clinical safety data obtained in other studies of ATYR1940. The Sponsor elected not to conduct Stage 2.

| Reporting group values | ATYR1940 | Total | |
|---------------------------|----------|-------|--|
| Number of subjects | 8 | 8 | |
| Age categorical | | | |
| Units: Subjects | | | |
| Adolescents (12-17 years) | 3 | 3 | |
| Adults (18-64 years) | 5 | 5 | |
| Age continuous | | | |
| Units: years | | | |
| arithmetic mean | 17.9 | | |
| full range (min-max) | 16 to 20 | - | |
| Gender categorical | | | |
| Units: Subjects | | | |
| Female | 3 | 3 | |
| Male | 5 | 5 | |

End points

End points reporting groups

| | |
|--|----------|
| Reporting group title | ATYR1940 |
| Reporting group description: | |
| Enrollment into the study was to be conducted in 2 stages, based on patient age. In Stage 1, up to 8 patients between the ages of 16 and 25 years with early onset FSHD who met study entry criteria were enrolled. Stage 2 of enrollment, which was planned to include patients with early onset FSHD between the ages of 12 and 15 years, was to be initiated following an amendment to the study, based on consideration of safety data of ATYR1940 gathered in Stage 1 of this study, along with clinical safety data obtained in other studies of ATYR1940. The Sponsor elected not to conduct Stage 2. | |

Primary: Anti-drug antibodies

| | |
|---|-------------------------------------|
| End point title | Anti-drug antibodies ^[1] |
| End point description: | |
| End point type | Primary |
| End point timeframe: | |
| Screening and weeks 4, 6, 8, 10, 13, 14, 17 and 25. The visits through week 13 were on-treatment, and visits 14, 17, 25 are post-treatment follow-up. | |
| Notes: | |
| [1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point. | |
| Justification: No statistical analysis was performed for any of the primary/safety endpoints. | |

| | | | | |
|---|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: number (frequency) of confirmed positive | 4 | | | |

Statistical analyses

No statistical analyses for this end point

Primary: Anti-Jo1 antibodies

| | |
|---|------------------------------------|
| End point title | Anti-Jo1 antibodies ^[2] |
| End point description: | |
| End point type | Primary |
| End point timeframe: | |
| Screening and weeks 3 to 25 | |
| 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 analysis was performed for any of the primary/safety endpoints. | |

| | | | | |
|---|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: number of patients positive or equivocal | 0 | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Heart Rate

| | |
|-----------------|------------|
| End point title | Heart Rate |
|-----------------|------------|

End point description:

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

End point timeframe:

Change from baseline at 1-week post-treatment follow-up

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 6 | | | |
| Units: BEATS/MIN | | | | |
| arithmetic mean (full range (min-max)) | -2.5 (-12 to 8) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Electrocardiogram - PR Duration

| | |
|-----------------|---------------------------------|
| End point title | Electrocardiogram - PR Duration |
|-----------------|---------------------------------|

End point description:

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

End point timeframe:

Change from baseline at 1-week post-treatment follow-up

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 6 | | | |
| Units: msec | | | | |
| arithmetic mean (full range (min-max)) | -7.7 (-32 to 8) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Electrocardiogram - QRS Duration

| | |
|-----------------|----------------------------------|
| End point title | Electrocardiogram - QRS Duration |
|-----------------|----------------------------------|

End point description:

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

End point timeframe:

Change from baseline at 1-week post-treatment follow-up

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 6 | | | |
| Units: msec | | | | |
| arithmetic mean (full range (min-max)) | -4.8 (-12 to 3) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Electrocardiogram - QTcF Interval

| | |
|-----------------|-----------------------------------|
| End point title | Electrocardiogram - QTcF Interval |
|-----------------|-----------------------------------|

End point description:

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

End point timeframe:

Change from baseline at 1-week post-treatment follow-up

| | | | | |
|--|------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 6 | | | |
| Units: msec | | | | |
| arithmetic mean (full range (min-max)) | -1.8 (-13 to 13) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: PFT - FEV1/FVC Ratio

| | |
|---------------------------------|----------------------|
| End point title | PFT - FEV1/FVC Ratio |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at week 13 | |

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: % | | | | |
| arithmetic mean (full range (min-max)) | 2.6 (-8 to 38) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Manual Muscle Testing - Overall total score

| | |
|---|---|
| End point title | Manual Muscle Testing - Overall total score |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Percent change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|--------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: percent | | | | |
| arithmetic mean (full range (min-max)) | 3.8 (-6.5 to 19.3) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: INQoL - QoL Score

| | |
|---|-------------------|
| End point title | INQoL - QoL Score |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|----------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 6 | | | |
| Units: None | | | | |
| arithmetic mean (full range (min-max)) | -1.2 (-17.3 to 13.4) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Hematocrit

| | |
|---|------------|
| End point title | Hematocrit |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

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|--|---------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: percent | | | | |
| arithmetic mean (full range (min-max)) | -0.01 (-2.5 to 3.1) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Hemoglobin

| | |
|---|------------|
| End point title | Hemoglobin |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: g/L | | | | |
| arithmetic mean (full range (min-max)) | 0.7 (-7 to 11) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Erythrocytes

| | |
|---|--------------|
| End point title | Erythrocytes |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|-----------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: 10 ¹² /L | | | | |
| arithmetic mean (full range (min-max)) | 0.027 (-0.16 to 0.36) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Leukocytes

| | |
|---|------------|
| End point title | Leukocytes |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|---------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: 10 ⁹ /L | | | | |
| arithmetic mean (full range (min-max)) | -0.10 (-1.2 to 1.0) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Platelets

| | |
|---|-----------|
| End point title | Platelets |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: 10 ⁹ /L | | | | |
| arithmetic mean (full range (min-max)) | 11.9 (-24 to 82) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Neutrophils

| | |
|---|-------------|
| End point title | Neutrophils |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|--------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: percent | | | | |
| arithmetic mean (full range (min-max)) | 1.84 (-6.2 to 7.9) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Lymphocytes

| | |
|---|-------------|
| End point title | Lymphocytes |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|---------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: percent | | | | |
| arithmetic mean (full range (min-max)) | -1.13 (-7.3 to 6.1) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Monocytes

| | |
|---|-----------|
| End point title | Monocytes |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|---------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: percent | | | | |
| arithmetic mean (full range (min-max)) | -0.50 (-1.4 to 0.2) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Eosinophils

| | |
|---|-------------|
| End point title | Eosinophils |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|---------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: percent | | | | |
| arithmetic mean (full range (min-max)) | -0.39 (-1.3 to 0.7) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Basophils

| | |
|---|-----------|
| End point title | Basophils |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|-------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: percent | | | | |
| arithmetic mean (full range (min-max)) | 0.17 (0.0 to 0.5) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Aspartate Aminotransferase

| | |
|---|----------------------------|
| End point title | Aspartate Aminotransferase |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: U/L | | | | |
| arithmetic mean (full range (min-max)) | 2.1 (-9 to 8) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Alanine Aminotransferase

| | |
|---|--------------------------|
| End point title | Alanine Aminotransferase |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: U/L | | | | |
| arithmetic mean (full range (min-max)) | -0.3 (-10 to 10) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Bilirubin

| | |
|---|-----------|
| End point title | Bilirubin |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|--------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: mcmol/L | | | | |
| arithmetic mean (full range (min-max)) | 1.69 (-0.7 to 5.7) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Blood Urea Nitrogen

| | |
|---|---------------------|
| End point title | Blood Urea Nitrogen |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|------------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: mmol/L | | | | |
| arithmetic mean (full range (min-max)) | -0.359 (-1.43 to 0.71) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Creatinine

| | |
|---|------------|
| End point title | Creatinine |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: mcmol/L | | | | |
| arithmetic mean (full range (min-max)) | 0.0 (-9 to 9) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Creatine Kinase

| | |
|---|-----------------|
| End point title | Creatine Kinase |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|--------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: U/L | | | | |
| arithmetic mean (full range (min-max)) | 99.4 (-296 to 485) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Cholesterol

| | |
|---|-------------|
| End point title | Cholesterol |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|------------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: mmol/L | | | | |
| arithmetic mean (full range (min-max)) | -0.100 (-1.22 to 0.54) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Sodium

| | |
|---|-----------|
| End point title | Sodium |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: mmol/L | | | | |
| arithmetic mean (full range (min-max)) | 0.1 (-3 to 3) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Potassium

| | |
|---|-----------|
| End point title | Potassium |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|---------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: mmol/L | | | | |
| arithmetic mean (full range (min-max)) | -0.04 (-0.3 to 0.3) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Bicarbonate

| | |
|---|-------------|
| End point title | Bicarbonate |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|-----------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 8 | | | |
| Units: mmol/L | | | | |
| arithmetic mean (full range (min-max)) | -1.9 (-7 to 3) | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Calcium

| | |
|---|-----------|
| End point title | Calcium |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| Change from baseline at 1-week post-treatment follow-up | |

| | | | | |
|--|-----------------------|--|--|--|
| End point values | ATYR1940 | | | |
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 7 | | | |
| Units: mmol/L | | | | |
| arithmetic mean (full range (min-max)) | 0.030 (-0.03 to 0.15) | | | |

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Throughout the study

Adverse event reporting additional description:

TEAEs reported for ≥ 2 patients treated with ATYR1940 are listed in the section below.

The number of occurrences per TEAE is not available in the source data, the field "Occurrences all number" therefore corresponds to the number of subjects affected per TEAE.

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

Dictionary used

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

| | |
|--------------------|------|
| Dictionary version | 18.1 |
|--------------------|------|

Reporting groups

| | |
|-----------------------|----------|
| Reporting group title | ATYR1940 |
|-----------------------|----------|

Reporting group description: -

| Serious adverse events | ATYR1940 | | |
|---|---------------|--|--|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 0 / 8 (0.00%) | | |
| number of deaths (all causes) | 0 | | |
| number of deaths resulting from adverse events | | | |

Frequency threshold for reporting non-serious adverse events: 1 %

| Non-serious adverse events | ATYR1940 | | |
|---|-----------------|--|--|
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 8 / 8 (100.00%) | | |
| Nervous system disorders | | | |
| Paraesthesia | | | |
| subjects affected / exposed | 3 / 8 (37.50%) | | |
| occurrences (all) | 3 | | |
| Headache | | | |
| subjects affected / exposed | 2 / 8 (25.00%) | | |
| occurrences (all) | 2 | | |
| Gastrointestinal disorders | | | |
| Nausea | | | |

| | | | |
|--|---------------------|--|--|
| subjects affected / exposed occurrences (all) | 2 / 8 (25.00%) 2 | | |
| Musculoskeletal and connective tissue disorders Myalgia subjects affected / exposed occurrences (all) | 3 / 8 (37.50%) 3 | | |
| Infections and infestations Nasopharyngitis subjects affected / exposed occurrences (all) | 2 / 8 (25.00%) 2 | | |

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

| Date | Amendment |
|-----------------|--|
| 12 January 2016 | Protocol version 2.0 dated 12 January 2016 |

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported