# Clinical trial results:
# A randomized, open-label, single center, phase I, cross-over study to evaluate the pharmacokinetic comparability of deferasirox new tablet formulation with the reference dispersible formulation in healthy adult subjects

## Summary

| | |
|---|---|
| EudraCT number | 2016-000248-32 |
| Trial protocol | Outside EU/EEA |
| Global end of trial date | 27 September 2012 |

**Results information**

| | |
|---|---|
| Result version number | v1 (current) |
| This version publication date | 26 July 2018 |
| First version publication date | 26 July 2018 |

## Trial information

**Trial identification**

| | |
|---|---|
| Sponsor protocol code | CICL670F2102 |

**Additional study identifiers**

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

Notes:

**Sponsors**

| | |
|---|---|
| Sponsor organisation name | Novartis Pharma AG |
| Sponsor organisation address | CH 4002, Basel, Switzerland, |
| Public contact | Clinical Disclosure Office, Novartis Pharma AG, +41 613241111, |
| Scientific contact | Clinical Disclosure Office, Novartis Pharma AG, +41 613241111, |

Notes:

**Paediatric regulatory details**

| | |
|---|---|
| Is trial part of an agreed paediatric investigation plan (PIP) | Yes |
| EMA paediatric investigation plan number(s) | EMEA-001103-PIP01-10 |
| 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 | 27 September 2012 |
| Is this the analysis of the primary completion data? | No |

| | |
|---|---|
| Global end of trial reached? | Yes |
| Global end of trial date | 27 September 2012 |
| Was the trial ended prematurely? | No |

Notes:

## General information about the trial

Main objective of the trial:

The main objective of the trial was to evaluate the pharmacokinetic (PK) comparability of deferasirox with a reduced dosage strength of the new oral film coated tablet vs. the reference marketed dispersible tablets in healthy subjects under fasted conditions.

Protection of trial subjects:

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

| | |
|---|---|
| Background therapy: - | |
| Evidence for comparator: - | |
| Actual start date of recruitment | 19 July 2012 |
| Long term follow-up planned | No |
| Independent data monitoring committee (IDMC) involvement? | No |

Notes:

## Population of trial subjects

### Subjects enrolled per country

| | |
|---|---|
| Country: Number of subjects enrolled | United States: 44 |
| Worldwide total number of subjects | 44 |
| EEA total number of subjects | 0 |

Notes:

### Subjects enrolled per age group

| | |
|---|---|
| In utero | 0 |
| Preterm newborn - gestational age < 37 wk | 0 |
| Newborns (0-27 days) | 0 |
| Infants and toddlers (28 days-23 months) | 0 |
| Children (2-11 years) | 0 |
| Adolescents (12-17 years) | 0 |
| Adults (18-64 years) | 44 |
| From 65 to 84 years | 0 |

| | |
|---|---|
| 85 years and over | 0 |

## Subject disposition

### Recruitment

Recruitment details:

The study was conducted at a single center in United States.

### Pre-assignment

Screening details:

Of the 44 subjects enrolled, only 34 subjects were randomized and treated. Remaining 10 subjects were not randomized, received only iron-supplementation and discontinued due to following reasons: Administrative problem (7), Subject withdrawn consent (2), Lost to follow-up (1).

### 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 |
| **Arm title** | Deferasirox (Film coated then dispersible tablets) |

Arm description:

Subjects were initially administered with 3*360 milligrams (mg) film coated tablets of deferasirox for a total dose of 1080 mg, followed by 3*500 mg dispersible tablets of deferasirox for a total of 1500 mg. Subjects received deferasirox with a glass of water (240 milliliter (mL) in the morning after an overnight fast (last meal or snack taken at least 10 hours (hr) earlier) and at least 1 hr before breakfast. After ingestion of dispersible tablets, the glass was rinsed with an additional 40 mL of water and administered to the subject. A washout period of 8 days was maintained between two treatments.

| | |
|---|---|
| Arm type | Experimental |
| Investigational medicinal product name | Deferasirox |
| Investigational medicinal product code | ICL670 |
| Other name | |
| Pharmaceutical forms | Dispersible tablet, Film-coated tablet |
| Routes of administration | Oral use |

Dosage and administration details:

Subjects were orally administered with 3*360 mg deferasirox film coated tablet followed by 3*500 mg deferasirox dispersible tablet in morning after an overnight fast (last meal or snack taken at least 10 hrs earlier) and at least 1 hr before breakfast. A washout period of 8 day was maintained between two treatments.

| | |
|---|---|
| **Arm title** | Deferasirox (Dispersible then film coated tablets) |

Arm description:

Subjects were initially administered with 3*500 mg dispersible tablets of deferasirox for a total of 1500 mg followed by 3*360 mg film coated tablets of deferasirox for a total dose of 1080 mg. Subjects received deferasirox with a glass of water (240 mL in the morning after an overnight fast (last meal or snack taken at least 10 hr earlier) and at least 1 hr before breakfast. After ingestion of dispersible tablets, the glass was rinsed with an additional 40 mL of water and administered to the subject. A washout period of 8 day was maintained between two treatments.

| | |
|---|---|
| Arm type | Experimental |
| Investigational medicinal product name | Deferasirox |
| Investigational medicinal product code | ICL670 |
| Other name | |
| Pharmaceutical forms | Dispersible tablet, Film-coated tablet |
| Routes of administration | Oral use |

Dosage and administration details:

Subjects were orally administered with 3*500 mg deferasirox dispersible tablet followed by 3*360 mg deferasirox film coated tablet in morning after an overnight fast (last meal or snack taken at least 10 hr

earlier) and at least 1 hr before breakfast. A washout period of 8 day was maintained between two treatments.

| **Number of subjects in period 1[1]** | Deferasirox (Film coated then dispersible tablets) | Deferasirox (Dispersible then film coated tablets) |
|---|---|---|
| Started | 17 | 17 |
| Completed | 16 | 16 |
| Not completed | 1 | 1 |
| Consent withdrawn by subject | - | 1 |
| Administrative Problems | 1 | - |

Notes:

[1] - The number of subjects reported to be in the baseline 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 number of subjects reported to be in the baseline period are not the same as the worldwide number enrolled as out of 44 subjects, only 34 subjects were randomized and treated in the trial.

## Baseline characteristics

| Reporting groups | |
|---|---|
| Reporting group title | Deferasirox (Film coated then dispersible tablets) |

Reporting group description:

Subjects were initially administered with 3*360 milligrams (mg) film coated tablets of deferasirox for a total dose of 1080 mg, followed by 3*500 mg dispersible tablets of deferasirox for a total of 1500 mg. Subjects received deferasirox with a glass of water (240 milliliter (mL) in the morning after an overnight fast (last meal or snack taken at least 10 hours (hr) earlier) and at least 1 hr before breakfast. After ingestion of dispersible tablets, the glass was rinsed with an additional 40 mL of water and administered to the subject. A washout period of 8 days was maintained between two treatments.

| Reporting group title | Deferasirox (Dispersible then film coated tablets) |
|---|---|

Reporting group description:

Subjects were initially administered with 3*500 mg dispersible tablets of deferasirox for a total of 1500 mg followed by 3*360 mg film coated tablets of deferasirox for a total dose of 1080 mg. Subjects received deferasirox with a glass of water (240 mL in the morning after an overnight fast (last meal or snack taken at least 10 hr earlier) and at least 1 hr before breakfast. After ingestion of dispersible tablets, the glass was rinsed with an additional 40 mL of water and administered to the subject. A washout period of 8 day was maintained between two treatments.

| Reporting group values | Deferasirox (Film coated then dispersible tablets) | Deferasirox (Dispersible then film coated tablets) | Total |
|---|---|---|---|
| Number of subjects | 17 | 17 | 34 |
| Age categorical<br>Units: Subjects | | | |
| Adults (18-55 years) | 17 | 17 | 34 |
| Age continuous<br>Units: years | | | |
| arithmetic mean | 35.53 | 36.71 | |
| standard deviation | ± 12.841 | ± 11.351 | - |
| Gender categorical<br>Units: Subjects | | | |
| Female | 5 | 5 | 10 |
| Male | 12 | 12 | 24 |

## End points

### End points reporting groups

| Reporting group title | Deferasirox (Film coated then dispersible tablets) |
|---|---|

Reporting group description:

Subjects were initially administered with 3*360 milligrams (mg) film coated tablets of deferasirox for a total dose of 1080 mg, followed by 3*500 mg dispersible tablets of deferasirox for a total of 1500 mg. Subjects received deferasirox with a glass of water (240 milliliter (mL) in the morning after an overnight fast (last meal or snack taken at least 10 hours (hr) earlier) and at least 1 hr before breakfast. After ingestion of dispersible tablets, the glass was rinsed with an additional 40 mL of water and administered to the subject. A washout period of 8 days was maintained between two treatments.

| Reporting group title | Deferasirox (Dispersible then film coated tablets) |
|---|---|

Reporting group description:

Subjects were initially administered with 3*500 mg dispersible tablets of deferasirox for a total of 1500 mg followed by 3*360 mg film coated tablets of deferasirox for a total dose of 1080 mg. Subjects received deferasirox with a glass of water (240 mL in the morning after an overnight fast (last meal or snack taken at least 10 hr earlier) and at least 1 hr before breakfast. After ingestion of dispersible tablets, the glass was rinsed with an additional 40 mL of water and administered to the subject. A washout period of 8 day was maintained between two treatments.

| Subject analysis set title | Deferasirox (Film coated tablet) |
|---|---|
| Subject analysis set type | Sub-group analysis |

Subject analysis set description:

All subjects who were administered with a total dose of 3*360 mg (1080 mg) deferasirox as film coated tablets with a glass of water (240 mL in the morning after an overnight fast (last meal or snack taken at least 10 hr earlier) and at least 1 hr before breakfast. The subjects were analysed based on PK analysis set (PAS) defined as all safety subjects (subjects who received at least one dose of investigational product) who completed both treatment periods and provided evaluable PK.

| Subject analysis set title | Deferasirox (Dispersible tablet) |
|---|---|
| Subject analysis set type | Sub-group analysis |

Subject analysis set description:

All subjects who were administered with a total dose of 3*500 mg (1500 mg) deferasirox as dispersible tablets with a glass of water (240 mL in the morning after an overnight fast (last meal or snack taken at least 10 hr earlier) and at least 1 hr before breakfast. After ingestion of dispersible tablets, the glass was rinsed with an additional 40 mL of water and administered to the subject. The analysis was performed on PAS population.

| Subject analysis set title | Iron Supplement |
|---|---|
| Subject analysis set type | Safety analysis |

Subject analysis set description:

Subjects were daily administered with iron supplement (ferrous sulfate) 325 mg oral tablet as supportive treatment for 8 days prior to deferasirox treatment. A washout period of 6 days was maintained between supportive and deferasirox treatment. The subjects were analysed based on safety set defined as all randomized subjects who received at least one dose of investigational product (deferasirox or iron supplement).

### Primary: Area under the concentration-time curve from time zero to the last measurable concentration (AUClast) of deferasirox

| End point title | Area under the concentration-time curve from time zero to the last measurable concentration (AUClast) of deferasirox |
|---|---|

End point description:

AUClast was defined as the area under the concentration-time curve from time zero to the last measurable concentration sampling time micromole (µmol)*hr /Litre (L). The AUClast was derived based on the non-compartmental methods using Phoenix WinNonlin version 6.2. The analysis was performed in PAS population.

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

End point timeframe:

Pre-dose, 0.5, 1, 1.5, 2, 3, 4, 6, 8, 12, 24, 36, 48, and 72 hr post-dose

| End point values | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | | |
|---|---|---|---|---|
| Subject group type | Subject analysis set | Subject analysis set | | |
| Number of subjects analysed | 32 | 32 | | |
| Units: µmol*hr/L | | | | |
| geometric mean (geometric coefficient of variation) | 1273.78 (± 39.22) | 1270.79 (± 49.7) | | |

## Statistical analyses

| Statistical analysis title | AUClast of deferasirox |
|---|---|

Statistical analysis description:

Geometric mean ratio of AUClast for deferasirox (film-coated tablet and dispersible tablet) was evaluated. The total number of subjects analysed in this endpoint were 32 but as this is a cross-over study the subjects analysed below are featuring as 64 as the number of subjects adds up on selecting the two arms that are being compared [Deferasirox Film coated tablet (N=32) and Deferasirox Dispersible tablet (N=32)].

| | |
|---|---|
| Comparison groups | Deferasirox (Dispersible tablet) v Deferasirox (Film coated tablet) |
| Number of subjects included in analysis | 64 |
| Analysis specification | Pre-specified |
| Analysis type | other |
| Parameter estimate | Geometric Mean Ratio |
| Point estimate | 1 |
| Confidence interval | |
| level | 90 % |
| sides | 2-sided |
| lower limit | 0.932 |
| upper limit | 1.078 |

## Primary: Area under the plasma concentration-time curve from time zero to infinity (AUCinf) of deferasirox

| End point title | Area under the plasma concentration-time curve from time zero to infinity (AUCinf) of deferasirox |
|---|---|

End point description:

AUCinf was defined as the area under the plasma concentration-time curve from time zero to infinity (µmol*hr/L). The AUCinf was derived based on the non-compartmental methods using Phoenix WinNonlin version 6.2. The analysis was performed in PAS population.

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

End point timeframe:

Pre-dose, 0.5, 1, 1.5, 2, 3, 4, 6, 8, 12, 24, 36, 48, and 72 hr post-dose

| End point values | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | | |
|---|---|---|---|---|
| Subject group type | Subject analysis set | Subject analysis set | | |
| Number of subjects analysed | 32 | 32 | | |
| Units: µmol*hr/L | | | | |
| geometric mean (geometric coefficient of variation) | 1307.04 (± 39.15) | 1327.01 (± 50.29) | | |

## Statistical analyses

| Statistical analysis title | AUCinf of deferasirox |
|---|---|

Statistical analysis description:

Geometric mean ratio of AUCinf for deferasirox (film-coated tablet and dispersible tablet) was evaluated. The total number of subjects analysed in this endpoint were 32 but as this is a cross-over study the subjects analysed below are featuring as 64 as the number of subjects adds up on selecting the two arms that are being compared [Deferasirox Film coated tablet (N=32) and Deferasirox Dispersible tablet (N=32)].

| | |
|---|---|
| Comparison groups | Deferasirox (Film coated tablet) v Deferasirox (Dispersible tablet) |
| Number of subjects included in analysis | 64 |
| Analysis specification | Pre-specified |
| Analysis type | other |
| Parameter estimate | Geometric Ratio |
| Point estimate | 0.98 |
| Confidence interval | |
| level | 90 % |
| sides | 2-sided |
| lower limit | 0.916 |
| upper limit | 1.059 |

## Primary: Maximum (peak) observed plasma concentration after a single-dose administration (Cmax) of deferasirox

| End point title | Maximum (peak) observed plasma concentration after a single-dose administration (Cmax) of deferasirox |
|---|---|

End point description:

Cmax was defined as the maximum (peak) observed plasma concentration after a single-dose administration (µmol/L). The Cmax was derived based on the non-compartmental methods using Phoenix WinNonlin version 6.2. The analysis was performed in PAS population.

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

End point timeframe:

Pre-dose, 0.5, 1, 1.5, 2, 3, 4, 6, 8, 12, 24, 36, 48, and 72 hr post-dose

| End point values | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | | |
|---|---|---|---|---|
| Subject group type | Subject analysis set | Subject analysis set | | |
| Number of subjects analysed | 32 | 32 | | |
| Units: µmol/L | | | | |
| geometric mean (geometric coefficient of variation) | 105.83 (± 27.54) | 81.54 (± 33.35) | | |

## Statistical analyses

| Statistical analysis title | Cmax of deferasirox |
|---|---|

Statistical analysis description:

Geometric mean ratio of Cmax for deferasirox (film-coated tablet and dispersible tablet) was evaluated. The total number of subjects analysed in this endpoint were 32 but as this is a cross-over study the subjects analysed below are featuring as 64 as the number of subjects adds up on selecting the two arms that are being compared [Deferasirox Film coated tablet (N=32) and Deferasirox Dispersible tablet (N=32)].

| | |
|---|---|
| Comparison groups | Deferasirox (Film coated tablet) v Deferasirox (Dispersible tablet) |
| Number of subjects included in analysis | 64 |
| Analysis specification | Pre-specified |
| Analysis type | other |
| Parameter estimate | Geometric Ratio |
| Point estimate | 1.3 |
| Confidence interval | |
| level | 90 % |
| sides | 2-sided |
| lower limit | 1.203 |
| upper limit | 1.4 |

## Secondary: Time to reach maximum (peak) plasma concentration after a single-dose administration (Tmax) of deferasirox

| End point title | Time to reach maximum (peak) plasma concentration after a single-dose administration (Tmax) of deferasirox |
|---|---|

End point description:

Tmax was defined as the time to reach maximum (peak) plasma concentration after a single-dose administration (hr). The Tmax was derived based on the non-compartmental methods using Phoenix WinNonlin version 6.2. The analysis was performed in PAS population.

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

End point timeframe:

Pre-dose, 0.5, 1, 1.5, 2, 3, 4, 6, 8, 12, 24, 36, 48, and 72 hr post-dose

| End point values | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | | |
|---|---|---|---|---|
| Subject group type | Subject analysis set | Subject analysis set | | |
| Number of subjects analysed | 32 | 32 | | |
| Units: hr | | | | |
| median (full range (min-max)) | 2 (1.5 to 6.03) | 3 (1 to 8) | | |

### Statistical analyses

No statistical analyses for this end point

## Secondary: Elimination half-life with the terminal slope (lambda_z) of a semi-logarithmic concentration-time curve (T1/2) of deferasirox

| End point title | Elimination half-life with the terminal slope (lambda_z) of a semi-logarithmic concentration-time curve (T1/2) of deferasirox |
|---|---|

End point description:

T1/2 was defined as the elimination half-life with the terminal slope (i.e. lambda_z) of a semi-logarithmic concentration-time curve (hr). The T1/2 was derived based on the non-compartmental methods using Phoenix WinNonlin version 6.2. The analysis was performed in PAS population.

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

End point timeframe:

Pre-dose, 0.5, 1, 1.5, 2, 3, 4, 6, 8, 12, 24, 36, 48, and 72 hr post-dose

| End point values | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | | |
|---|---|---|---|---|
| Subject group type | Subject analysis set | Subject analysis set | | |
| Number of subjects analysed | 32 | 32 | | |
| Units: hr | | | | |
| geometric mean (geometric coefficient of variation) | 12.48 (± 30.69) | 15.64 (± 29.47) | | |

### Statistical analyses

No statistical analyses for this end point

## Secondary: Terminal slope of the elimination phase (Lambda_z) of deferasirox

| End point title | Terminal slope of the elimination phase (Lambda_z) of deferasirox |
|---|---|

End point description:

Lambda_z was defined as the terminal slope of elimination phase (1/hr). The Lambda_z was derived based on the non-compartmental methods using Phoenix WinNonlin version 6.2. The analysis was performed in PAS population.

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

End point timeframe:

Pre-dose, 0.5, 1, 1.5, 2, 3, 4, 6, 8, 12, 24, 36, 48, and 72 hr post-dose

| End point values | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | | |
|---|---|---|---|---|
| Subject group type | Subject analysis set | Subject analysis set | | |
| Number of subjects analysed | 32 | 32 | | |
| Units: 1/hr | | | | |
| geometric mean (geometric coefficient of variation) | 0.06 (± 30.7) | 0.04 (± 29.45) | | |

## Statistical analyses

No statistical analyses for this end point

## Secondary: Number of subjects with Adverse events (AEs), Serious adverse events (SAEs), Discontinued due to AEs and who died

| End point title | Number of subjects with Adverse events (AEs), Serious adverse events (SAEs), Discontinued due to AEs and who died |
|---|---|

End point description:

AEs are defined as any unfavorable and unintended diagnosis, symptom, sign (including an abnormal laboratory finding), syndrome or disease which either occurs during study, having been absent at baseline, or, if present at baseline, appears to worsen. Serious adverse events are any untoward medical occurrences that result in death, are life threatening, require (or prolong) hospitalisation, cause persistent or significant disability/incapacity, result in congenital anomalies or birth defects, or are other conditions which in judgment of investigators represent significant hazards. The analysis was performed on safety set population.

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

End point timeframe:

From Day 1 up to Day 43

| End point values | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | Iron Supplement | |
|---|---|---|---|---|
| Subject group type | Subject analysis set | Subject analysis set | Subject analysis set | |
| Number of subjects analysed | 32 | 32 | 34 | |
| Units: Subjects | | | | |
| AEs | 11 | 6 | 3 | |
| SAEs | 0 | 0 | 0 | |
| Deaths | 0 | 0 | 0 | |
| AEs leading to discontinuation | 0 | 0 | 0 | |

## Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

AEs were collected from First Subject First Visit (FSFV) until Last Subject Last Visit (LSLV). All AEs reported in this record were from date of First Subject First Treatment until LSLV.

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

### Dictionary used

| | |
|---|---|
| Dictionary name | MedDRA |
| Dictionary version | 15.0 |

### Reporting groups

| | |
|---|---|
| Reporting group title | Deferasirox (Film coated tablet) |

Reporting group description:

All subjects who received a total dose of 3*360 mg (1080 mg) deferasirox as film coated tablets with a glass of water (240 mL in the morning after an overnight fast (last meal or snack taken at least 10 hr earlier) and at least 1 hr before breakfast during the study.

| | |
|---|---|
| Reporting group title | Deferasirox (Dispersible tablet) |

Reporting group description:

All subjects who recieved a total dose of 3*500 mg (1500 mg) deferasirox as dispersible tablets with a glass of water (240 mL in the morning after an overnight fast (last meal or snack taken at least 10 hr earlier) and at least 1 hr before breakfast during the study.

| | |
|---|---|
| Reporting group title | Iron supplement |

Reporting group description:

All subjects who recieved iron supplement (ferrous sulfate) 325 mg oral tablet as supportive treatment for 8 days prior to deferasirox treatment during the study.

| | |
|---|---|
| Reporting group title | All Subjects |

Reporting group description:

All Subjects who received at least one dose of study treatment.

| Serious adverse events | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | Iron supplement |
|---|---|---|---|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 0 / 32 (0.00%) | 0 / 32 (0.00%) | 0 / 34 (0.00%) |
| number of deaths (all causes) | 0 | 0 | 0 |
| number of deaths resulting from adverse events | 0 | 0 | 0 |

| Serious adverse events | All Subjects | | |
|---|---|---|---|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 0 / 34 (0.00%) | | |
| number of deaths (all causes) | 0 | | |
| number of deaths resulting from adverse events | 0 | | |

Frequency threshold for reporting non-serious adverse events: 2 %

| **Non-serious adverse events** | Deferasirox (Film coated tablet) | Deferasirox (Dispersible tablet) | Iron supplement |
|---|---|---|---|
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 11 / 32 (34.38%) | 6 / 32 (18.75%) | 3 / 34 (8.82%) |
| Nervous system disorders | | | |
| Dizziness | | | |
| subjects affected / exposed | 1 / 32 (3.13%) | 0 / 32 (0.00%) | 0 / 34 (0.00%) |
| occurrences (all) | 1 | 0 | 0 |
| Headache | | | |
| subjects affected / exposed | 1 / 32 (3.13%) | 2 / 32 (6.25%) | 0 / 34 (0.00%) |
| occurrences (all) | 1 | 2 | 0 |
| General disorders and administration site conditions | | | |
| Fatigue | | | |
| subjects affected / exposed | 1 / 32 (3.13%) | 0 / 32 (0.00%) | 0 / 34 (0.00%) |
| occurrences (all) | 1 | 0 | 0 |
| Pain | | | |
| subjects affected / exposed | 0 / 32 (0.00%) | 1 / 32 (3.13%) | 0 / 34 (0.00%) |
| occurrences (all) | 0 | 1 | 0 |
| Gastrointestinal disorders | | | |
| Abdominal distension | | | |
| subjects affected / exposed | 1 / 32 (3.13%) | 0 / 32 (0.00%) | 0 / 34 (0.00%) |
| occurrences (all) | 1 | 0 | 0 |
| Abnormal faeces | | | |
| subjects affected / exposed | 0 / 32 (0.00%) | 0 / 32 (0.00%) | 1 / 34 (2.94%) |
| occurrences (all) | 0 | 0 | 1 |
| Diarrhoea | | | |
| subjects affected / exposed | 7 / 32 (21.88%) | 3 / 32 (9.38%) | 0 / 34 (0.00%) |
| occurrences (all) | 7 | 3 | 0 |
| Dyspepsia | | | |
| subjects affected / exposed | 1 / 32 (3.13%) | 1 / 32 (3.13%) | 0 / 34 (0.00%) |
| occurrences (all) | 1 | 1 | 0 |
| Faeces discoloured | | | |
| subjects affected / exposed | 0 / 32 (0.00%) | 0 / 32 (0.00%) | 1 / 34 (2.94%) |
| occurrences (all) | 0 | 0 | 1 |
| Infrequent bowel movements | | | |

| | | | |
|---|---|---|---|
| subjects affected / exposed | 1 / 32 (3.13%) | 0 / 32 (0.00%) | 0 / 34 (0.00%) |
| occurrences (all) | 1 | 0 | 0 |
| Reproductive system and breast disorders | | | |
| Menstruation irregular | | | |
| subjects affected / exposed | 1 / 32 (3.13%) | 0 / 32 (0.00%) | 0 / 34 (0.00%) |
| occurrences (all) | 1 | 0 | 0 |
| Skin and subcutaneous tissue disorders | | | |
| Erythema | | | |
| subjects affected / exposed | 0 / 32 (0.00%) | 0 / 32 (0.00%) | 1 / 34 (2.94%) |
| occurrences (all) | 0 | 0 | 1 |
| Papule | | | |
| subjects affected / exposed | 0 / 32 (0.00%) | 1 / 32 (3.13%) | 0 / 34 (0.00%) |
| occurrences (all) | 0 | 1 | 0 |
| Pruritus | | | |
| subjects affected / exposed | 0 / 32 (0.00%) | 0 / 32 (0.00%) | 1 / 34 (2.94%) |
| occurrences (all) | 0 | 0 | 1 |

| **Non-serious adverse events** | All Subjects | | |
|---|---|---|---|
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 15 / 34 (44.12%) | | |
| Nervous system disorders | | | |
| Dizziness | | | |
| subjects affected / exposed | 1 / 34 (2.94%) | | |
| occurrences (all) | 1 | | |
| Headache | | | |
| subjects affected / exposed | 3 / 34 (8.82%) | | |
| occurrences (all) | 3 | | |
| General disorders and administration site conditions | | | |
| Fatigue | | | |
| subjects affected / exposed | 1 / 34 (2.94%) | | |
| occurrences (all) | 1 | | |
| Pain | | | |
| subjects affected / exposed | 1 / 34 (2.94%) | | |
| occurrences (all) | 1 | | |
| Gastrointestinal disorders | | | |

| | | | |
|---|---|---|---|
| Abdominal distension<br>subjects affected / exposed<br>occurrences (all) | 1 / 34 (2.94%)<br>1 | | |
| Abnormal faeces<br>subjects affected / exposed<br>occurrences (all) | 1 / 34 (2.94%)<br>1 | | |
| Diarrhoea<br>subjects affected / exposed<br>occurrences (all) | 8 / 34 (23.53%)<br>10 | | |
| Dyspepsia<br>subjects affected / exposed<br>occurrences (all) | 2 / 34 (5.88%)<br>2 | | |
| Faeces discoloured<br>subjects affected / exposed<br>occurrences (all) | 1 / 34 (2.94%)<br>1 | | |
| Infrequent bowel movements<br>subjects affected / exposed<br>occurrences (all) | 1 / 34 (2.94%)<br>1 | | |
| Reproductive system and breast disorders | | | |
| Menstruation irregular<br>subjects affected / exposed<br>occurrences (all) | 1 / 34 (2.94%)<br>1 | | |
| Skin and subcutaneous tissue disorders | | | |
| Erythema<br>subjects affected / exposed<br>occurrences (all) | 1 / 34 (2.94%)<br>1 | | |
| Papule<br>subjects affected / exposed<br>occurrences (all) | 1 / 34 (2.94%)<br>1 | | |
| Pruritus<br>subjects affected / exposed<br>occurrences (all) | 1 / 34 (2.94%)<br>1 | | |

## More information

### Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

### Interruptions (globally)

Were there any global interruptions to the trial? No

### Limitations and caveats

None reported