



Clinical trial results:

Randomised, open-label, active-controlled, multicentre, comparative study to evaluate the safety and efficacy of ferric maltol (iron(III)-maltol complex) (ST10) oral suspension compared to ferrous sulfate oral liquid in children and adolescents aged 2 to 17 years with iron-deficiency anaemia, incorporating a single arm study in infants aged 1 month to less than 2 years

Summary

EudraCT number	2018-000078-31
Trial protocol	Outside EU/EEA
Global end of trial date	09 June 2024

Results information

Result version number	v1 (current)
This version publication date	27 July 2025
First version publication date	27 July 2025

Trial information

Trial identification

Sponsor protocol code	ST10-01-305
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05126901
WHO universal trial number (UTN)	-
Other trial identifiers	USA IND: 114832

Notes:

Sponsors

Sponsor organisation name	Shield TX (UK) Ltd.
Sponsor organisation address	Northern Design Centre, Baltic Business Quarter, Gateshead, United Kingdom, NE8 3DF
Public contact	Clinical Operations, Shield TX (UK) Ltd., info@shieldtherapeutics.com
Scientific contact	Clinical Operations, Shield TX (UK) Ltd., info@shieldtherapeutics.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMA-001195-PIP01-11
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	09 June 2024
Is this the analysis of the primary completion data?	Yes
Primary completion date	09 June 2024
Global end of trial reached?	Yes
Global end of trial date	09 June 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

1. To compare the safety and gastrointestinal tolerability of ferric maltol oral suspension and ferrous sulfate oral liquid in children and adolescents aged 2 years to 17 years, and assess the safety and tolerability of ferric maltol oral suspension in children 1 month to less than 2 years, in the treatment of iron deficiency anaemia during the 12 weeks treatment period; and
2. To assess the effect on Hb in children and adolescents aged 1 month to 17 years after BID ferric maltol oral suspension administration for 12 weeks.

Protection of trial subjects:

The study was conducted in accordance with the Declaration of Helsinki and with all applicable laws and regulations of the locales and countries where the study was conducted, and in compliance with Good Clinical Practice Guidelines.

Background therapy: -

Evidence for comparator:

The mainstay of treatment of iron deficiency anemia is oral iron supplements. Ferrous compounds (sulfate, fumarate, and gluconate), which are available both in solid and liquid forms, are the most common due to the extremely low bioavailability of conventional ferric preparations.

Actual start date of recruitment	03 November 2021
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: 52
Country: Number of subjects enrolled	United Kingdom: 13
Worldwide total number of subjects	65
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)	4
Children (2-11 years)	21
Adolescents (12-17 years)	40
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This was a multicenter study with a total of 23 clinical sites located in the United States (including Puerto Rico) and United Kingdom. A total of 65 patients were enrolled in the study.

Pre-assignment

Screening details:

All eligible subjects aged 1 month to less than 2 years entered a Pre-assignment phase, 1-day Pharmacokinetic assessment day following a single dose of ferric maltol oral suspension.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	Ferrous Sulfate Randomized

Arm description:

Patients aged 2 to 17 years were randomized 1:1 to receive ferric maltol oral suspension or ferrous sulfate oral liquid.

Arm type	Active comparator
Investigational medicinal product name	Ferrous sulfate
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Oral liquid
Routes of administration	Oral use

Dosage and administration details:

Ferrous sulfate oral liquid 125 mg/mL (25 mg/mL elemental iron) or equivalent concentration was used for all children/adolescents. To maximize the iron replenishment, patients aged 2 to 17 years were dosed with 0.24 mL (6 mg elemental iron) per kg body weight, up to a maximum of 8 mL given daily in 2 divided doses.

Arm title	Ferric Maltol Randomized
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Arm description:

Subjects aged 2-17 will be randomised 1:1 to receive ferric maltol oral suspension or ferrous sulfate oral liquid.

The first 12 subjects randomised to ferric maltol in each age sub-group (2 - 9 yrs, 10 - 17 yrs respectively) will enter a PK phase with 2 PK days.

Following PK Day 2 subjects will continue until Week 12. Once the 18 subjects in each age subgroup have finished their PK visits, they will continue until week 12.

Ferrous maltol: 2-11 years 15 mg elemental iron (2.5mL) BID; 12-17 years 30mg elemental iron (5mL) BID

Arm type	Experimental
Investigational medicinal product name	Ferric maltol
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Oral liquid
Routes of administration	Oral use

Dosage and administration details:

The dose of ferric maltol oral suspension administered for children aged 2 to - 11 yrs was 2.5 ml BID and for children aged 12-17 yrs: 5 ml BID.

Arm title	Ferric Maltol Assigned
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Arm description:

12 weeks open-label Treatment Period for ferric maltol children aged 1 month to <2 years

Arm type	Experimental
Investigational medicinal product name	Ferric maltol
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Oral liquid
Routes of administration	Oral use

Dosage and administration details:

The dose of ferric maltol oral suspension that was administered for children aged 1 month to < 2 yrs is 0.1 ml/kg BID

Number of subjects in period 1	Ferrous Sulfate Randomized	Ferric Maltol Randomized	Ferric Maltol Assigned
Started	30	31	4
Completed	25	28	3
Not completed	5	3	1
Consent withdrawn by subject	4	1	1
Adverse event, non-fatal	1	-	-
Lost to follow-up	-	2	-

Baseline characteristics

Reporting groups

Reporting group title	Ferrous Sulfate Randomized
Reporting group description: Patients aged 2 to 17 years were randomized 1:1 to receive ferric maltol oral suspension or ferrous sulfate oral liquid.	
Reporting group title	Ferric Maltol Randomized
Reporting group description: Subjects aged 2-17 will be randomised 1:1 to receive ferric maltol oral suspension or ferrous sulfate oral liquid. The first 12 subjects randomised to ferric maltol in each age sub-group (2 - 9 yrs, 10 - 17 yrs respectively) will enter a PK phase with 2 PK days. Following PK Day 2 subjects will continue until Week 12. Once the 18 subjects in each age subgroup have finished their PK visits, they will continue until week 12. Ferrous maltol: 2-11 years 15 mg elemental iron (2.5mL) BID; 12-17 years 30mg elemental iron (5mL) BID	
Reporting group title	Ferric Maltol Assigned
Reporting group description: 12 weeks open-label Treatment Period for ferric maltol children aged 1 month to <2 years	

Reporting group values	Ferrous Sulfate Randomized	Ferric Maltol Randomized	Ferric Maltol Assigned
Number of subjects	30	31	4
Age categorical 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)	0	0	4
Children (2-11 years)	11	10	0
Adolescents (12-17 years)	19	21	0
Adults (18-64 years)	0	0	0
From 65-84 years	0	0	0
85 years and over	0	0	0
Gender categorical Units: Subjects			
Female	22	23	3
Male	8	8	1

Reporting group values	Total		
Number of subjects	65		
Age categorical 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)	4		
Children (2-11 years)	21		

Adolescents (12-17 years)	40		
Adults (18-64 years)	0		
From 65-84 years	0		
85 years and over	0		
Gender categorical			
Units: Subjects			
Female	48		
Male	17		

End points

End points reporting groups

Reporting group title	Ferrous Sulfate Randomized
Reporting group description: Patients aged 2 to 17 years were randomized 1:1 to receive ferric maltol oral suspension or ferrous sulfate oral liquid.	
Reporting group title	Ferric Maltol Randomized
Reporting group description: Subjects aged 2-17 will be randomised 1:1 to receive ferric maltol oral suspension or ferrous sulfate oral liquid. The first 12 subjects randomised to ferric maltol in each age sub-group (2 - 9 yrs, 10 - 17 yrs respectively) will enter a PK phase with 2 PK days. Following PK Day 2 subjects will continue until Week 12. Once the 18 subjects in each age subgroup have finished their PK visits, they will continue until week 12. Ferrous maltol: 2-11 years 15 mg elemental iron (2.5mL) BID; 12-17 years 30mg elemental iron (5mL) BID	
Reporting group title	Ferric Maltol Assigned
Reporting group description: 12 weeks open-label Treatment Period for ferric maltol children aged 1 month to <2 years	
Subject analysis set title	mITT Ferrous Sulfate
Subject analysis set type	Modified intention-to-treat
Subject analysis set description: The Randomized/ITT Population is defined as all patients who were randomized/assigned to treatment arms. The mITT Population is defined as all patients in the ITT Population who received at least 1 treatment dose.	
Subject analysis set title	mITT Ferric Maltol
Subject analysis set type	Modified intention-to-treat
Subject analysis set description: The Randomized/ITT Population is defined as all patients who were randomized/assigned to treatment arms. The mITT Population is defined as all patients in the ITT Population who received at least 1 treatment dose.	
Subject analysis set title	mITT Ferric Maltol Assigned
Subject analysis set type	Modified intention-to-treat
Subject analysis set description: The Randomized/ITT Population is defined as all patients who were randomized/assigned to treatment arms. The mITT Population is defined as all patients in the ITT Population who received at least 1 treatment dose.	
Subject analysis set title	Safety Population Ferrous Sulfate
Subject analysis set type	Safety analysis
Subject analysis set description: The Safety Population is defined as all randomized/assigned patients who received at least 1 dose of study drug.	
Subject analysis set title	Safety Population Ferric Maltol
Subject analysis set type	Safety analysis
Subject analysis set description: The Safety Population is defined as all randomized/assigned patients who received at least 1 dose of study drug.	
Subject analysis set title	Safety Population Ferric Maltol Assigned
Subject analysis set type	Safety analysis
Subject analysis set description: The Safety Population is defined as all randomized/assigned patients who received at least 1 dose of study drug.	
Subject analysis set title	PK Population Ferric Maltol 15mg (2-9 y) Day 1
Subject analysis set type	Full analysis

Subject analysis set description:

The PK Population is defined as all randomized/assigned patients who had at least 1 dose of study drug and who had at least 1 evaluable post-dose PK sample (applicable only for the Ferric Maltol group). A total of 20 patients in the ferric maltol group were included in the PK Population. Of these patients, there were 7 patients (3 female patients and 4 male patients) who were 2 to 9 years of age, 3 patients (all female patients) who were 10 to 17 years of age and who received 15 mg BID of ferric maltol, and 10 patients (9 female patients and 1 male patient) who were 10 to 17 years of age and who received 30 mg BID of ferric maltol.

Subject analysis set title	PK Population Ferric Maltol 15mg (10-17 y) Day 1
Subject analysis set type	Full analysis

Subject analysis set description:

The PK Population is defined as all randomized/assigned patients who had at least 1 dose of study drug and who had at least 1 evaluable post-dose PK sample (applicable only for the Ferric Maltol group). A total of 20 patients in the ferric maltol group were included in the PK Population. Of these patients, there were 7 patients (3 female patients and 4 male patients) who were 2 to 9 years of age, 3 patients (all female patients) who were 10 to 17 years of age and who received 15 mg BID of ferric maltol, and 10 patients (9 female patients and 1 male patient) who were 10 to 17 years of age and who received 30 mg BID of ferric maltol.

Subject analysis set title	PK Population Ferric Maltol 30mg (10-17 y) Day 1
Subject analysis set type	Full analysis

Subject analysis set description:

The PK Population is defined as all randomized/assigned patients who had at least 1 dose of study drug and who had at least 1 evaluable post-dose PK sample (applicable only for the Ferric Maltol group). A total of 20 patients in the ferric maltol group were included in the PK Population. Of these patients, there were 7 patients (3 female patients and 4 male patients) who were 2 to 9 years of age, 3 patients (all female patients) who were 10 to 17 years of age and who received 15 mg BID of ferric maltol, and 10 patients (9 female patients and 1 male patient) who were 10 to 17 years of age and who received 30 mg BID of ferric maltol.

Subject analysis set title	PK Population Ferric Maltol 15mg (2-9 y) Day 7-10
Subject analysis set type	Full analysis

Subject analysis set description:

The PK Population is defined as all randomized/assigned patients who had at least 1 dose of study drug and who had at least 1 evaluable post-dose PK sample (applicable only for the Ferric Maltol group). A total of 20 patients in the ferric maltol group were included in the PK Population. Of these patients, there were 7 patients (3 female patients and 4 male patients) who were 2 to 9 years of age, 3 patients (all female patients) who were 10 to 17 years of age and who received 15 mg BID of ferric maltol, and 10 patients (9 female patients and 1 male patient) who were 10 to 17 years of age and who received 30 mg BID of ferric maltol.

Subject analysis set title	PK Population Ferric Maltol 15mg (10-17 y) Day 7-10
Subject analysis set type	Full analysis

Subject analysis set description:

The PK Population is defined as all randomized/assigned patients who had at least 1 dose of study drug and who had at least 1 evaluable post-dose PK sample (applicable only for the Ferric Maltol group). A total of 20 patients in the ferric maltol group were included in the PK Population. Of these patients, there were 7 patients (3 female patients and 4 male patients) who were 2 to 9 years of age, 3 patients (all female patients) who were 10 to 17 years of age and who received 15 mg BID of ferric maltol, and 10 patients (9 female patients and 1 male patient) who were 10 to 17 years of age and who received 30 mg BID of ferric maltol.

Subject analysis set title	PK Population Ferric Maltol 30mg (10-17 y) Day 7-10
Subject analysis set type	Full analysis

Subject analysis set description:

The PK Population is defined as all randomized/assigned patients who had at least 1 dose of study drug and who had at least 1 evaluable post-dose PK sample (applicable only for the Ferric Maltol group). A total of 20 patients in the ferric maltol group were included in the PK Population. Of these patients, there were 7 patients (3 female patients and 4 male patients) who were 2 to 9 years of age, 3 patients (all female patients) who were 10 to 17 years of age and who received 15 mg BID of ferric maltol, and 10 patients (9 female patients and 1 male patient) who were 10 to 17 years of age and who received 30 mg BID of ferric maltol.

Primary: Change in hemoglobin concentration

End point title	Change in hemoglobin concentration ^[1]
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End point description:

End point type	Primary
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End point timeframe:

From baseline to week 12

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: The change in Hb concentration from baseline to Week 12 is summarised based on the mITT Population for each treatment group using descriptive statistics summarised by mean, standard deviation, median, and range (minimum and maximum).

End point values	mITT Ferrous Sulfate	mITT Ferric Maltol	mITT Ferric Maltol Assigned	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed				
Units: g/L				
arithmetic mean (standard deviation)	11.5 (± 13.97)	12.5 (± 13.89)	17.7 (± 13.61)	

Statistical analyses

No statistical analyses for this end point

Secondary: Changes in ferritin concentration

End point title	Changes in ferritin concentration
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End point description:

End point type	Secondary
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End point timeframe:

From baseline to week 12

End point values	mITT Ferrous Sulfate	mITT Ferric Maltol	mITT Ferric Maltol Assigned	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	28	31	3	
Units: µg/L				
arithmetic mean (standard deviation)	20.6 (± 30.97)	8.1 (± 11.06)	6.3 (± 8.74)	

Statistical analyses

No statistical analyses for this end point

Secondary: Change in Iron concentration

End point title	Change in Iron concentration
End point description:	
End point type	Secondary
End point timeframe:	
From baseline to week 12.	

End point values	mITT Ferrous Sulfate	mITT Ferric Maltol	mITT Ferric Maltol Assigned	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	28	31	3	
Units: µmol/L				
arithmetic mean (standard deviation)	3.64 (± 8.911)	5.77 (± 8.518)	1.07 (± 0.577)	

Statistical analyses

No statistical analyses for this end point

Secondary: Change in transferrin saturation

End point title	Change in transferrin saturation
End point description:	
End point type	Secondary
End point timeframe:	
From baseline to week 12.	

End point values	mITT Ferrous Sulfate	mITT Ferric Maltol	mITT Ferric Maltol Assigned	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	28	31	2	
Units: %				
arithmetic mean (standard deviation)	6.5 (± 12.39)	7.7 (± 10.14)	2.0 (± 0.00)	

Statistical analyses

No statistical analyses for this end point

Secondary: Cmax for plasma maltol glucuronide

End point title	Cmax for plasma maltol glucuronide
End point description:	
After a single dose of ferric maltol oral suspension on Visit 2 (PK Day 1) and after twice daily administrations for at least 6 days, on Visit 3 (PK Day 2) after a single morning dose.	

End point type	Secondary
End point timeframe:	
PK parameters assessed at Day 1 (Visit 2) and Day 7-10 (Visit 3)	

End point values	PK Population Ferric Maltol 15mg (2-9 y) Day 1	PK Population Ferric Maltol 15mg (10-17 y) Day 1	PK Population Ferric Maltol 30mg (10-17 y) Day 1	PK Population Ferric Maltol 15mg (2-9 y) Day 7-10
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	3	10	7
Units: ng/mL				
number (not applicable)	4350	6810	6420	4250

End point values	PK Population Ferric Maltol 15mg (10-17 y) Day 7-10	PK Population Ferric Maltol 30mg (10-17 y) Day 7-10		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	3	10		
Units: ng/mL				
number (not applicable)	5940	8160		

Statistical analyses

No statistical analyses for this end point

Secondary: Tmax for plasma maltol glucuronide

End point title	Tmax for plasma maltol glucuronide
End point description:	
After a single dose of ferric maltol oral suspension on Visit 2 (PK Day 1) and after twice daily administrations for at least 6 days, on Visit 3 (PK Day 2) after a single morning dose.	
End point type	Secondary
End point timeframe:	
PK parameters assessed at Day 1 (Visit 2) and Day 7-10 (Visit 3)	

End point values	PK Population Ferric Maltol 15mg (2-9 y) Day 1	PK Population Ferric Maltol 15mg (10-17 y) Day 1	PK Population Ferric Maltol 30mg (10-17 y) Day 1	PK Population Ferric Maltol 15mg (2-9 y) Day 7-10
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	3	10	7
Units: h				
number (not applicable)	1.00	2.13	0.52	0.98

End point values	PK Population Ferric Maltol 15mg (10-17 y) Day 7-10	PK Population Ferric Maltol 30mg (10-17 y) Day 7-10		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	3	10		
Units: h				
number (not applicable)	2.78	3.63		

Statistical analyses

No statistical analyses for this end point

Secondary: AUC0-t for plasma maltol glucuronide

End point title	AUC0-t for plasma maltol glucuronide
End point description:	
After a single dose of ferric maltol oral suspension on Visit 2 (PK Day 1) and after twice daily administrations for at least 6 days, on Visit 3 (PK Day 2) after a single morning dose.	
End point type	Secondary
End point timeframe:	
PK parameters assessed at Day 1 (Visit 2) and Day 7-10 (Visit 3)	

End point values	PK Population Ferric Maltol 15mg (2-9 y) Day 1	PK Population Ferric Maltol 15mg (10-17 y) Day 1	PK Population Ferric Maltol 30mg (10-17 y) Day 1	PK Population Ferric Maltol 15mg (2-9 y) Day 7-10
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	3	10	7
Units: hxng/mL				
number (not applicable)	4670	12000	14600	5900

End point values	PK Population Ferric Maltol 15mg (10-17 y) Day 7-10	PK Population Ferric Maltol 30mg (10-17 y) Day 7-10		
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Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	3	10		
Units: hxng/mL				
number (not applicable)	8070	16700		

Statistical analyses

No statistical analyses for this end point

Secondary: Cmax for baseline corrected serum iron

End point title	Cmax for baseline corrected serum iron
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End point description:

After a single dose of ferric maltol oral suspension on Visit 2 (PK Day 1) and after twice daily administrations for at least 6 days, on Visit 3 (PK Day 2) after a single morning dose.

End point type	Secondary
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End point timeframe:

PK parameters assessed at Day 1 (Visit 2) and Day 7-10 (Visit 3)

End point values	PK Population Ferric Maltol 15mg (2-9 y) Day 1	PK Population Ferric Maltol 15mg (10-17 y) Day 1	PK Population Ferric Maltol 30mg (10-17 y) Day 1	PK Population Ferric Maltol 15mg (2-9 y) Day 7-10
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	3	10	7
Units: µg/dL				
number (not applicable)	87.0	40.0	119	96.5

End point values	PK Population Ferric Maltol 15mg (10-17 y) Day 7-10	PK Population Ferric Maltol 30mg (10-17 y) Day 7-10		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	3	10		
Units: µg/dL				
number (not applicable)	21.0	364		

Statistical analyses

No statistical analyses for this end point

Secondary: Tmax for baseline corrected serum iron

End point title	Tmax for baseline corrected serum iron
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End point description:

After a single dose of ferric maltol oral suspension on Visit 2 (PK Day 1) and after twice daily administrations for at least 6 days, on Visit 3 (PK Day 2) after a single morning dose.

End point type	Secondary
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End point timeframe:

PK parameters assessed at Day 1 (Visit 2) and Day 7-10 (Visit 3)

End point values	PK Population Ferric Maltol 15mg (2-9 y) Day 1	PK Population Ferric Maltol 15mg (10-17 y) Day 1	PK Population Ferric Maltol 30mg (10-17 y) Day 1	PK Population Ferric Maltol 15mg (2-9 y) Day 7-10
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	3	10	7
Units: h				
number (not applicable)	2.37	2.13	2.00	2.10

End point values	PK Population Ferric Maltol 15mg (10-17 y) Day 7-10	PK Population Ferric Maltol 30mg (10-17 y) Day 7-10		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	3	10		
Units: h				
number (not applicable)	2.78	6.63		

Statistical analyses

No statistical analyses for this end point

Secondary: AUC0-t for baseline corrected serum iron

End point title	AUC0-t for baseline corrected serum iron
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End point description:

After a single dose of ferric maltol oral suspension on Visit 2 (PK Day 1) and after twice daily administrations for at least 6 days, on Visit 3 (PK Day 2) after a single morning dose.

End point type	Secondary
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End point timeframe:

PK parameters assessed at Day 1 (Visit 2) and Day 7-10 (Visit 3)

End point values	PK Population Ferric Maltol 15mg (2-9 y) Day 1	PK Population Ferric Maltol 15mg (10-17 y) Day 1	PK Population Ferric Maltol 30mg (10-17 y) Day 1	PK Population Ferric Maltol 15mg (2-9 y) Day 7-10
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	7	3	10	7
Units: hxµg/dL				
number (not applicable)	64.4	60.1	374	100

End point values	PK Population Ferric Maltol 15mg (10-17 y) Day 7-10	PK Population Ferric Maltol 30mg (10-17 y) Day 7-10		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	3	10		
Units: hxµg/dL				
number (not applicable)	1.93	1180		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

During the 12-week treatment period and within 2 weeks after receiving the last dose of the study drug

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	24.0
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Reporting groups

Reporting group title	Ferrous Sulfate Randomized
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Reporting group description:

Patients aged 2 to 17 years were randomized 1:1 to receive ferric maltol oral suspension or ferrous sulfate oral liquid.

Reporting group title	Ferric Maltol Randomized
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Reporting group description:

Subjects aged 2-17 will be randomised 1:1 to receive ferric maltol oral suspension or ferrous sulfate oral liquid.

The first 12 subjects randomised to ferric maltol in each age sub-group (2 - 9 yrs, 10 - 17 yrs respectively) will enter a PK phase with 2 PK days.

Following PK Day 2 subjects will continue until Week 12. Once the 18 subjects in each age subgroup have finished their PK visits, they will continue until week 12.

Ferrous sulfate 125 mg/ml (25 mg elemental iron) or equivalent dose was used for all children/adolescents. To maximise the iron replenishment for subjects within this group as well; aged 2 - 17 yrs were dosed 6 mg/kg to the maximum of 4 ml BID.

Reporting group title	Ferric Maltol Assigned
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Reporting group description:

12 weeks open-label Treatment Period for ferric maltol children aged 1 month to <2 years

Serious adverse events	Ferrous Sulfate Randomized	Ferric Maltol Randomized	Ferric Maltol Assigned
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 30 (0.00%)	0 / 31 (0.00%)	1 / 3 (33.33%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Respiratory, thoracic and mediastinal disorders			
Wheezing	Additional description: Occurrences are provided for Treatment Emergent Adverse Events (TEAEs). TEAEs were defined as AEs that started after the first dose of study drug.		
subjects affected / exposed	0 / 30 (0.00%)	0 / 31 (0.00%)	1 / 3 (33.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

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

Non-serious adverse events	Ferrous Sulfate Randomized	Ferric Maltol Randomized	Ferric Maltol Assigned
Total subjects affected by non-serious adverse events subjects affected / exposed	3 / 30 (10.00%)	4 / 31 (12.90%)	2 / 3 (66.67%)
Blood and lymphatic system disorders Iron deficiency anaemia subjects affected / exposed occurrences (all)	0 / 30 (0.00%) 0	0 / 31 (0.00%) 0	1 / 3 (33.33%) 1
General disorders and administration site conditions Pyrexia	Additional description: Occurrences are provided for Treatment Emergent Adverse Events (TEAEs). TEAEs were defined as AEs that started after the first dose of study drug.		
subjects affected / exposed occurrences (all)	0 / 30 (0.00%) 0	2 / 31 (6.45%) 2	0 / 3 (0.00%) 0
Gastrointestinal disorders Diarrhoea	Additional description: Occurrences are provided for Treatment Emergent Adverse Events (TEAEs). TEAEs were defined as AEs that started after the first dose of study drug.		
subjects affected / exposed occurrences (all)	0 / 30 (0.00%) 0	1 / 31 (3.23%) 1	1 / 3 (33.33%) 1
Teething	Additional description: Occurrences are provided for Treatment Emergent Adverse Events (TEAEs). TEAEs were defined as AEs that started after the first dose of study drug.		
subjects affected / exposed occurrences (all)	0 / 30 (0.00%) 0	0 / 31 (0.00%) 0	1 / 3 (33.33%) 1
Nausea	Additional description: Occurrences are provided for Treatment Emergent Adverse Events (TEAEs). TEAEs were defined as AEs that started after the first dose of study drug.		
subjects affected / exposed occurrences (all)	3 / 30 (10.00%) 3	2 / 31 (6.45%) 3	0 / 3 (0.00%) 0
Respiratory, thoracic and mediastinal disorders Cough	Additional description: Occurrences are provided for Treatment Emergent Adverse Events (TEAEs). TEAEs were defined as AEs that started after the first dose of study drug.		
subjects affected / exposed occurrences (all)	0 / 30 (0.00%) 0	1 / 31 (3.23%) 1	1 / 3 (33.33%) 1
Infections and infestations Conjunctivitis	Additional description: Occurrences are provided for Treatment Emergent Adverse Events (TEAEs). TEAEs were defined as AEs that started after the first dose of study drug.		
subjects affected / exposed occurrences (all)	0 / 30 (0.00%) 0	0 / 31 (0.00%) 0	1 / 3 (33.33%) 1

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
18 May 2021	• Study design updated with addition of urine sample in the pre-assignment PK phase and assigned treatment phase. • Update of visit window for Visit 3.
03 November 2022	• Changes to PK sampling, blood volume and study design. • Changes to exclusion/discontinuation criteria and permitted medications. • Changes to the secondary endpoints and secondary endpoint analysis.
14 March 2023	• Changes to study design • Changes to the primary endpoints/primary objectives
06 July 2023	• Removal of ferrous sulfate sample size reduction • Removal of late timepoints in PK sampling for younger cohort (2-9 years) to match sampling for older age group (10-17 years) • Addition of potential interim analysis and associated stopping rules • Removal of blood draws during preassignment PK in infants • Inclusion of quantitative efficacy measure

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported