



Clinical trial results:

A 48-week, Open-label, 2-arm, Parallel-group, Randomized Exploratory Study to Assess Liver Iron Concentration Measured by FerriScan® (R2) Magnetic Resonance Imaging in Beta-thalassemia Subjects Administered SPD602 (SSP-004184AQ) or Exjade® (deferasirox) for Treatment of Chronic Transfusional Iron Overload

Summary

EudraCT number	2013-000743-33
Trial protocol	BE GB GR IT
Global end of trial date	15 July 2014

Results information

Result version number	v1 (current)
This version publication date	05 April 2019
First version publication date	05 April 2019

Trial information

Trial identification

Sponsor protocol code	SPD602-204
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01927913
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Shire
Sponsor organisation address	300 Shire Way, Lexington, United States, MA 02421
Public contact	Study Director, Shire, ClinicalTransparency@shire.com
Scientific contact	Study Director, Shire, ClinicalTransparency@shire.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	15 July 2014
Is this the analysis of the primary completion data?	No
Global end of trial reached?	Yes
Global end of trial date	15 July 2014
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The main objective of this trial was to assess change in liver iron concentration (LIC) from baseline utilizing FerriScan® (R2) magnetic resonance imaging (MRI) in subjects with transfusional iron overload receiving SPD602 (SSP-004184AQ) or deferasirox whose primary diagnosis is beta-thalassemia.

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '999999' was entered in Population of Trial Subjects section since '0' could not be entered due to EudraCT system constraints.

Protection of trial subjects:

This study was conducted in accordance with International Conference on Harmonization (ICH) Good Clinical Practice, the principles of the Declaration of Helsinki, as well as other applicable local ethical and legal requirements.

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '999999' was entered in Population of Trial Subjects section since '0' could not be entered due to EudraCT system constraints.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	15 July 2014
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	Belgium: 999999
Worldwide total number of subjects	999999
EEA total number of subjects	999999

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)	999999
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	SPD602

Arm description:

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered since '0' could not be entered due to EudraCT system constraints.

Arm type	Experimental
Investigational medicinal product name	SPD602
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule
Routes of administration	Oral use

Dosage and administration details:

16-75 mg/kg/day

Arm title	Deferasirox
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Arm description:

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered since '0' could not be entered due to EudraCT system constraints.

Arm type	Active comparator
Investigational medicinal product name	Deferasirox
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Per approved country specific label

Number of subjects in period 1^[1]	SPD602	Deferasirox
Started	99999	99999
Completed	0	0
Not completed	99999	99999
Study was withdrawn with 0 participants	99999	99999

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: Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered for Number of Subjects for each arm since '0' could not be entered due to EudraCT system constraints.

Baseline characteristics

Reporting groups

Reporting group title	SPD602
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Reporting group description:

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered since '0' could not be entered due to EudraCT system constraints.

Reporting group title	Deferasirox
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Reporting group description:

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered since '0' could not be entered due to EudraCT system constraints.

Reporting group values	SPD602	Deferasirox	Total
Number of subjects	99999	99999	199998
Age categorical			
Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered since '0' could not be entered due to EudraCT system constraints.			
Units: Subjects			
Age Categorical	99999	99999	199998
Gender categorical			
Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered since '0' could not be entered due to EudraCT system constraints.			
Units:			
All	99999	99999	199998

End points

End points reporting groups

Reporting group title	SPD602
Reporting group description: Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered since '0' could not be entered due to EudraCT system constraints.	
Reporting group title	Deferasirox
Reporting group description: Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started. '99999' was entered since '0' could not be entered due to EudraCT system constraints.	

Primary: Change from baseline in Liver Iron Concentration (LIC) as assessed by FerriScan® R2 at Weeks 12, 24, and 48.

End point title	Change from baseline in Liver Iron Concentration (LIC) as assessed by FerriScan® R2 at Weeks 12, 24, and 48. ^[1]
End point description: Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started.	
End point type	Primary
End point timeframe: Baseline to Weeks 12, 24, and 48.	

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: Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started.

End point values	SPD602	Deferasirox		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	0 ^[2]	0 ^[3]		
Units: mg iron per g				
arithmetic mean (standard deviation)	()	()		

Notes:

[2] - Study was withdrawn with 0 participants.

[3] - Study was withdrawn with 0 participants.

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information^[1]

Timeframe for reporting adverse events:

All AEs are collected from the time the informed consent is signed until the defined follow-up period.

Adverse event reporting additional description:

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started.

Assessment type	Non-systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	NA
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Frequency threshold for reporting non-serious adverse events: 5 %

Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.

Justification: Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started.

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
03 October 2013	Clarified that total daily dose range will be 16-75 mg/kg/day. Modified starting doses for this study from 32, 50, and 75 mg/kg/day to 16 and 20mg/kg BID. Clarified that total daily doses are divided into 2 daily doses. Removed baseline LIC criterion for determination of starting dose; starting dose is determined by TII. Dose adjustments will be made based on LIC, CIC and/or serum ferritin results. Updated EQ-5D-3L to EQ-5D-5L. Updated information on current treatment options to be consistent with newly available data; updated rationale for SPD602 iron overload indication. Updated Inclusion Criteria 4 & 6 and Exclusion Criteria 5, 6 & 9. Clarified that subjects will be stratified by baseline LIC value and historical transfusion requirement after 2-week washout. Updated Rationale for Twice Daily Dosing text. Updated Rationale for the Dosing Algorithm text. Updated acceptable forms of birth control for women of child-bearing potential. Updated in vitro data for drug-drug interaction potential. Revised information regarding suspension or modification of dosing. Added table showing dose titration increments and BID to total daily dose equivalency. Modified dose increments as well as up and down titrations. Indicated that the investigator will be provided with local prescribing information for deferasirox. Updated safety measurements to include serum creatinine and eGFR. Updated renal function assessments to include eGFR. Added laboratory tests related to sensory disturbances as part of the biochemistry panel at select time points and in the event of a neurological adverse event. Specified that Vitamin D is to be assessed as part of biochemistry panel in the event of neurological adverse event requiring follow-up. Added LPI to biochemistry panel. Added NTBI and LPI to the clinical pharmacology assessments discussion. Specified blood sample details for neurology-specific biochemistry testing and updated estimated total mL volume of blood drawn.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

Study was withdrawn with 0 participants. Sponsor followed EMA's advice and contacted NCAs to enter comment that no participants were recruited. There is still no EudraCT functionality to inform the public that recruitment never started.

Notes: