Clinical trial results:
A Follow Up Investigation For Patients Completing Study A1481276 To Investigate Developmental Progress 12 And 24 Months Following Completion Of Sildenafil Treatment

Summary

EudraCT number	2010-021266-30
Trial protocol	GB
Global end of trial date	19 January 2014

Results information

Result version number	v1 (current)
This version publication date	05 January 2017
First version publication date	05 January 2017
Summary attachment (see zip file)	A1481283_Public Disclosure Synopsis (A1481283 Public Disclosure Synopsis.pdf)

Trial information

Trial identification

Sponsor protocol code	A1481283

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01801982
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Pfizer, Inc.
Sponsor organisation address	235 E 42nd Street, New York, United States, NY 10017
Public contact	Pfizer ClinicalTrials.gov Call Center, Pfizer, Inc., 001 800--718--1021, ClinicalTrials.gov_Inquiries@pfizer.com
Scientific contact	Pfizer ClinicalTrials.gov Call Center, Pfizer, Inc., 001 800--718--1021, ClinicalTrials.gov_Inquiries@pfizer.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?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	12 May 2014
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	19 January 2014
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To monitor the developmental progress of persistent pulmonary hypertension of the newborn (PPHN) subjects treated with sildenafil in study A1481276 (NCT01069861), at 12 and 24 months after completion of sildenafil treatment.

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 Council for Harmonization (ICH) Good Clinical Practice (GCP) Guidelines. All the local regulatory requirements pertinent to safety of trials subjects were followed.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	26 November 2011
Long term follow-up planned	Yes
Long term follow-up rationale	Safety
Long term follow-up duration	24 Months
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United Kingdom: 1
Worldwide total number of subjects	1
EEA total number of subjects	1

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)	1
Children (2-11 years)	0
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0

85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

This study was conducted at a single site in United Kingdom. Study started on 26 Nov 2011 and completed on 19 Jan 2014.

Period 1

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

Arms

Arm title	Sildenafil

Arm description:

Subjects who received intravenous sildenafil treatment in study A1481276 (NCT01069861) were followed-up for safety, up to Month 24.

Arm type	Follow up
Investigational medicinal product name	Sildenafil
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use

Dosage and administration details:

Subjects received intravenous sildenafil treatment in study A1481276 (NCT01069861).

Number of subjects in period 1	Sildenafil
Started	1
Completed	0
Not completed	1
Lost to follow-up	1

Baseline characteristics

Reporting groups	
Reporting group title	Over All
Reporting group description:	
Subjects who received intravenous sildenafil treatment in study A1481276 (NCT01069861) were followed-up for safety, up to Month 24.	

Reporting group values	Over All	Total	
Number of subjects	1	1	
Age Categorical Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	1	1	
Children (2-11 years)	0	0	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	0	0	
From 65-84 years	0	0	
85 years and over	0	0	
Age Continuous			
Here, 99999 signifies standard deviation was not estimable as only 1 subject was evaluated.			
Units: years			
arithmetic mean	1.2		
standard deviation	± 99999	-	
Gender Categorical Units: Subjects			
Female	1	1	
Male	0	0	

End points

End points reporting groups

Reporting group title	Sildenafil

Reporting group description:

Subjects who received intravenous sildenafil treatment in study A1481276 (NCT01069861) were followed-up for safety, up to Month 24.

Primary: Number of Subjects With Physical Examination Abnormalities at Month 12

End point title	Number of Subjects With Physical Examination Abnormalities at Month 12[1]

End point description:

Physical examinations included height, weight, head circumference, general appearance, skin examination, abdominal examination, respiratory system, neurological examination, hearing and ophthalmology assessments. Physical examination abnormalities were based on investigator discretion. Full analysis set (FAS) included all enrolled subjects.

End point type	Primary

End point timeframe:

Month 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: Only descriptive data was planned to be analyzed for this endpoint.

End point values	Sildenafil			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: subjects				
number (not applicable)	0			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Subjects With Physical Examination Abnormalities at Month 24

End point title	Number of Subjects With Physical Examination Abnormalities at Month 24[2]

End point description:

Physical examinations included height, weight, head circumference, general appearance, skin examination, abdominal examination, respiratory system, neurological examination, hearing and ophthalmology assessments. Physical examination abnormalities were based on investigator discretion.

End point type	Primary

End point timeframe:

Month 24

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: Only descriptive data was planned to be analyzed for this endpoint.

End point values	Sildenafil			
Subject group type	Reporting group			
Number of subjects analysed	0[3]			
Units: subjects				
number (not applicable)				

Notes:

[3] - Data was not possible to report as subject lost to follow-up at Month 24 after Month 12 follow-up.

Statistical analyses

No statistical analyses for this end point

Primary: Number of Subjects With Clinically Significant Medical History at Month 12

End point title	Number of Subjects With Clinically Significant Medical History at Month 12[4]

End point description:

Criteria for clinically significant medical history included any hospital admissions or any medications given since discharge from Study A1481276 (NCT01069861) that were considered clinically significant by the investigator. FAS included all enrolled subjects.

End point type	Primary

End point timeframe:

Month 12

Notes:

[4] - 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: Only descriptive data was planned to be analyzed for this endpoint.

End point values	Sildenafil			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: subjects				
number (not applicable)	0			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Subjects With Clinically Significant Medical History at Month 24

End point title	Number of Subjects With Clinically Significant Medical History at Month 24[5]

End point description:

Criteria for clinically significant medical history included any hospital admissions or any medications given since discharge from Study A1481276 (NCT01069861) that were considered clinically significant by the investigator.

End point type	Primary

End point timeframe:

Month 24

Notes:

[5] - 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: Only descriptive data was planned to be analyzed for this endpoint.

End point values	Sildenafil			
Subject group type	Reporting group			
Number of subjects analysed	0[6]			
Units: subjects				
number (not applicable)				

Notes:

[6] - Data was not possible to report as subject lost to follow-up at Month 24 after Month 12 follow-up.

Statistical analyses

No statistical analyses for this end point

Secondary: Overall Survival at Month 12

End point title	Overall Survival at Month 12

End point description:

Overall survival was the duration from enrollment to death. For subjects who are alive, overall survival was censored at the last contact. Number of subjects who were alive at Month 12 was to be reported. FAS included all enrolled subjects.

End point type	Secondary

End point timeframe:

Month 12

End point values	Sildenafil			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: subjects				
number (not applicable)	1			

Statistical analyses

No statistical analyses for this end point

Secondary: Overall Survival at Month 24

End point title	Overall Survival at Month 24

End point description:

Overall survival was the duration from enrollment to death. For subjects who are alive, overall survival was censored at the last contact. Number of subjects who were alive at Month 24 was to be reported.

End point type	Secondary

End point timeframe:

Month 24

End point values	Sildenafil			
Subject group type	Reporting group			
Number of subjects analysed	0[7]			
Units: subjects				
number (not applicable)				

Notes:

[7] - Data was not possible to report as subject lost to follow-up at Month 24 after Month 12 follow-up.

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Adverse Events (AE) and Serious Adverse Events (SAE)

End point title	Number of Subjects With Adverse Events (AE) and Serious Adverse Events (SAE)

End point description:

An AE was any untoward medical occurrence attributed to a subject who received study drug without regard to possibility of causal relationship. An SAE is an AE resulting in any of the following outcomes or deemed significant for any other reason: death; initial or prolonged in-patient hospitalization; life threatening experience (immediate risk of dying); persistent or significant disability/incapacity; congenital anomaly. FAS included all enrolled subjects.

End point type	Secondary

End point timeframe:

Day 1 up to Month 24

End point values	Sildenafil			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: subjects				
number (not applicable)	0			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]

Timeframe for reporting adverse events:

Day 1 up to Month 24

Assessment type	Non-systematic

Dictionary used

Dictionary name	NA
Dictionary version	0.0

Reporting groups

Reporting group title	Sildenafil

Reporting group description:

Subjects who received intravenous sildenafil treatment in study A1481276 (NCT01069861) were followed-up for safety, up to Month 24.

Serious adverse events	Sildenafil		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 1 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	Sildenafil		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 1 (0.00%)		

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: No non-serious adverse event occurred in this study.

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