Clinical trial results:
An Open-label, Multicenter Extension Study to Evaluate the Long-term Safety of LUM001, an Apical Sodium-dependent Bile Acid Transporter Inhibitor (ASBTi), in Patients with Alagille Syndrome (ALGS) or Progressive Familial Intrahepatic Cholestasis (PFIC)

Summary

EudraCT number	2015-000906-20
Trial protocol	GB
Global end of trial date	24 July 2015

Results information

Result version number	v1 (current)
This version publication date	27 November 2019
First version publication date	27 November 2019

Trial information

Trial identification

Sponsor protocol code	LUM001-601

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Mirum Pharmaceuticals, Inc. (Transferred from Shire to Mirum)
Sponsor organisation address	950 Tower Lane, Foster City, United States, CA 94404
Public contact	Chief Scientific Officer, Mirum Pharmaceuticals, Inc., medinfo@mirumpharma.com
Scientific contact	Chief Scientific Officer, Mirum Pharmaceuticals, Inc., medinfo@mirumpharma.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	24 July 2015
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	24 July 2015
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The main objective of this trial is to evaluate the long-term safety of LUM001 in patients with ALGS or PFIC.

Study was withdrawn with 0 participants. 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:

Study was withdrawn with 0 participants. 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	24 July 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	United Kingdom: 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)	999999
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:

Study was withdrawn with 0 participants. 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	Non-randomised - controlled
Blinding used	Not blinded

Arms

Arm title	LUM001

Arm description:

Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started. '999999' was entered since '0' could not be entered due to EudraCT system constraints.

Arm type	Experimental
Investigational medicinal product name	LUM001
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Oral solution
Routes of administration	Oral use

Dosage and administration details:

Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started. '999999' was entered since '0' could not be entered due to EudraCT system constraints.

Number of subjects in period 1	LUM001
Started	999999
Completed	0
Not completed	999999
Study was withdrawn with 0 participants	999999

Baseline characteristics

Reporting groups	
Reporting group title	LUM001
Reporting group description:	
Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started. '999999' was entered since '0' could not be entered due to EudraCT system constraints.	

Reporting group values	LUM001	Total	
Number of subjects	999999	999999	
Age categorical			
Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started. '999999' was entered since '0' could not be entered due to EudraCT system constraints.			
Units: Subjects			
Age Categorical	999999	999999	
Gender categorical			
Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started. '999999' was entered since '0' could not be entered due to EudraCT system constraints.			
Units: Subjects			
All	999999	999999	

End points

End points reporting groups

Reporting group title	LUM001

Reporting group description:

Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started. '999999' was entered since '0' could not be entered due to EudraCT system constraints.

Primary: Number of Participants with Treatment-Emergent Adverse Events (TEAEs)

End point title	Number of Participants with Treatment-Emergent Adverse Events (TEAEs)[1]

End point description:

Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started.

End point type	Primary

End point timeframe:

Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started.

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. There is still no EudraCT functionality to inform the public that recruitment never started.

End point values	LUM001			
Subject group type	Reporting group			
Number of subjects analysed	0[2]			
Units: Number of participants				

Notes:

[2] - 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:	
Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started.	
Adverse event reporting additional description:	
Study was withdrawn with 0 participants. There is still no EudraCT functionality to inform the public that recruitment never started.	
Assessment type	Non-systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	NA

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. 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? No

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. There is still no EudraCT functionality to inform the public that recruitment never started.

Notes: