Clinical trial results:
A Phase 2 open-label, dose-escalation study to evaluate the safety, pharmacokinetics, immunogenitiicy and pharmacodynamics/efficacy of EDI200, an EDA-A1 replacement protein, administered to male infants with X-Linked Hypohidrotic Ectodermal Dysplasia (XLHED).

Summary

EudraCT number	2012-003561-17
Trial protocol	DE GB IT FR
Global end of trial date	17 March 2016

Results information

Result version number	v1 (current)
This version publication date	27 August 2016
First version publication date	27 August 2016
Summary attachment (see zip file)	_Edimer ECP-002 Abbrv. CSR Final_31May2016_Signed wAtt (_Edimer ECP-002 Abbrv. CSR Final_31May2016_Signed wAtt.pdf)

Trial information

Trial identification

Sponsor protocol code	ECP-002

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01775462
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Edimer Pharmaceuticals, Inc.
Sponsor organisation address	55 Cambridge Parkway, Suite 102W, Cambridge, United States, MA 02142
Public contact	Clinical Trials Information, Edimer Pharmaceuticals, Inc., +1 6177584305, ramsey@edimerpharma.com
Scientific contact	Clinical Trials Information, Edimer Pharmaceuticals, Inc., +1 6177584305, ramsey@edimerpharma.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	28 September 2015
Is this the analysis of the primary completion data?	Yes
Primary completion date	28 September 2015
Global end of trial reached?	Yes
Global end of trial date	17 March 2016
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To assess the safety, pharmacokinetics and immunogenicity of EDI200 administered to XLHED-affected neonates

Protection of trial subjects:

Patients were always under close monitoring in the hospital by specific qualified and trained paediattic trial staff (Investigators and study nurses).
All possible steps were taken to prevent the patients from any stress, pain or discomfort.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	03 June 2013
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: 2
Country: Number of subjects enrolled	France: 1
Country: Number of subjects enrolled	Germany: 4
Country: Number of subjects enrolled	United States: 3
Worldwide total number of subjects	10
EEA total number of subjects	7

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	10
Infants and toddlers (28 days-23 months)	0
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:

Male neonates documented by genetic diagnosis to carry an EDA mutation associated with XLHED, and their male siblings

Period 1

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

Arms

Arm title	Treatment

Arm description: -

Arm type	Experimental
Investigational medicinal product name	EDI200
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

3/10/20 mg/kg (5 doses total) mg/kg milligram(s)/kilogram per day

Number of subjects in period 1	Treatment
Started	10
Cohort 1	3 [1]
Cohort 2	5 [2]
Cohort 3	2 [3]
Completed	10

Notes:

[1] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: 2 Subjects were enrolled in cohort 3 till study completion

[2] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: Dose was adjusted after 5 patients completed cohort 2

[3] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: Dose was adjusted after 3 patients completed cohort 1

Baseline characteristics

End points

End points reporting groups

Reporting group title	Treatment

Reporting group description: -

Primary: Clinical endpoints

End point title	Clinical endpoints[1]

End point description:

End point type	Primary

End point timeframe:

overall

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: Proof of concept study to be compared with a parallel natural history study

End point values	Treatment			
Subject group type	Reporting group			
Number of subjects analysed	10			
Units: growth and development assessments	10			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]	
Timeframe for reporting adverse events:	
16 Sep 2013 - 28 Sep 2015 Time from first patient in till last patient out visit	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	10.0

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

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: All serious advers events can be found in the Appendix 16.2.7 of the CSR

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
24 June 2013	Protocol Version 3 (see CSR 9.8 page 15))
04 December 2014	Protocol AMD 5 (See CSR 9.8 page 16)
11 February 2015	Protocol AMD 6 (See CSR 9.9 page 15)

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported