Clinical trial results:
Traitement des épidermolyses bulleuses simples de type Dowling Maera par l'érythromicine orale

Summary

EudraCT number	2010-024428-10
Trial protocol	FR
Global end of trial date	06 August 2014

Results information

Result version number	v1 (current)
This version publication date	29 June 2022
First version publication date	29 June 2022
Summary attachment (see zip file)	end study (courrier rsmé final.pdf) Publication (Chiaverini_et_al-2015-British_Journal_of_Dermatology.pdf)

Trial information

Trial identification

Sponsor protocol code	10-PP-19

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	chu de nice
Sponsor organisation address	DRCI-Hôpital de Cimiez - 4 avenue reine victoria, Nice, France, 06003
Public contact	Directeur de la Recherche clinique, CHU de nice - DRCI, +33 492034011, caillon.c@chu-nice.fr
Scientific contact	Investigateur Principal, CHU de Nice - DR Chiaverini, +33 492036488, chiaverini.c@chu-nice.fr

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	06 August 2014
Is this the analysis of the primary completion data?	Yes
Primary completion date	06 August 2014
Global end of trial reached?	Yes
Global end of trial date	06 August 2014
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The main objective is to estimate the efficiency of the oral érythromycine to decrease the number of cutaneous bubbles at the patients affected by EBS-DM after 3 months of treatment.

Protection of trial subjects:

Patients of both sexes, aged 1–8 years who had EBS-gen-sev with at least two new blisters per day were eligible for the study. The parents signed the consent for the clidrens'participation.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	29 June 2011
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	France: 5
Worldwide total number of subjects	5
EEA total number of subjects	5

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)	5
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:

At baseline, after 1 month and 3 months of treatment, the
patients were seen for body examination, questionnaire, photographs, blood tests and bacteriological swabs. Itch severity
and skin fragility were evaluated by parents on a visual analogue scale. At 5 months, we asked all parents for their opinion regarding the tolerability and efficacy

Period 1

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

Arms

Arm title	Oral erythromycin therapy

Arm description: -

Arm type	Experimental
Investigational medicinal product name	erythromycin
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Syrup
Routes of administration	Buccal use

Dosage and administration details:

. A weight-based dosage was calculated for the children (< 10 kg, 250 mg per day; 10–15 kg, 500 mg per day; 15–25 kg, 750 mg per day; 25–35 kg, 1000 mg per
day).

Number of subjects in period 1	Oral erythromycin therapy
Started	5
Completed	5

Baseline characteristics

End points

End points reporting groups

Reporting group title	Oral erythromycin therapy

Reporting group description: -

Primary: The efficacy of treatment evaluate by parents

End point title	The efficacy of treatment evaluate by parents[1]

End point description:

End point type	Primary

End point timeframe:

At baseline, after 1 month and 3 months of treatment

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 description is in the arcticle

End point values	Oral erythromycin therapy			
Subject group type	Reporting group			
Number of subjects analysed	5			
Units: NA	5			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]	
Timeframe for reporting adverse events:	
At baseline, after 1 month and 3 months of treatment	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	17

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: There is no adverse serious event

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
07 July 2011	Increase in upper age limit
07 July 2011	modification of the route of administration of the medicinal product

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported