Clinical trial results:
Clinical study to evaluate the efficacy and safety of Octagam® 10% in Idiopathic Thrombocytopenic Purpura in adults.

Summary

EudraCT number	2005-003552-35
Trial protocol	DE AT CZ FR
Global end of trial date	15 September 2008

Results information

Result version number	v1 (current)
This version publication date	03 December 2016
First version publication date	03 December 2016

Trial information

Trial identification

Sponsor protocol code	GAM10-02

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT00426270
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Octapharma AG
Sponsor organisation address	Seidenstrasse 2, Lachen, Switzerland, CH-8853
Public contact	Octapharma Pharmazeutika Prod.Ges.m.b.H., Octapharma Pharmazeutika Prod.Ges.m.b.H., 0043 1 610 32 0,
Scientific contact	Octapharma Pharmazeutika Prod.Ges.m.b.H., Octapharma Pharmazeutika Prod.Ges.m.b.H., 0043 1 610 32 0,

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	02 June 2009
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	15 September 2008
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The main objective of the study is to investigate the efficacy of Octagam® 10% in correcting the platelet count.

Protection of trial subjects:

This trial was conducted in accordance to the principles of GCP, ensuring that the rights, safety and wellbeing of patients are protected and in consistency with the Declaration of Helsinki. Inclusion and exclusion criteria were carefully defined in order to protect subjects from contraindications, interactions with other medication and safety factors associated with the investigational medicinal product. Throughout the study safety was assessed, such as occurrence of AEs, lab values, vital signs and physical examinations.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	13 June 2006
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	Austria: 1
Country: Number of subjects enrolled	Czech Republic: 27
Country: Number of subjects enrolled	France: 1
Country: Number of subjects enrolled	Germany: 18
Country: Number of subjects enrolled	Poland: 69
Worldwide total number of subjects	116
EEA total number of subjects	116

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)	1
Adults (18-64 years)	91
From 65 to 84 years	22
85 years and over	2

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

In total, 116 subjects were enrolled in the study in order to achieve 110 evaluable subjects with acute or chronic ITP.

Period 1

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

Arms

Arm title	Octagam 10%

Arm description:

Participants received Octagam 10% (human normal immunoglobulin) 1 g/kg intravenously once a day for 2 days

Arm type	Experimental
Investigational medicinal product name	Octagam 10%
Investigational medicinal product code	
Other name	Human Normal Immunoglobulin
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Participants received Octagam 10% (human normal immunoglobulin) 1 g/kg intravenously once a day for 2 days.

Number of subjects in period 1	Octagam 10%
Started	116
Completed	110
Not completed	6
Incorrectly Enrolled in the Study	1
Adverse Event	5

Baseline characteristics

Reporting groups	
Reporting group title	Overall Trial

Reporting group description: -

Reporting group values	Overall Trial	Total	
Number of subjects	116	116	
Age categorical Units: Subjects			
Age 17- 88	116	116	
Age continuous Units: years arithmetic mean standard deviation	 47.7 ± 19.1	 -	
Gender categorical Units: Subjects			
Female	74	74	
Male	42	42	

End points

End points reporting groups

Reporting group title	Octagam 10%

Reporting group description:

Participants received Octagam 10% (human normal immunoglobulin) 1 g/kg intravenously once a day for 2 days

Primary: Percentage of Participants with a Clinical Response

End point title	Percentage of Participants with a Clinical Response[1]

End point description:

A clinical response is defined as an increase in platelet count to ≥ 50 x 10^9/L on any day from Day 2 to Day 7.

End point type	Primary

End point timeframe:

Day 2 to Day 7

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 statistical analysis of the primary endpoint comprised the overall response rate in the full analysis set and the associated two-sided 95% confidence interval, and is given in the end point values table.

End point values	Octagam 10%			
Subject group type	Reporting group			
Number of subjects analysed	115[2]			
Units: Percentage of Participants				
number (confidence interval 95%)	80 (72.7 to 87.3)			

Notes:

[2] - One participant was incorrectly enrolled in the study and was not included in the efficacy analyses

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
The condition of the patients were monitored until day 21	
Adverse event reporting additional description:	
Safety population: All participants who received at least 1 dose of study medication.	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	9.0

Reporting groups	
Reporting group title	Safety Set
Reporting group description:	
All participants who received at least 1 dose of study medication	

Serious adverse events	Safety Set		
Total subjects affected by serious adverse events			
subjects affected / exposed	14 / 116 (12.07%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Investigations			
Platelet count decreased			
subjects affected / exposed	1 / 116 (0.86%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Extradural haemotoma			
subjects affected / exposed	1 / 116 (0.86%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Injury			
subjects affected / exposed	1 / 116 (0.86%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Overdose			

subjects affected / exposed	1 / 116 (0.86%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Headache			
subjects affected / exposed	2 / 116 (1.72%)		
occurrences causally related to treatment / all	1 / 2		
deaths causally related to treatment / all	0 / 0		
Transient ischaemic attack			
subjects affected / exposed	1 / 116 (0.86%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Blood and lymphatic system disorders			
Idiopathic thrombocytopenic purpura			
subjects affected / exposed	5 / 116 (4.31%)		
occurrences causally related to treatment / all	0 / 5		
deaths causally related to treatment / all	0 / 0		
Thrombocytopenia			
subjects affected / exposed	2 / 116 (1.72%)		
occurrences causally related to treatment / all	0 / 3		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Pneumonia			
subjects affected / exposed	1 / 116 (0.86%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	Safety Set		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	79 / 116 (68.10%)		
Investigations			
Heart rate decreased			

subjects affected / exposed	18 / 116 (15.52%)		
occurrences (all)	34		
Heart rate increased			
subjects affected / exposed	25 / 116 (21.55%)		
occurrences (all)	39		
Vascular disorders			
Hypertension			
subjects affected / exposed	7 / 116 (6.03%)		
occurrences (all)	12		
Nervous system disorders			
Headache			
subjects affected / exposed	35 / 116 (30.17%)		
occurrences (all)	49		
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	21 / 116 (18.10%)		
occurrences (all)	21		
Blood and lymphatic system disorders			
Idiopathic thrombocytopenic purpura			
subjects affected / exposed	6 / 116 (5.17%)		
occurrences (all)	6		
Gastrointestinal disorders			
Nausea			
subjects affected / exposed	7 / 116 (6.03%)		
occurrences (all)	7		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
08 May 2006	Amendment #2 1. If a subject was hospitalised before study drug treatment due to the underlying disease, or hospitalised for social reasons or for an elective procedure, this was not to be reported as an SAE. 2. If a study site was inactive and did not screen any subjects for the study, the monitor did not need to perform a trial termination visit.
21 August 2006	Amendment #3: 1. The descriptive interim analysis after recruitment of 30 subjects, described in the interim report dated 26 September 2007, was added to the protocol, in order to obtain marketing authorisation in Europe.
13 October 2006	Amendment #4: 1. The permitted and forbidden prior and concomitant medications were clarified. 2. Faster infusion rates were added (up to 0.12 mL/kg/min), to allow shorter infusion times for subjects who tolerated the Octagam 10% infusions well.
09 March 2007	Amendment #6: 1. Additional viral marker testing for HIV, HCV and HBV was added, to fulfil FDA requirements. 2. Deletion of possible sample size adaptation after interim analysis as requested by the FDA; total recruitment target remained at 110 subjects.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported