Clinical trial results:
DOUBLE-BLIND, RANDOMIZED, PLACEBO- CONTROLLED PHASE III STUDY EVALUATING EFFICACY AND SAFETY OF SUBCUTANEOUS HUMAN IMMUNOGLOBULIN (OCTANORM) IN PATIENTS WITH DERMATOMYOSITIS.

Summary

EudraCT number	2017-002710-31
Trial protocol	DE CZ HU RO
Global end of trial date	29 November 2018

Results information

Result version number	v1 (current)
This version publication date	17 October 2019
First version publication date	17 October 2019

Trial information

Trial identification

Sponsor protocol code	SCGAM-02

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03686969
WHO universal trial number (UTN)	-
Other trial identifiers	IND: 17515

Notes:

Sponsors

Sponsor organisation name	Octapharma Pharmazeutika Produktionsges.m.b.H.
Sponsor organisation address	Oberlaaer Strasse 235, Vienna, Austria, 1100
Public contact	Global Clinical Project Manager, Octapharma Pharmazeutika Produktionsges.m.b.H. , 43 1610321202, clinical.department@octapharma.com
Scientific contact	Global Clinical Project Manager, Octapharma Pharmazeutika Produktionsges.m.b.H. , 43 1610321202, clinical.department@octapharma.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	16 March 2019
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	29 November 2018
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this study is to determine the efficacy of subcutaneous immunoglobulin octanorm in the maintenance treatment of DM patients who have previously responded to IGIV therapy.

Protection of trial subjects:

This trial was conducted in accordance to the principles of ICH- GCP, ensuring that the rights, safety and well-being 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 risk factors associated with the investigational medicinal product. Throughout the study safety was assessed, such as occurrence of AEs, local injection site reactions, safety labs, vital signs and physical examinations.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	02 August 2018
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	Russian Federation: 1
Worldwide total number of subjects	1
EEA total number of subjects	0

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)	0
Adults (18-64 years)	1
From 65 to 84 years	0

85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Patients with documented diagnosis of dermatomyositis who have previously responded to IGIV treatment were to be enrolled in the study on the basis of the in- and exclusion criteria defined in the study protocol.

Period 1

Period 1 title	Overall Trial (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator

Arms

Arm title	octanorm or placebo

Arm description:

Subjects were to be randomly assigned to either 0.5 g/kg/week octanorm or to placebo. A dose of 0.5 g/kg/week had to be administered subcutaneously every week (±2 days). A minimum interval of 4 days must have been kept in between two consecutive subcutaneous infusion cycles.

Arm type	Experimental
Investigational medicinal product name	octanorm
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

The only patient treated in the study was assigned to octanorm. Octanorm had to be administered subcutaneously every week (±2 days) using a syringe driver for precise infusion rates. The total dose/volume of a weekly infusion was calculated on the basis of the body weight (0.5 g/kg). The weekly infusion was performed in two separate sessions (= 1 infusion cycle for a given weekly administration). An infusion cycle comprises both sessions to be administered on 1 or 2 days, either on the same day or on two consecutive days or with maximum one day in between two sessions.

Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

A dose of 0.5 g/kg/week had to be administered subcutaneously every week (±2 days). A minimum interval of 4 days must have been kept in between two consecutive subcutaneous infusion cycles.

Number of subjects in period 1	octanorm or placebo
Started	1
Completed	0
Not completed	1
Premature termination of the study	1

Baseline characteristics

Reporting groups	
Reporting group title	Overall Trial

Reporting group description:

Only one patient was enrolled. The study treatment could not be completed for the one patient enrolled because of premature study termination.

Reporting group values	Overall Trial	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)	0	0	
Children (2-11 years)	0	0	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	1	1	
From 65-84 years	0	0	
85 years and over	0	0	
Gender categorical Units: Subjects			
Female	1	1	
Male	0	0	

End points

End points reporting groups

Reporting group title	octanorm or placebo

Reporting group description:

Subjects were to be randomly assigned to either 0.5 g/kg/week octanorm or to placebo. A dose of 0.5 g/kg/week had to be administered subcutaneously every week (±2 days). A minimum interval of 4 days must have been kept in between two consecutive subcutaneous infusion cycles.

Primary: Clinically important deterioration

End point title	Clinically important deterioration[1]

End point description:

Primary endpoint is defined as the proportion of patients with clinically important deterioration during the treatment period up to Week 32. A patient with clinically important deterioration is defined as a patient with 1) MMT 8 worsening ≥ 6 points (scale of 150) OR CDASI worsening ≥ 5 points, AND 2) a Physician's Global Disease Activity VAS worsening ≥ 2 cm.

End point type	Primary

End point timeframe:

week 32

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: In view of the limited data available (only from one patient), no efficacy and safety analyses were performed.

End point values	octanorm or placebo			
Subject group type	Reporting group			
Number of subjects analysed	0[2]			
Units: proportion				
number (not applicable)				

Notes:

[2] - Only 1 patient was enrolled. Because of premature termination of the study no analysis was possible.

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Throughout the whole study	
Assessment type	Systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	18.1
Reporting groups	
Reporting group title	octanorm or placebo
Reporting group description:	
Subjects were to be randomly assigned to either 0.5 g/kg/week octanorm or to placebo. A dose of 0.5 g/kg/week had to be administered subcutaneously every week (±2 days). A minimum interval of 4 days must have been kept in between two consecutive subcutaneous infusion cycles.	

Serious adverse events	octanorm or placebo		
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	octanorm or placebo		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	1 / 1 (100.00%)		
Investigations			
Blood lactate dehydrogenase increased			
subjects affected / exposed	1 / 1 (100.00%)		
occurrences (all)	1		
Blood creatine phosphokinase increased			
subjects affected / exposed	1 / 1 (100.00%)		
occurrences (all)	1		
General disorders and administration site conditions			
Infusion site haematoma			

subjects affected / exposed	1 / 1 (100.00%)		
occurrences (all)	1		
Dermatomyositis, Myalgia worsening			
subjects affected / exposed	1 / 1 (100.00%)		
occurrences (all)	1		
Skin and subcutaneous tissue disorders			
Dermatomyositis, Gottron´s sign worsening			
subjects affected / exposed	1 / 1 (100.00%)		
occurrences (all)	1		
Infections and infestations			
Aldolase increased			
subjects affected / exposed	1 / 1 (100.00%)		
occurrences (all)	1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
19 April 2018	Protocol version 06 - Contraception Language updated, - pregnancy testing frequency - Dose adjustment acc. body weight measurements - study prolongation by 2 months. - Site infusions possible until 7th cycle and afterwards if deemed medically relevant by the investigator.
16 July 2018	Protocol Version 07 - number of syringe drivers adapted - clarification of concomitant medication - exclusion criteron juvenile dermatomyositis has been added - SAE reporting details updated

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? Yes

Date	Interruption	Restart date
29 November 2018	The study was designed to include patients from the GAM10-08 study, a prospective, Double-blind, Randomized, Placebo-Controlled Phase III Study Evaluating Efficacy and Safety of Octagam 10% in Patients With Dermatomyositis ("ProDERM study") as roll-over patients and new patients meeting eligibility criteria. The first site was initiated on 11-Jul-2018 and until November 2018 only one patient had been randomized. Between July and November 2018 more than 10 potentially eligible roll over patients refused to participate in the study SCGAM-02. Based on the analysis of the reasons of the patients' refusals it was decided to terminate the study because of the higher than expected study complexity and slow enrolment.	-

Notes:

Limitations and caveats

None reported