Clinical trial results:
A MULTICENTER, OPEN-LABEL, MULTIPLE-DOSE STUDY TO EVALUATE THE SAFETY, TOLERABILITY, AND EFFICACY OF UCB7665 IN SUBJECTS WITH PRIMARY IMMUNE THROMBOCYTOPENIA

Summary

EudraCT number	2015-003984-12
Trial protocol	DE CZ ES BE PL LT BG IT
Global end of trial date	04 February 2019

Results information

Result version number	v1
This version publication date	19 February 2020
First version publication date	19 February 2020

Trial information

Trial identification

Sponsor protocol code	TP0001

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT02718716
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	UCB Biopharma SPRL
Sponsor organisation address	Allée de la Recherche 60, Brussels, Belgium, B-1070
Public contact	Clin Trial Reg & Results Disclosure, UCB BIOSCIENCES GmbH, clinicaltrials@ucb.com
Scientific contact	Clin Trial Reg & Results Disclosure, UCB BIOSCIENCES GmbH, clinicaltrials@ucb.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	05 March 2019
Is this the analysis of the primary completion data?	Yes
Primary completion date	04 February 2019
Global end of trial reached?	Yes
Global end of trial date	04 February 2019
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety and tolerability of UCB7665 administered by subcutaneous (sc) infusion in patients with immune thrombocytopenia (ITP)

Protection of trial subjects:

During the conduct of the study all participants were closely monitored.

Background therapy:

Background therapy as permitted in the protocol.

Evidence for comparator:

Not Applicable

Actual start date of recruitment	02 March 2016
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	Bulgaria: 1
Country: Number of subjects enrolled	Czech Republic: 2
Country: Number of subjects enrolled	Georgia: 6
Country: Number of subjects enrolled	Germany: 5
Country: Number of subjects enrolled	Italy: 4
Country: Number of subjects enrolled	Moldova, Republic of: 8
Country: Number of subjects enrolled	Poland: 27
Country: Number of subjects enrolled	Romania: 5
Country: Number of subjects enrolled	Spain: 5
Country: Number of subjects enrolled	United Kingdom: 3
Worldwide total number of subjects	66
EEA total number of subjects	52

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)	49
From 65 to 84 years	16
85 years and over	1

Subject disposition

Recruitment

Recruitment details:

The study started to enroll patients in March 2016 and concluded in February 2019.

Pre-assignment

Screening details:

The study included a Screening Period (1 to 28 days), a Dosing Period of 1 to 4 weeks, and an Observation Period of 8 weeks.
Participant Flow refers to the Safety Set.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	UCB7665 dose 1

Arm description:

Participants in this arm received 5 subcutaneous (sc) doses of UCB7665 dose 1 at 1-week intervals.

Arm type	Experimental
Investigational medicinal product name	UCB7665
Investigational medicinal product code	UCB7665
Other name	Rozanolixizumab
Pharmaceutical forms	Powder for solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

UCB7665 100 mg/mL was administered as a subcutaneous (sc) infusion using an infusion pump which was programmed at a constant flow rate.

Arm title	UCB7665 dose 2

Arm description:

Participants in this arm received 3 subcutaneous (sc) doses of UCB7665 dose 2 at 1-week intervals.

Arm type	Experimental
Investigational medicinal product name	UCB7665
Investigational medicinal product code	UCB7665
Other name	Rozanolixizumab
Pharmaceutical forms	Powder for solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

UCB7665 100 mg/mL was administered as a subcutaneous (sc) infusion using an infusion pump which was programmed at a constant flow rate.

Arm title	UCB7665 dose 3

Arm description:

Participants in this arm received 2 subcutaneous (sc) doses of UCB7665 dose 3 at 1-week intervals.

Arm type	Experimental

Investigational medicinal product name	UCB7665
Investigational medicinal product code	UCB7665
Other name	Rozanolixizumab
Pharmaceutical forms	Powder for solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

UCB7665 100 mg/mL was administered as a subcutaneous (sc) infusion using an infusion pump which was programmed at a constant flow rate.

Arm title	UCB7665 dose 4

Arm description:

Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 4.

Arm type	Experimental
Investigational medicinal product name	UCB7665
Investigational medicinal product code	UCB7665
Other name	Rozanolixizumab
Pharmaceutical forms	Powder for solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

UCB7665 100 mg/mL was administered as a subcutaneous (sc) infusion using an infusion pump which was programmed at a constant flow rate.

Arm title	UCB7665 dose 5

Arm description:

Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 5.

Arm type	Experimental
Investigational medicinal product name	UCB7665
Investigational medicinal product code	UCB7665
Other name	Rozanolixizumab
Pharmaceutical forms	Powder for solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

UCB7665 100 mg/mL was administered as a subcutaneous (sc) infusion using an infusion pump which was programmed at a constant flow rate.

Number of subjects in period 1	UCB7665 dose 1	UCB7665 dose 2	UCB7665 dose 3
Started	15	15	12
Completed	14	15	12
Not completed	1	0	0
Lack of efficacy	1	-	-

Number of subjects in period 1	UCB7665 dose 4	UCB7665 dose 5
Started	12	12
Completed	12	12
Not completed	0	0
Lack of efficacy	-	-

Baseline characteristics

Reporting groups	
Reporting group title	UCB7665 dose 1
Reporting group description:	
Participants in this arm received 5 subcutaneous (sc) doses of UCB7665 dose 1 at 1-week intervals.	
Reporting group title	UCB7665 dose 2
Reporting group description:	
Participants in this arm received 3 subcutaneous (sc) doses of UCB7665 dose 2 at 1-week intervals.	
Reporting group title	UCB7665 dose 3
Reporting group description:	
Participants in this arm received 2 subcutaneous (sc) doses of UCB7665 dose 3 at 1-week intervals.	
Reporting group title	UCB7665 dose 4
Reporting group description:	
Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 4.	
Reporting group title	UCB7665 dose 5
Reporting group description:	
Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 5.	

Reporting group values	UCB7665 dose 1	UCB7665 dose 2	UCB7665 dose 3
Number of subjects	15	15	12
Age categorical Units: Subjects			
<=18 years	0	0	0
Between 18 and 65 years	7	14	10
>=65 years	8	1	2
Age continuous Units: years			
arithmetic mean	59.1	46.0	46.3
standard deviation	± 18.4	± 15.9	± 16.8
Gender categorical Units: Subjects			
Male	7	4	5
Female	8	11	7

Reporting group values	UCB7665 dose 4	UCB7665 dose 5	Total
Number of subjects	12	12	66
Age categorical Units: Subjects			
<=18 years	0	0	0
Between 18 and 65 years	11	7	49
>=65 years	1	5	17
Age continuous Units: years			
arithmetic mean	45.8	56.1	
standard deviation	± 14.6	± 18.2	-

Gender categorical Units: Subjects			
Male	5	3	24
Female	7	9	42

End points

End points reporting groups

Reporting group title	UCB7665 dose 1
Reporting group description:	
Participants in this arm received 5 subcutaneous (sc) doses of UCB7665 dose 1 at 1-week intervals.	
Reporting group title	UCB7665 dose 2
Reporting group description:	
Participants in this arm received 3 subcutaneous (sc) doses of UCB7665 dose 2 at 1-week intervals.	
Reporting group title	UCB7665 dose 3
Reporting group description:	
Participants in this arm received 2 subcutaneous (sc) doses of UCB7665 dose 3 at 1-week intervals.	
Reporting group title	UCB7665 dose 4
Reporting group description:	
Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 4.	
Reporting group title	UCB7665 dose 5
Reporting group description:	
Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 5.	
Subject analysis set title	UCB7665 dose 1 (SS)
Subject analysis set type	Safety analysis
Subject analysis set description:	
Participants in this arm received 5 subcutaneous (sc) doses of UCB7665 dose 1 at 1-week intervals. Participants formed the Safety Set (SS).	
Subject analysis set title	UCB7665 dose 2 (SS)
Subject analysis set type	Safety analysis
Subject analysis set description:	
Participants in this arm received 3 subcutaneous (sc) doses of UCB7665 dose 2 at 1-week intervals. Participants formed the SS.	
Subject analysis set title	UCB7665 dose 3 (SS)
Subject analysis set type	Safety analysis
Subject analysis set description:	
Participants in this arm received 2 subcutaneous (sc) doses of UCB7665 dose 3 at 1-week intervals. Participants formed the SS.	
Subject analysis set title	UCB7665 dose 4 (SS)
Subject analysis set type	Safety analysis
Subject analysis set description:	
Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 4. Participants formed the SS.	
Subject analysis set title	UCB7665 dose 5 (SS)
Subject analysis set type	Safety analysis
Subject analysis set description:	
Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 5. Participants formed the SS.	

Primary: Percentage of participants experiencing at least one Treatment Emergent Adverse Event (TEAE) during the study

End point title	Percentage of participants experiencing at least one Treatment Emergent Adverse Event (TEAE) during the study[1]
End point description:	
TEAEs were defined as Adverse Events starting after the time of first Investigational Medicinal Product (IMP) administration up to and including 8 weeks after the final dose.	
End point type	Primary

End point timeframe:

From Visit 2 (Week 1) until End of Study Visit or Early Termination (up to 12 weeks after the first investigational medicinal product (IMP) administration)

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: No formal statistical hypothesis testing was planned for this study. Results were summarized as descriptive statistics only.

End point values	UCB7665 dose 1 (SS)	UCB7665 dose 2 (SS)	UCB7665 dose 3 (SS)	UCB7665 dose 4 (SS)
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	15	15	12	12
Units: percentage of participants				
number (not applicable)	80.0	60.0	58.3	91.7

End point values	UCB7665 dose 5 (SS)			
Subject group type	Subject analysis set			
Number of subjects analysed	12			
Units: percentage of participants				
number (not applicable)	100			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Adverse events were collected from Week 1 until the End-of-Study Visit (8 weeks following the final dose)	
Assessment type	Non-systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	21.1

Reporting groups	
Reporting group title	UCB7665 dose 1 (SS)
Reporting group description:	
Participants in this arm received 5 subcutaneous (sc) doses of UCB7665 dose 1 at 1-week intervals. Participants formed the Safety Set (SS).	
Reporting group title	UCB7665 dose 2 (SS)
Reporting group description:	
Participants in this arm received 3 subcutaneous (sc) doses of UCB7665 dose 2 at 1-week intervals. Participants formed the SS.	
Reporting group title	UCB7665 dose 3 (SS)
Reporting group description:	
Participants in this arm received 2 subcutaneous (sc) doses of UCB7665 dose 3 at 1-week intervals. Participants formed the SS.	
Reporting group title	UCB7665 dose 4 (SS)
Reporting group description:	
Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 4. Participants formed the SS.	
Reporting group title	UCB7665 dose 5 (SS)
Reporting group description:	
Participants in this arm received 1 subcutaneous (sc) dose of UCB7665 dose 5. Participants formed the SS.	

Serious adverse events	UCB7665 dose 1 (SS)	UCB7665 dose 2 (SS)	UCB7665 dose 3 (SS)
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	1 / 12 (8.33%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Investigations			
Platelet count decreased			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Thrombocytopenia			

subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	1 / 12 (8.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Reproductive system and breast disorders			
Genital haemorrhage			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	UCB7665 dose 4 (SS)	UCB7665 dose 5 (SS)	
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 12 (16.67%)	0 / 12 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	
Investigations			
Platelet count decreased			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood and lymphatic system disorders			
Thrombocytopenia			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Reproductive system and breast disorders			
Genital haemorrhage			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

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

Non-serious adverse events	UCB7665 dose 1 (SS)	UCB7665 dose 2 (SS)	UCB7665 dose 3 (SS)
Total subjects affected by non-serious adverse events			
subjects affected / exposed	11 / 15 (73.33%)	9 / 15 (60.00%)	7 / 12 (58.33%)
Vascular disorders			
Hypertension			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Hypotension			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	1 / 12 (8.33%)
occurrences (all)	0	0	1
Asthenia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Influenza like illness			
subjects affected / exposed	1 / 15 (6.67%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	1	1	0
Chest discomfort			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Fatigue			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Infusion site oedema			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Injection site reaction			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Oedema peripheral			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Reproductive system and breast disorders			

Menorrhagia			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Respiratory, thoracic and mediastinal disorders			
Rhinorrhoea			
subjects affected / exposed	1 / 15 (6.67%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	1	1	0
Cough			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Dyspnoea			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Epistaxis			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Oropharyngeal pain			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Rhinitis allergic			
subjects affected / exposed	0 / 15 (0.00%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	0	1	0
Psychiatric disorders			
Insomnia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Investigations			
Neutrophil count decreased			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Blood creatinine increased			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Blood pressure increased			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0

Injury, poisoning and procedural complications			
Contusion			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Arthropod bite			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Fall			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Traumatic haematoma			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Nervous system disorders			
Headache			
subjects affected / exposed	3 / 15 (20.00%)	6 / 15 (40.00%)	3 / 12 (25.00%)
occurrences (all)	6	7	4
Dizziness			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 15 (6.67%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	1	1	0
Immune thrombocytopenic purpura			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Ear and labyrinth disorders			
Vertigo			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	2	0	0
Ear pain			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	1 / 12 (8.33%)
occurrences (all)	0	0	1
Tinnitus			

subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Eye disorders			
Visual impairment			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	2	0	0
Keratitis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	1 / 15 (6.67%)	2 / 15 (13.33%)	1 / 12 (8.33%)
occurrences (all)	1	2	1
Vomiting			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	1 / 12 (8.33%)
occurrences (all)	1	0	1
Mouth haemorrhage			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Toothache			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	2	0	0
Abdominal pain			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Abdominal pain upper			
subjects affected / exposed	0 / 15 (0.00%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	0	1	0
Angina bullosa haemorrhagica			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Constipation			

subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Dyspepsia			
subjects affected / exposed	0 / 15 (0.00%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	0	1	0
Gastritis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Skin reaction			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	2	0	0
Alopecia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Renal and urinary disorders			
Dysuria			
subjects affected / exposed	0 / 15 (0.00%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	0	1	0
Haematuria			
subjects affected / exposed	0 / 15 (0.00%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	0	1	0
Urinary incontinence			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Musculoskeletal and connective tissue disorders			
Joint swelling			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Arthralgia			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Myalgia			

subjects affected / exposed	0 / 15 (0.00%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	0	1	0
Pain in extremity			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Infections and infestations			
Upper respiratory tract infection			
subjects affected / exposed	1 / 15 (6.67%)	1 / 15 (6.67%)	1 / 12 (8.33%)
occurrences (all)	1	1	1
Influenza			
subjects affected / exposed	1 / 15 (6.67%)	1 / 15 (6.67%)	0 / 12 (0.00%)
occurrences (all)	2	1	0
Viral infection			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	1 / 12 (8.33%)
occurrences (all)	1	0	1
Nasopharyngitis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Respiratory tract infection			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	0	0	0
Rhinitis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0
Urinary tract infection			
subjects affected / exposed	0 / 15 (0.00%)	0 / 15 (0.00%)	1 / 12 (8.33%)
occurrences (all)	0	0	1
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	1 / 15 (6.67%)	0 / 15 (0.00%)	0 / 12 (0.00%)
occurrences (all)	1	0	0

Non-serious adverse events	UCB7665 dose 4 (SS)	UCB7665 dose 5 (SS)	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	10 / 12 (83.33%)	12 / 12 (100.00%)	
Vascular disorders			

Hypertension			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Hypotension			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	1 / 12 (8.33%)	3 / 12 (25.00%)	
occurrences (all)	1	3	
Asthenia			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Influenza like illness			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Chest discomfort			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Fatigue			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Infusion site oedema			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
Injection site reaction			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Oedema peripheral			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Reproductive system and breast disorders			
Menorrhagia			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Respiratory, thoracic and mediastinal			

disorders			
Rhinorrhoea			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Cough			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
Dyspnoea			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Epistaxis			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Oropharyngeal pain			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Rhinitis allergic			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Psychiatric disorders			
Insomnia			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
Investigations			
Neutrophil count decreased			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	3	
Blood creatinine increased			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Blood pressure increased			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Injury, poisoning and procedural complications			
Contusion			

subjects affected / exposed	1 / 12 (8.33%)	2 / 12 (16.67%)	
occurrences (all)	1	4	
Arthropod bite			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Fall			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Traumatic haematoma			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Nervous system disorders			
Headache			
subjects affected / exposed	5 / 12 (41.67%)	9 / 12 (75.00%)	
occurrences (all)	6	9	
Dizziness			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Immune thrombocytopenic purpura			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
Ear and labyrinth disorders			
Vertigo			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Ear pain			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Tinnitus			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Eye disorders			

Visual impairment			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Keratitis			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	2 / 12 (16.67%)	2 / 12 (16.67%)	
occurrences (all)	2	2	
Vomiting			
subjects affected / exposed	2 / 12 (16.67%)	4 / 12 (33.33%)	
occurrences (all)	2	4	
Nausea			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Mouth haemorrhage			
subjects affected / exposed	1 / 12 (8.33%)	1 / 12 (8.33%)	
occurrences (all)	1	1	
Toothache			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Abdominal pain			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Abdominal pain upper			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Angina bullosa haemorrhagica			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
Constipation			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Dyspepsia			

subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Gastritis			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Skin reaction			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Alopecia			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Renal and urinary disorders			
Dysuria			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Haematuria			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Urinary incontinence			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Musculoskeletal and connective tissue disorders			
Joint swelling			
subjects affected / exposed	0 / 12 (0.00%)	2 / 12 (16.67%)	
occurrences (all)	0	2	
Arthralgia			
subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
Myalgia			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Pain in extremity			

subjects affected / exposed	0 / 12 (0.00%)	1 / 12 (8.33%)	
occurrences (all)	0	1	
Infections and infestations			
Upper respiratory tract infection			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Influenza			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Viral infection			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Nasopharyngitis			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Respiratory tract infection			
subjects affected / exposed	1 / 12 (8.33%)	0 / 12 (0.00%)	
occurrences (all)	1	0	
Rhinitis			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Urinary tract infection			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	0 / 12 (0.00%)	0 / 12 (0.00%)	
occurrences (all)	0	0	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
19 May 2016	The primary purpose of this substantial amendment (dated 19 May 2016) was to include additional laboratory tests (serology test) at the Screening Visit in order to exclude study participants with chronic and ongoing infections with human immunodeficiency virus (HIV), hepatitis B, and hepatitis C. Exclusion criteria related to medical history were updated to define the criteria precisely and increase clarity. Withdrawal criteria for potential drug-induced liver injury (PDILI) and evaluation of PDILI were also updated to enable the effective management and assessment of any PDILI cases as outlined in the Food and Drug Administration Guidance for Industry, Drug-Induced Liver Injury: Premarketing Clinical Evaluation (Jul 2009). UCB has developed prespecified criteria for managing any PDILI events and discontinuing investigational medicinal product (IMP). In this amendment, a more conservative approach compared with the previous version was included. However, there were no changes in the potential risk of PDILI with rozanolixizumab since the previous version. In addition, height was added in order to be able to calculate body mass index (BMI), the serious adverse event (SAE) reporting details were updated, and several additional exploratory biomarkers were added as immunological variables.
21 October 2016	This substantial amendment (dated 21 Oct 2016) was written to introduce a third cohort of 4 mg/kg subcutaneous (sc) twice weekly. However, based on preliminary emerging data, it was expected that the planned inclusion of a third dose arm with 4 mg/kg of subcutaneous (sc) rozanolixizumab given twice per week would not create additional insight regarding the safety and the tolerability of rozanolixizumab in study participants with immune thrombocytopenia (ITP). Therefore, the planned implementation of Global Protocol Amendment 2.0 was canceled.
15 February 2017	This substantial amendment (dated 15 Feb 2017) was written to further explore the safety, tolerability, and pharmacodynamic (PD) effect of the same cumulative dose of rozanolixizumab administered with higher doses given in fewer sc infusions by integrating 3 new dose arms into the study. The primary purpose of this amendment was to include 3 additional cohorts (Dose Arms 3, 4, and 5) and a nonmandatory genomic substudy. In addition, the required period for contraceptive use and pregnancy testing was reduced from 3 months to 2 months after the final dose in female study participants of childbearing potential.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported