Clinical trial results:
A Multicenter, Randomized, Subject-Blind, Investigator-Blind, Placebo-Controlled, Parallel-Group Study Evaluating the Efficacy, Safety, and Tolerability of Rozanolixizumab in Subjects With Chronic Inflammatory Demyelinating Polyradiculoneuropathy (CIDP)

Summary

EudraCT number	2016-002411-17
Trial protocol	BE DK NL DE ES FR GB
Global end of trial date	31 March 2021

Results information

Result version number	v2 (current)
This version publication date	13 May 2022
First version publication date	25 March 2022
Version creation reason	

Trial information

Trial identification

Sponsor protocol code	CIDP01

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03861481
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	UCB Biopharma SRL
Sponsor organisation address	Allée de la Recherche 60, Brussels, Belgium, 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	28 May 2021
Is this the analysis of the primary completion data?	Yes
Primary completion date	31 March 2021
Global end of trial reached?	Yes
Global end of trial date	31 March 2021
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the clinical efficacy of rozanolixizumab as a treatment for participants with chronic inflammatory demyelinating polyradiculoneuropathy (CIDP).

Protection of trial subjects:

During the conduct of the study all participants were closely monitored. Immediate rescue therapy upon CIDP relapse

Background therapy:

Background therapy as permitted in the protocol.

Evidence for comparator: -

Actual start date of recruitment	26 March 2019
Long term follow-up planned	Yes
Long term follow-up rationale	Ethical reason
Long term follow-up duration	18 Months
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Belgium: 5
Country: Number of subjects enrolled	Denmark: 3
Country: Number of subjects enrolled	France: 3
Country: Number of subjects enrolled	Germany: 3
Country: Number of subjects enrolled	Netherlands: 2
Country: Number of subjects enrolled	Spain: 4
Country: Number of subjects enrolled	United Kingdom: 1
Country: Number of subjects enrolled	United States: 13
Worldwide total number of subjects	34
EEA total number of subjects	20

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)	28
From 65 to 84 years	6
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

The study started to enroll study participants in March 2019 and concluded in March 2021.

Pre-assignment

Screening details:

Participant Flow refers to the Randomized Set.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	Placebo

Arm description:

Participants received placebo matched to rozanolixizumab as a subcutaneous injection once weekly for 12 weeks.

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

Dosage and administration details:

Participants received placebo matched to rozanolixizumab at prespecified time points.

Arm title	Rozanolixizumab

Arm description:

Participants received rozanolixizumab Dose A as a subcutaneous injection once weekly for 12 weeks.

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

Dosage and administration details:

Participants received rozanolixizumab dose A at prespecified time points.

Number of subjects in period 1	Placebo	Rozanolixizumab
Started	17	17
Completed	11	10
Not completed	6	7
COVID-19 pandemic circumstances	-	1
Relapse	1	-
Consent withdrawn by subject	-	1
Participant unable to participate in study CIDP04	1	-
Lack of efficacy	4	5

Baseline characteristics

Reporting groups	
Reporting group title	Placebo
Reporting group description:	
Participants received placebo matched to rozanolixizumab as a subcutaneous injection once weekly for 12 weeks.	
Reporting group title	Rozanolixizumab
Reporting group description:	
Participants received rozanolixizumab Dose A as a subcutaneous injection once weekly for 12 weeks.	

Reporting group values	Placebo	Rozanolixizumab	Total
Number of subjects	17	17	34
Age Categorical			
Units: participants			
<=18 years	0	0	0
Between 18 and 65 years	16	12	28
>=65 years	1	5	6
Age Continuous			
Units: years			
arithmetic mean	56.4	57.3	
standard deviation	± 7.4	± 13.3	-
Sex: Female, Male			
Units: participants			
Female	8	8	16
Male	9	9	18

End points

End points reporting groups

Reporting group title	Placebo
Reporting group description:	
Participants received placebo matched to rozanolixizumab as a subcutaneous injection once weekly for 12 weeks.	
Reporting group title	Rozanolixizumab
Reporting group description:	
Participants received rozanolixizumab Dose A as a subcutaneous injection once weekly for 12 weeks.	

Primary: Change from Baseline to Week 13 (Day 85) in inflammatory Rasch-built Overall Disability Scale (iRODS) score

End point title	Change from Baseline to Week 13 (Day 85) in inflammatory Rasch-built Overall Disability Scale (iRODS) score
End point description:	
iRODS: linearly weighted patient-reported endpoint (questionnaire) that captures activity and social participation limitations in participants with CIDP. It consisted of 24 items and assesses participant's ability to perform daily and social activities. Participants had 3 response options: 0=impossible to perform; 1=performed with difficulty; 2=easily performed, performed without difficulty. Raw sum scores of iRODS (range 0 to 48, where 0=worse and 48=best) were translated to log odds units (logits) scale, placing estimates on same logit scale which had a score range of -6.95 (most severe activity; social participation restrictions) to 8.11 (no activity; social participation limitations). A positive change is associated with better outcome of less disease activity and more social activity.Full Analysis Set: all participants who received at least 1 dose of treatment and had Baseline and post-Baseline iRODS measurement up to Week 13 (Day 85)/premature end of treatment (inclusively).	
End point type	Primary
End point timeframe:	
From Baseline up to Week 13 (Day 85)	

End point values	Placebo	Rozanolixizumab		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	17	16		
Units: score on a scale (logits)				
least squares mean (standard error)	0.234 (± 0.379)	0.181 (± 0.468)		

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	Rozanolixizumab v Placebo

Number of subjects included in analysis	33
Analysis specification	Pre-specified
Analysis type	
Parameter estimate	LS Mean difference (Rozimab - Placebo)
Point estimate	-0.052
Confidence interval	
level	90 %
sides	2-sided
lower limit	-0.892
upper limit	0.788

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From Baseline until the Safety Follow-up Visit (up to Week 24)

Adverse event reporting additional description:

A TEAE is defined as any event that was not present prior the first administration of investigational medicinal product (IMP) or any unresolved event already present before the first administration of IMP that worsens in intensity following exposure to treatment until 8 weeks following the last administration of IMP.

Assessment type	Non-systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	24.0

Reporting groups

Reporting group title	Rozanolixizumab

Reporting group description:

Participants received rozanolixizumab Dose A as a subcutaneous injection once weekly for 12 weeks.

Reporting group title	Placebo

Reporting group description:

Participants received placebo matched to rozanolixizumab as a subcutaneous injection once weekly for 12 weeks.

Serious adverse events	Rozanolixizumab	Placebo	
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 17 (11.76%)	0 / 17 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	
Nervous system disorders			
Chronic inflammatory demyelinating polyradiculoneuropathy			
subjects affected / exposed	2 / 17 (11.76%)	0 / 17 (0.00%)	
occurrences causally related to treatment / all	0 / 2	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	Rozanolixizumab	Placebo	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	15 / 17 (88.24%)	14 / 17 (82.35%)	
Investigations			

Bacterial test positive			
subjects affected / exposed	2 / 17 (11.76%)	0 / 17 (0.00%)	
occurrences (all)	2	0	
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	0 / 17 (0.00%)	2 / 17 (11.76%)	
occurrences (all)	0	2	
Nervous system disorders			
Headache			
subjects affected / exposed	6 / 17 (35.29%)	5 / 17 (29.41%)	
occurrences (all)	9	10	
Chronic inflammatory demyelinating polyradiculoneuropathy			
subjects affected / exposed	4 / 17 (23.53%)	4 / 17 (23.53%)	
occurrences (all)	4	4	
Hypoaesthesia			
subjects affected / exposed	0 / 17 (0.00%)	2 / 17 (11.76%)	
occurrences (all)	0	2	
General disorders and administration site conditions			
Infusion site erythema			
subjects affected / exposed	3 / 17 (17.65%)	0 / 17 (0.00%)	
occurrences (all)	4	0	
Peripheral swelling			
subjects affected / exposed	3 / 17 (17.65%)	0 / 17 (0.00%)	
occurrences (all)	3	0	
Fatigue			
subjects affected / exposed	0 / 17 (0.00%)	3 / 17 (17.65%)	
occurrences (all)	0	3	
Pain			
subjects affected / exposed	0 / 17 (0.00%)	2 / 17 (11.76%)	
occurrences (all)	0	2	
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	2 / 17 (11.76%)	3 / 17 (17.65%)	
occurrences (all)	2	5	
Nausea			

subjects affected / exposed	0 / 17 (0.00%)	2 / 17 (11.76%)	
occurrences (all)	0	6	
Skin and subcutaneous tissue disorders			
Rash			
subjects affected / exposed	0 / 17 (0.00%)	2 / 17 (11.76%)	
occurrences (all)	0	3	
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	3 / 17 (17.65%)	0 / 17 (0.00%)	
occurrences (all)	3	0	
Infections and infestations			
Urinary tract infection			
subjects affected / exposed	0 / 17 (0.00%)	3 / 17 (17.65%)	
occurrences (all)	0	4	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
15 November 2018	After the protocol was completed at UCB, but before it was submitted to any regulatory authorities or IRBs/IECs, it was updated to better align it with the current understanding of available data from completed and ongoing studies with rozanolixizumab. The updated protocol was then submitted to regulatory authorities and the study began.
09 July 2019	The purpose of Amendment 2 was to provide clarification on the dose of IMP administered (including an allowance of ±10% compared with the target dose A), as well as a flexible infusion rate. Sensitivity analyses were introduced to account for deviations outside the ±10% target dosage; the descriptive analyses were to inform about actual doses administered to the study participants. The visit windows and the scheduling of the Randomization Visit in view of the Ig treatment at study start were clarified. The predominance of objective criteria over the investigator's judgement was confirmed for the assessment of CIDP relapse. The timeframe of expected use of contraception poststudy completion was extended to 90 days in view of the probable half-life of rozanolixizumab. Exclusion Criterion No. 3 was extended to a prediabetic condition. The expectation with regards to the use of cannabidiols and medicinal marijuana was clarified in the concomitant medication section. This amendment confirmed the expectation of a single rater for the INCAT assessment to ensure consistency of the rating during the course of the study.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? Yes

Date	Interruption	Restart date
20 March 2020	Recruitment was stopped from 20 Mar 20 due to Covid pandemic (participants in screening at the moment could not be randomized – participants under treatment continued in the study as judged appropriate by the investigators/participants).	03 June 2020

Notes:

Limitations and caveats

None reported