# 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<br>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<br>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<br>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<br>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