



## 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          | v2 (current)     |
| This version publication date  | 07 March 2024    |
| First version publication date | 19 February 2020 |
| Version creation reason        |                  |

#### 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 SRL   |
| 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 (rozanolixizumab) 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? | Yes           |

Notes:

## Population of trial subjects

### Subjects enrolled per country

|                                      |                         |
|--------------------------------------|-------------------------|
| Country: Number of subjects enrolled | Bulgaria: 1             |
| Country: Number of subjects enrolled | Czechia: 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         | 49                      |

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             |
| <b>Arm title</b>             | UCB7665 4 mg/kg |

Arm description:

Participants in this arm received 5 subcutaneous (sc) doses of UCB7665 (rozanolixizumab) 4 milligram per kilograms (mg/kg) 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 sc infusion using an infusion pump which was programmed at a constant flow rate.

|                  |                 |
|------------------|-----------------|
| <b>Arm title</b> | UCB7665 7 mg/kg |
|------------------|-----------------|

Arm description:

Participants in this arm received 3 sc doses of UCB7665 (rozanolixizumab) 7 mg/kg 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 sc infusion using an infusion pump which was programmed at a constant flow rate.

|                  |                  |
|------------------|------------------|
| <b>Arm title</b> | UCB7665 10 mg/kg |
|------------------|------------------|

Arm description:

Participants in this arm received 2 sc doses of UCB7665 (rozanolixizumab) 10 mg/kg 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 sc infusion using an infusion pump which was programmed at a constant flow rate.

|                  |                  |
|------------------|------------------|
| <b>Arm title</b> | UCB7665 15 mg/kg |
|------------------|------------------|

Arm description:

Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 15 mg/kg.

|  |                                  |
|--|----------------------------------|
| 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 sc infusion using an infusion pump which was programmed at a constant flow rate.

|                  |                  |
|------------------|------------------|
| <b>Arm title</b> | UCB7665 20 mg/kg |
|------------------|------------------|

Arm description:

Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 20 mg/kg.

|  |                                  |
|--|----------------------------------|
| 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 sc infusion using an infusion pump which was programmed at a constant flow rate.

| <b>Number of subjects in period 1</b> | UCB7665 4 mg/kg | UCB7665 7 mg/kg | UCB7665 10 mg/kg |
|---------------------------------------|-----------------|-----------------|------------------|
| Started                               | 15              | 15              | 12               |
| Completed                             | 14              | 15              | 12               |
| Not completed                         | 1               | 0               | 0                |
| Lack of efficacy                      | 1               | -               | -                |

| <b>Number of subjects in period 1</b> | UCB7665 15 mg/kg | UCB7665 20 mg/kg |
|---------------------------------------|------------------|------------------|
| Started                               | 12               | 12               |
| Completed                             | 12               | 12               |
| Not completed                         | 0                | 0                |
| Lack of efficacy                      | -                | -                |



## Baseline characteristics

### Reporting groups

|   |                  |
|---|------------------|
| Reporting group title   | UCB7665 4 mg/kg  |
| Reporting group description:  |                  |
| Participants in this arm received 5 subcutaneous (sc) doses of UCB7665 (rozanolixizumab) 4 milligram per kilograms (mg/kg) at 1-week intervals. |                  |
| Reporting group title   | UCB7665 7 mg/kg  |
| Reporting group description:  |                  |
| Participants in this arm received 3 sc doses of UCB7665 (rozanolixizumab) 7 mg/kg at 1-week intervals.  |                  |
| Reporting group title   | UCB7665 10 mg/kg |
| Reporting group description:  |                  |
| Participants in this arm received 2 sc doses of UCB7665 (rozanolixizumab) 10 mg/kg at 1-week intervals.   |                  |
| Reporting group title   | UCB7665 15 mg/kg |
| Reporting group description:  |                  |
| Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 15 mg/kg.  |                  |
| Reporting group title   | UCB7665 20 mg/kg |
| Reporting group description:  |                  |
| Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 20 mg/kg.  |                  |

| Reporting group values  | UCB7665 4 mg/kg | UCB7665 7 mg/kg | UCB7665 10 mg/kg |
|-------------------------|-----------------|-----------------|------------------|
| 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 15 mg/kg | UCB7665 20 mg/kg | 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 4 mg/kg       |
| Reporting group description:<br>Participants in this arm received 5 subcutaneous (sc) doses of UCB7665 (rozanolixizumab) 4 milligram per kilograms (mg/kg) at 1-week intervals.      |                       |
| Reporting group title  | UCB7665 7 mg/kg       |
| Reporting group description:<br>Participants in this arm received 3 sc doses of UCB7665 (rozanolixizumab) 7 mg/kg at 1-week intervals.   |                       |
| Reporting group title  | UCB7665 10 mg/kg      |
| Reporting group description:<br>Participants in this arm received 2 sc doses of UCB7665 (rozanolixizumab) 10 mg/kg at 1-week intervals.  |                       |
| Reporting group title  | UCB7665 15 mg/kg      |
| Reporting group description:<br>Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 15 mg/kg.   |                       |
| Reporting group title  | UCB7665 20 mg/kg      |
| Reporting group description:<br>Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 20 mg/kg.   |                       |
| Subject analysis set title   | UCB7665 4 mg/kg (SS)  |
| Subject analysis set type  | Safety analysis       |
| Subject analysis set description:<br>Participants in this arm received 5 sc doses of UCB7665 (rozanolixizumab) 4 mg/kg at 1-week intervals. Participants formed the Safety Set (SS). |                       |
| Subject analysis set title   | UCB7665 7 mg/kg (SS)  |
| Subject analysis set type  | Safety analysis       |
| Subject analysis set description:<br>Participants in this arm received 3 sc doses of UCB7665 (rozanolixizumab) 7 mg/kg at 1-week intervals. Participants formed the SS.              |                       |
| Subject analysis set title   | UCB7665 10 mg/kg (SS) |
| Subject analysis set type  | Safety analysis       |
| Subject analysis set description:<br>Participants in this arm received 2 sc doses of UCB7665 (rozanolixizumab) 10 mg/kg at 1-week intervals. Participants formed the SS.             |                       |
| Subject analysis set title   | UCB7665 15 mg/kg (SS) |
| Subject analysis set type  | Safety analysis       |
| Subject analysis set description:<br>Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 15 mg/kg. Participants formed the SS.                                  |                       |
| Subject analysis set title   | UCB7665 20 mg/kg (SS) |
| Subject analysis set type  | Safety analysis       |
| Subject analysis set description:<br>Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 20 mg/kg. 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 <sup>[1]</sup> |
| End point description:<br>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 in tables as descriptive statistics only.

| End point values                  | UCB7665 4<br>mg/kg (SS) | UCB7665 7<br>mg/kg (SS) | UCB7665 10<br>mg/kg (SS) | UCB7665 15<br>mg/kg (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 20<br>mg/kg (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) (up to 12 weeks after the first dose of IMP)

|                 |                |
|-----------------|----------------|
| Assessment type | Non-systematic |
|-----------------|----------------|

### Dictionary used

|                 |        |
|-----------------|--------|
| Dictionary name | MedDRA |
|-----------------|--------|

|                    |      |
|--------------------|------|
| Dictionary version | 21.1 |
|--------------------|------|

### Reporting groups

|                       |                      |
|-----------------------|----------------------|
| Reporting group title | UCB7665 4 mg/kg (SS) |
|-----------------------|----------------------|

Reporting group description:

Participants in this arm received 5 sc doses of UCB7665 (rozanolixizumab) 4 mg/kg at 1-week intervals. Participants formed the Safety Set (SS).

|                       |                      |
|-----------------------|----------------------|
| Reporting group title | UCB7665 7 mg/kg (SS) |
|-----------------------|----------------------|

Reporting group description:

Participants in this arm received 3 sc doses of UCB7665 (rozanolixizumab) 7 mg/kg at 1-week intervals. Participants formed the SS.

|                       |                       |
|-----------------------|-----------------------|
| Reporting group title | UCB7665 20 mg/kg (SS) |
|-----------------------|-----------------------|

Reporting group description:

Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 20 mg/kg. Participants formed the SS.

|                       |                       |
|-----------------------|-----------------------|
| Reporting group title | UCB7665 15 mg/kg (SS) |
|-----------------------|-----------------------|

Reporting group description:

Participants in this arm received 1 sc dose of UCB7665 (rozanolixizumab) 15 mg/kg. Participants formed the SS.

|                       |                       |
|-----------------------|-----------------------|
| Reporting group title | UCB7665 10 mg/kg (SS) |
|-----------------------|-----------------------|

Reporting group description:

Participants in this arm received 2 sc doses of UCB7665 (rozanolixizumab) 10 mg/kg at 1-week intervals. Participants formed the SS.

| Serious adverse events                            | UCB7665 4 mg/kg (SS) | UCB7665 7 mg/kg (SS) | UCB7665 20 mg/kg (SS) |
|---|----------------------|----------------------|-----------------------|
| Total subjects affected by serious adverse events |                      |                      |                       |
| subjects affected / exposed                       | 1 / 15 (6.67%)       | 0 / 15 (0.00%)       | 0 / 12 (0.00%)        |
| 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%) | 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          |
| 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          |

| <b>Serious adverse events</b>                     | UCB7665 15 mg/kg (SS) | UCB7665 10 mg/kg (SS) |  |
|---|-----------------------|-----------------------|--|
| Total subjects affected by serious adverse events |                       |                       |  |
| subjects affected / exposed                       | 2 / 12 (16.67%)       | 1 / 12 (8.33%)        |  |
| 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%)        | 1 / 12 (8.33%)        |  |
| occurrences causally related to treatment / all   | 0 / 1                 | 0 / 1                 |  |
| 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 %

| <b>Non-serious adverse events</b>                           | UCB7665 4 mg/kg<br>(SS) | UCB7665 7 mg/kg<br>(SS) | UCB7665 20 mg/kg<br>(SS) |
|---|-------------------------|-------------------------|--------------------------|
| Total subjects affected by non-serious adverse events       |                         |                         |                          |
| subjects affected / exposed                                 | 11 / 15 (73.33%)        | 9 / 15 (60.00%)         | 12 / 12 (100.00%)        |
| <b>Vascular disorders</b>                                   |                         |                         |                          |
| 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%)          | 1 / 12 (8.33%)           |
| occurrences (all)   | 0                       | 0                       | 1                        |
| <b>General disorders and administration site conditions</b> |                         |                         |                          |
| Pyrexia   |                         |                         |                          |
| subjects affected / exposed                                 | 0 / 15 (0.00%)          | 0 / 15 (0.00%)          | 3 / 12 (25.00%)          |
| occurrences (all)   | 0                       | 0                       | 3                        |
| 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                        |
| 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%)          | 1 / 12 (8.33%)           |
| occurrences (all)   | 0                       | 0                       | 1                        |
| 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                        |
| Chest discomfort  |                         |                         |                          |
| subjects affected / exposed                                 | 1 / 15 (6.67%)          | 0 / 15 (0.00%)          | 0 / 12 (0.00%)           |
| occurrences (all)   | 1                       | 0                       | 0                        |
| <b>Reproductive system and breast disorders</b>             |                         |                         |                          |

|  |                     |                     |                     |
|--|---------------------|---------------------|---------------------|
| Menorrhagia<br>subjects affected / exposed<br>occurrences (all)                | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Respiratory, thoracic and mediastinal disorders                                |                     |                     |                     |
| Rhinorrhoea<br>subjects affected / exposed<br>occurrences (all)                | 1 / 15 (6.67%)<br>1 | 1 / 15 (6.67%)<br>1 | 0 / 12 (0.00%)<br>0 |
| Cough<br>subjects affected / exposed<br>occurrences (all)                      | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 1 / 12 (8.33%)<br>1 |
| Dyspnoea<br>subjects affected / exposed<br>occurrences (all)                   | 1 / 15 (6.67%)<br>1 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Oropharyngeal pain<br>subjects affected / exposed<br>occurrences (all)         | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Epistaxis<br>subjects affected / exposed<br>occurrences (all)                  | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Rhinitis allergic<br>subjects affected / exposed<br>occurrences (all)          | 0 / 15 (0.00%)<br>0 | 1 / 15 (6.67%)<br>1 | 0 / 12 (0.00%)<br>0 |
| Psychiatric disorders  |                     |                     |                     |
| Insomnia<br>subjects affected / exposed<br>occurrences (all)                   | 1 / 15 (6.67%)<br>1 | 0 / 15 (0.00%)<br>0 | 1 / 12 (8.33%)<br>1 |
| Investigations   |                     |                     |                     |
| Blood pressure increased<br>subjects affected / exposed<br>occurrences (all)   | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Blood creatinine increased<br>subjects affected / exposed<br>occurrences (all) | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Neutrophil count decreased<br>subjects affected / exposed<br>occurrences (all) | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 1 / 12 (8.33%)<br>3 |

|  |                 |                 |                 |
|--|-----------------|-----------------|-----------------|
| Injury, poisoning and procedural complications |                 |                 |                 |
| Traumatic haematoma                            |                 |                 |                 |
| 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               |
| Arthropod bite                                 |                 |                 |                 |
| subjects affected / exposed                    | 1 / 15 (6.67%)  | 0 / 15 (0.00%)  | 0 / 12 (0.00%)  |
| occurrences (all)                              | 1               | 0               | 0               |
| Contusion                                      |                 |                 |                 |
| subjects affected / exposed                    | 0 / 15 (0.00%)  | 0 / 15 (0.00%)  | 2 / 12 (16.67%) |
| occurrences (all)                              | 0               | 0               | 4               |
| Nervous system disorders                       |                 |                 |                 |
| Headache                                       |                 |                 |                 |
| subjects affected / exposed                    | 3 / 15 (20.00%) | 6 / 15 (40.00%) | 9 / 12 (75.00%) |
| occurrences (all)                              | 6               | 7               | 9               |
| Dizziness                                      |                 |                 |                 |
| subjects affected / exposed                    | 0 / 15 (0.00%)  | 0 / 15 (0.00%)  | 1 / 12 (8.33%)  |
| occurrences (all)                              | 0               | 0               | 1               |
| 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%)  | 1 / 12 (8.33%)  |
| occurrences (all)                              | 0               | 0               | 1               |
| 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               |
| Tinnitus                                       |                 |                 |                 |
| subjects affected / exposed                    | 1 / 15 (6.67%)  | 0 / 15 (0.00%)  | 0 / 12 (0.00%)  |
| occurrences (all)                              | 1               | 0               | 0               |
| Ear pain                                       |                 |                 |                 |

|  |                     |                     |                     |
|--|---------------------|---------------------|---------------------|
| subjects affected / exposed<br>occurrences (all) | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Eye disorders                                    |                     |                     |                     |
| Keratitis  |                     |                     |                     |
| subjects affected / exposed                      | 1 / 15 (6.67%)      | 0 / 15 (0.00%)      | 0 / 12 (0.00%)      |
| occurrences (all)                                | 1                   | 0                   | 0                   |
| Visual impairment                                |                     |                     |                     |
| subjects affected / exposed                      | 1 / 15 (6.67%)      | 0 / 15 (0.00%)      | 0 / 12 (0.00%)      |
| occurrences (all)                                | 2                   | 0                   | 0                   |
| Gastrointestinal disorders                       |                     |                     |                     |
| Mouth haemorrhage                                |                     |                     |                     |
| subjects affected / exposed                      | 0 / 15 (0.00%)      | 0 / 15 (0.00%)      | 1 / 12 (8.33%)      |
| occurrences (all)                                | 0                   | 0                   | 1                   |
| 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                   |
| Nausea   |                     |                     |                     |
| subjects affected / exposed                      | 1 / 15 (6.67%)      | 0 / 15 (0.00%)      | 0 / 12 (0.00%)      |
| occurrences (all)                                | 1                   | 0                   | 0                   |
| Vomiting   |                     |                     |                     |
| subjects affected / exposed                      | 0 / 15 (0.00%)      | 0 / 15 (0.00%)      | 4 / 12 (33.33%)     |
| occurrences (all)                                | 0                   | 0                   | 4                   |
| Diarrhoea  |                     |                     |                     |
| subjects affected / exposed                      | 1 / 15 (6.67%)      | 2 / 15 (13.33%)     | 2 / 12 (16.67%)     |
| occurrences (all)                                | 1                   | 2                   | 2                   |
| 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%)      | 1 / 12 (8.33%)      |
| occurrences (all)                                | 0                   | 0                   | 1                   |
| Constipation                                     |                     |                     |                     |



|  |                     |                     |                     |
|--|---------------------|---------------------|---------------------|
| subjects affected / exposed<br>occurrences (all)   | 1 / 15 (6.67%)<br>1 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Dyspepsia<br>subjects affected / exposed<br>occurrences (all)  | 0 / 15 (0.00%)<br>0 | 1 / 15 (6.67%)<br>1 | 0 / 12 (0.00%)<br>0 |
| Gastritis<br>subjects affected / exposed<br>occurrences (all)  | 1 / 15 (6.67%)<br>1 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Skin and subcutaneous tissue disorders<br>Pruritus<br>subjects affected / exposed<br>occurrences (all)         | 1 / 15 (6.67%)<br>1 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Skin reaction<br>subjects affected / exposed<br>occurrences (all)  | 1 / 15 (6.67%)<br>2 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Alopecia<br>subjects affected / exposed<br>occurrences (all)   | 1 / 15 (6.67%)<br>1 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Renal and urinary disorders<br>Urinary incontinence<br>subjects affected / exposed<br>occurrences (all)        | 1 / 15 (6.67%)<br>1 | 0 / 15 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |
| Dysuria<br>subjects affected / exposed<br>occurrences (all)  | 0 / 15 (0.00%)<br>0 | 1 / 15 (6.67%)<br>1 | 0 / 12 (0.00%)<br>0 |
| Haematuria<br>subjects affected / exposed<br>occurrences (all)   | 0 / 15 (0.00%)<br>0 | 1 / 15 (6.67%)<br>1 | 0 / 12 (0.00%)<br>0 |
| Musculoskeletal and connective tissue disorders<br>Myalgia<br>subjects affected / exposed<br>occurrences (all) | 0 / 15 (0.00%)<br>0 | 1 / 15 (6.67%)<br>1 | 0 / 12 (0.00%)<br>0 |
| Arthralgia<br>subjects affected / exposed<br>occurrences (all)   | 0 / 15 (0.00%)<br>0 | 0 / 15 (0.00%)<br>0 | 1 / 12 (8.33%)<br>1 |
| Joint swelling   |                     |                     |                     |

|   |                       |                       |                 |
|---|-----------------------|-----------------------|-----------------|
| subjects affected / exposed                           | 0 / 15 (0.00%)        | 0 / 15 (0.00%)        | 2 / 12 (16.67%) |
| occurrences (all)                                     | 0                     | 0                     | 2               |
| Pain in extremity                                     |                       |                       |                 |
| subjects affected / exposed                           | 0 / 15 (0.00%)        | 0 / 15 (0.00%)        | 1 / 12 (8.33%)  |
| occurrences (all)                                     | 0                     | 0                     | 1               |
| Infections and infestations                           |                       |                       |                 |
| Influenza   |                       |                       |                 |
| subjects affected / exposed                           | 1 / 15 (6.67%)        | 1 / 15 (6.67%)        | 0 / 12 (0.00%)  |
| occurrences (all)                                     | 2                     | 1                     | 0               |
| Upper respiratory tract infection                     |                       |                       |                 |
| subjects affected / exposed                           | 1 / 15 (6.67%)        | 1 / 15 (6.67%)        | 0 / 12 (0.00%)  |
| occurrences (all)                                     | 1                     | 1                     | 0               |
| Respiratory tract infection                           |                       |                       |                 |
| subjects affected / exposed                           | 0 / 15 (0.00%)        | 0 / 15 (0.00%)        | 0 / 12 (0.00%)  |
| occurrences (all)                                     | 0                     | 0                     | 0               |
| Nasopharyngitis                                       |                       |                       |                 |
| subjects affected / exposed                           | 1 / 15 (6.67%)        | 0 / 15 (0.00%)        | 0 / 12 (0.00%)  |
| occurrences (all)                                     | 1                     | 0                     | 0               |
| Viral infection                                       |                       |                       |                 |
| subjects affected / exposed                           | 1 / 15 (6.67%)        | 0 / 15 (0.00%)        | 0 / 12 (0.00%)  |
| occurrences (all)                                     | 1                     | 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%)        | 0 / 12 (0.00%)  |
| occurrences (all)                                     | 0                     | 0                     | 0               |
| 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               |
| <b>Non-serious adverse events</b>                     | UCB7665 15 mg/kg (SS) | UCB7665 10 mg/kg (SS) |                 |
| Total subjects affected by non-serious adverse events |                       |                       |                 |
| subjects affected / exposed                           | 10 / 12 (83.33%)      | 7 / 12 (58.33%)       |                 |
| Vascular disorders                                    |                       |                       |                 |

|   |                     |                     |  |
|---|---------------------|---------------------|--|
| Hypertension<br>subjects affected / exposed<br>occurrences (all)  | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| Hypotension<br>subjects affected / exposed<br>occurrences (all)   | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| General disorders and administration site conditions<br>Pyrexia<br>subjects affected / exposed<br>occurrences (all) | 1 / 12 (8.33%)<br>1 | 1 / 12 (8.33%)<br>1 |  |
| Asthenia<br>subjects affected / exposed<br>occurrences (all)  | 1 / 12 (8.33%)<br>1 | 0 / 12 (0.00%)<br>0 |  |
| Influenza like illness<br>subjects affected / exposed<br>occurrences (all)  | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| Fatigue<br>subjects affected / exposed<br>occurrences (all)   | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| Infusion site oedema<br>subjects affected / exposed<br>occurrences (all)  | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| Injection site reaction<br>subjects affected / exposed<br>occurrences (all)   | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| Oedema peripheral<br>subjects affected / exposed<br>occurrences (all)   | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| Chest discomfort<br>subjects affected / exposed<br>occurrences (all)  | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| Reproductive system and breast disorders<br>Menorrhagia<br>subjects affected / exposed<br>occurrences (all)         | 1 / 12 (8.33%)<br>1 | 0 / 12 (0.00%)<br>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%) | 0 / 12 (0.00%) |  |
| occurrences (all)                              | 0              | 0              |  |
| Dyspnoea                                       |                |                |  |
| subjects affected / exposed                    | 0 / 12 (0.00%) | 0 / 12 (0.00%) |  |
| occurrences (all)                              | 0              | 0              |  |
| Oropharyngeal pain                             |                |                |  |
| subjects affected / exposed                    | 1 / 12 (8.33%) | 0 / 12 (0.00%) |  |
| occurrences (all)                              | 1              | 0              |  |
| Epistaxis                                      |                |                |  |
| 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%) | 0 / 12 (0.00%) |  |
| occurrences (all)                              | 0              | 0              |  |
| Investigations                                 |                |                |  |
| Blood pressure increased                       |                |                |  |
| subjects affected / exposed                    | 1 / 12 (8.33%) | 0 / 12 (0.00%) |  |
| occurrences (all)                              | 1              | 0              |  |
| Blood creatinine increased                     |                |                |  |
| subjects affected / exposed                    | 1 / 12 (8.33%) | 0 / 12 (0.00%) |  |
| occurrences (all)                              | 1              | 0              |  |
| Neutrophil count decreased                     |                |                |  |
| subjects affected / exposed                    | 0 / 12 (0.00%) | 0 / 12 (0.00%) |  |
| occurrences (all)                              | 0              | 0              |  |
| Injury, poisoning and procedural complications |                |                |  |
| Traumatic haematoma                            |                |                |  |

|   |                      |                      |  |
|---|----------------------|----------------------|--|
| subjects affected / exposed<br>occurrences (all)  | 0 / 12 (0.00%)<br>0  | 0 / 12 (0.00%)<br>0  |  |
| Fall<br>subjects affected / exposed<br>occurrences (all)  | 0 / 12 (0.00%)<br>0  | 0 / 12 (0.00%)<br>0  |  |
| Arthropod bite<br>subjects affected / exposed<br>occurrences (all)                                  | 0 / 12 (0.00%)<br>0  | 0 / 12 (0.00%)<br>0  |  |
| Contusion<br>subjects affected / exposed<br>occurrences (all)                                       | 1 / 12 (8.33%)<br>1  | 0 / 12 (0.00%)<br>0  |  |
| Nervous system disorders<br>Headache<br>subjects affected / exposed<br>occurrences (all)            | 5 / 12 (41.67%)<br>6 | 3 / 12 (25.00%)<br>4 |  |
| Dizziness<br>subjects affected / exposed<br>occurrences (all)                                       | 0 / 12 (0.00%)<br>0  | 0 / 12 (0.00%)<br>0  |  |
| Blood and lymphatic system disorders<br>Anaemia<br>subjects affected / exposed<br>occurrences (all) | 1 / 12 (8.33%)<br>1  | 0 / 12 (0.00%)<br>0  |  |
| Immune thrombocytopenic purpura<br>subjects affected / exposed<br>occurrences (all)                 | 0 / 12 (0.00%)<br>0  | 0 / 12 (0.00%)<br>0  |  |
| Ear and labyrinth disorders<br>Vertigo<br>subjects affected / exposed<br>occurrences (all)          | 0 / 12 (0.00%)<br>0  | 0 / 12 (0.00%)<br>0  |  |
| Tinnitus<br>subjects affected / exposed<br>occurrences (all)  | 0 / 12 (0.00%)<br>0  | 0 / 12 (0.00%)<br>0  |  |
| Ear pain<br>subjects affected / exposed<br>occurrences (all)  | 0 / 12 (0.00%)<br>0  | 1 / 12 (8.33%)<br>1  |  |
| Eye disorders   |                      |                      |  |

|                              |                 |                |  |
|------------------------------|-----------------|----------------|--|
| Keratitis                    |                 |                |  |
| subjects affected / exposed  | 0 / 12 (0.00%)  | 0 / 12 (0.00%) |  |
| occurrences (all)            | 0               | 0              |  |
| Visual impairment            |                 |                |  |
| subjects affected / exposed  | 0 / 12 (0.00%)  | 0 / 12 (0.00%) |  |
| occurrences (all)            | 0               | 0              |  |
| Gastrointestinal disorders   |                 |                |  |
| Mouth haemorrhage            |                 |                |  |
| subjects affected / exposed  | 1 / 12 (8.33%)  | 0 / 12 (0.00%) |  |
| occurrences (all)            | 1               | 0              |  |
| 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              |  |
| Nausea                       |                 |                |  |
| subjects affected / exposed  | 1 / 12 (8.33%)  | 1 / 12 (8.33%) |  |
| occurrences (all)            | 1               | 1              |  |
| Vomiting                     |                 |                |  |
| subjects affected / exposed  | 2 / 12 (16.67%) | 0 / 12 (0.00%) |  |
| occurrences (all)            | 2               | 0              |  |
| Diarrhoea                    |                 |                |  |
| subjects affected / exposed  | 2 / 12 (16.67%) | 1 / 12 (8.33%) |  |
| occurrences (all)            | 2               | 1              |  |
| 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%)  | 0 / 12 (0.00%) |  |
| occurrences (all)            | 0               | 0              |  |
| Constipation                 |                 |                |  |
| subjects affected / exposed  | 0 / 12 (0.00%)  | 0 / 12 (0.00%) |  |
| occurrences (all)            | 0               | 0              |  |
| Dyspepsia                    |                 |                |  |

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

|  |                     |                     |  |
|--|---------------------|---------------------|--|
| subjects affected / exposed<br>occurrences (all) | 0 / 12 (0.00%)<br>0 | 0 / 12 (0.00%)<br>0 |  |
| Infections and infestations                      |                     |                     |  |
| Influenza  |                     |                     |  |
| subjects affected / exposed                      | 0 / 12 (0.00%)      | 0 / 12 (0.00%)      |  |
| occurrences (all)                                | 0                   | 0                   |  |
| Upper respiratory tract infection                |                     |                     |  |
| subjects affected / exposed                      | 1 / 12 (8.33%)      | 1 / 12 (8.33%)      |  |
| occurrences (all)                                | 1                   | 1                   |  |
| Respiratory tract infection                      |                     |                     |  |
| subjects affected / exposed                      | 1 / 12 (8.33%)      | 0 / 12 (0.00%)      |  |
| occurrences (all)                                | 1                   | 0                   |  |
| Nasopharyngitis                                  |                     |                     |  |
| subjects affected / exposed                      | 0 / 12 (0.00%)      | 0 / 12 (0.00%)      |  |
| occurrences (all)                                | 0                   | 0                   |  |
| Viral infection                                  |                     |                     |  |
| subjects affected / exposed                      | 0 / 12 (0.00%)      | 1 / 12 (8.33%)      |  |
| occurrences (all)                                | 0                   | 1                   |  |
| 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%)      | 1 / 12 (8.33%)      |  |
| occurrences (all)                                | 0                   | 1                   |  |
| 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