



## Clinical trial results:

### A Randomized, Global, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy and Safety of Vedolizumab IV for the Treatment of Primary Sclerosing Cholangitis, With Underlying Inflammatory Bowel Disease

#### Summary

|                          |                                  |
|--------------------------|----------------------------------|
| EudraCT number           | 2014-003942-28                   |
| Trial protocol           | ES BE GB HU SE DE CZ AT PL FR IT |
| Global end of trial date | 23 February 2017                 |

#### Results information

|                                |                   |
|--------------------------------|-------------------|
| Result version number          | v1 (current)      |
| This version publication date  | 13 September 2020 |
| First version publication date | 13 September 2020 |

#### Trial information

##### Trial identification

|                       |              |
|-----------------------|--------------|
| Sponsor protocol code | MLN0002-3023 |
|-----------------------|--------------|

##### Additional study identifiers

|                                    |  |
|------------------------------------|--|
| ISRCTN number                      | -  |
| ClinicalTrials.gov id (NCT number) | NCT03035058  |
| WHO universal trial number (UTN)   | U1111-1161-4900  |
| Other trial identifiers            | NL56650.056.16: CCMO, 16/LO/0288: NRES, 191059: HC-CTD |

Notes:

##### Sponsors

|                              |  |
|------------------------------|--|
| Sponsor organisation name    | Takeda   |
| Sponsor organisation address | One Takeda Parkway, Deerfield, United States, 60015  |
| Public contact               | Medical Director, Clinical Science, Takeda Development Centre Europe, Ltd., +44 1256 894003, |
| Scientific contact           | Medical Director, Clinical Science, Takeda Development Centre Europe, Ltd., +44 1256 894003, |

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:

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**Results analysis stage**

|  |                  |
|--|------------------|
| Analysis stage                                       | Final            |
| Date of interim/final analysis                       | 23 February 2017 |
| Is this the analysis of the primary completion data? | No               |
| Global end of trial reached?                         | Yes              |
| Global end of trial date                             | 23 February 2017 |
| Was the trial ended prematurely?                     | No               |

Notes:

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**General information about the trial**

Main objective of the trial:

The purpose of this study is to evaluate the efficacy and safety of vedolizumab intravenous (IV) in non-end-stage primary sclerosing cholangitis (PSC) participants with underlying inflammatory bowel disease (IBD).

Protection of trial subjects:

N/A

Background therapy: -

Evidence for comparator: -

|   |                  |
|---|------------------|
| Actual start date of recruitment                          | 23 February 2017 |
| Long term follow-up planned                               | No               |
| Independent data monitoring committee (IDMC) involvement? | Yes              |

Notes:

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**Population of trial subjects**

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**Subjects enrolled per country**

|                                      |               |
|--------------------------------------|---------------|
| Country: Number of subjects enrolled | France: 99999 |
| Worldwide total number of subjects   | 99999         |
| EEA total number of subjects         | 99999         |

Notes:

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**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)                      | 99999 |
| From 65 to 84 years                       | 0     |
| 85 years and over                         | 0     |

## Subject disposition

### Recruitment

Recruitment details:

99999 is "Not applicable" value or 0 participants, this trial was discontinued with no participants enrolled in the trial.

### Pre-assignment

Screening details:

N/A

### Period 1

|                              |                                |
|------------------------------|--------------------------------|
| Period 1 title               | Overall Trial (overall period) |
| Is this the baseline period? | Yes                            |
| Allocation method            | Randomised - controlled        |
| Blinding used                | Not blinded                    |

### Arms

|           |         |
|-----------|---------|
| Arm title | Overall |
|-----------|---------|

Arm description:

Vedolizumab 300 mg, intravenous (IV)

|  |  |
|--|--|
| Arm type                               | Experimental                                     |
| Investigational medicinal product name | Vedolizumab IV                                   |
| Investigational medicinal product code | MLN0002  |
| Other name                             |  |
| Pharmaceutical forms                   | Powder for concentrate for solution for infusion |
| Routes of administration               | Intravenous use                                  |

Dosage and administration details:

Dosage would have been once at Day 1 and Week 2 then once every 4 weeks from Week 6 to Week 102.

|                                       |         |
|---------------------------------------|---------|
| <b>Number of subjects in period 1</b> | Overall |
| Started                               | 99999   |
| Completed                             | 99999   |

## Baseline characteristics

### Reporting groups

|                       |         |
|-----------------------|---------|
| Reporting group title | Overall |
|-----------------------|---------|

Reporting group description:

Vedolizumab 300 mg, intravenous (IV)

| Reporting group values                                | Overall | Total |  |
|---|---------|-------|--|
| Number of subjects                                    | 99999   | 99999 |  |
| Age categorical                                       |         |       |  |
| Units: Subjects                                       |         |       |  |
| In utero  | 0       | 0     |  |
| Preterm newborn infants<br>(gestational age < 37 wks) | 0       | 0     |  |
| Newborns (0-27 days)                                  | 0       | 0     |  |
| Infants and toddlers (28 days-23<br>months)           | 0       | 0     |  |
| Children (2-11 years)                                 | 0       | 0     |  |
| Adolescents (12-17 years)                             | 0       | 0     |  |
| Adults (18-64 years)                                  | 99999   | 99999 |  |
| From 65-84 years                                      | 0       | 0     |  |
| 85 years and over                                     | 0       | 0     |  |
| Age continuous  |         |       |  |
| Units: years  |         |       |  |
| arithmetic mean                                       | 0       |       |  |
| standard deviation                                    | ± 0     | -     |  |
| Gender categorical                                    |         |       |  |
| Units: Subjects                                       |         |       |  |
| Female  | 99999   | 99999 |  |
| Male  | 0       | 0     |  |

## End points

### End points reporting groups

|  |         |
|--|---------|
| Reporting group title  | Overall |
| Reporting group description:<br>Vedolizumab 300 mg, intravenous (IV) |         |

### Primary: Percentage of Participants with No Worsening in Ishak Fibrosis Staging Score from Baseline to Week 106 Visit

|  |   |
|--|---|
| End point title  | Percentage of Participants with No Worsening in Ishak Fibrosis Staging Score from Baseline to Week 106 Visit <sup>[1]</sup> |
| End point description:<br>99999 is "Not applicable" value or 0 participants, this trial was discontinued with no participants enrolled in the trial. |   |
| End point type   | Primary   |
| End point timeframe:<br>N/A  |   |

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 statistical analyses have been specified for this primary end point.

| End point values            | Overall              |  |  |  |
|-----------------------------|----------------------|--|--|--|
| Subject group type          | Reporting group      |  |  |  |
| Number of subjects analysed | 99999 <sup>[2]</sup> |  |  |  |
| Units: number               | 99999                |  |  |  |

Notes:

[2] - No subjects were enrolled in the trial hence results are not available

### Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information<sup>[1]</sup>

Timeframe for reporting adverse events:

All adverse events occurring during the course of the clinical trial were to be collected.

Adverse event reporting additional description:

N/A

|                 |            |
|-----------------|------------|
| Assessment type | Systematic |
|-----------------|------------|

### Dictionary used

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

|                    |   |
|--------------------|---|
| Dictionary version | 0 |
|--------------------|---|

### Reporting groups

|                       |             |
|-----------------------|-------------|
| Reporting group title | Vedolizumab |
|-----------------------|-------------|

Reporting group description:

Vedolizumab 300 mg IV

| Serious adverse events                            | Vedolizumab       |  |  |
|---|-------------------|--|--|
| Total subjects affected by serious adverse events |                   |  |  |
| subjects affected / exposed                       | 0 / 99999 (0.00%) |  |  |
| number of deaths (all causes)                     | 0                 |  |  |
| number of deaths resulting from adverse events    | 0                 |  |  |

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

| Non-serious adverse events                            | Vedolizumab       |  |  |
|---|-------------------|--|--|
| Total subjects affected by non-serious adverse events |                   |  |  |
| subjects affected / exposed                           | 0 / 99999 (0.00%) |  |  |

Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.

Justification: There are no non-serious adverse events recorded for these results.

## More information

### Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

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### Interruptions (globally)

Were there any global interruptions to the trial? No

### Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

|  |
|--|
| 99999 is "Not applicable" value or 0 participants, this trial was discontinued with no participants enrolled in the trial. |
|--|

Notes: