



## Clinical trial results: Rotterdam Observational Study in CIDP of Pharmacokinetics of Intravenous -globulin

### Summary

|                          |                 |
|--------------------------|-----------------|
| EudraCT number           | 2013-004988-32  |
| Trial protocol           | NL              |
| Global end of trial date | 25 October 2015 |

### Results information

|                                   |                                                                                    |
|-----------------------------------|------------------------------------------------------------------------------------|
| Result version number             | v1 (current)                                                                       |
| This version publication date     | 13 December 2021                                                                   |
| First version publication date    | 13 December 2021                                                                   |
| Summary attachment (see zip file) | PKPD_IVIg_CIDP (Fokkink_et_al-2017-Clinical_Pharmacology_&_Therapeutics_final.pdf) |

### Trial information

#### Trial identification

|                       |                |
|-----------------------|----------------|
| Sponsor protocol code | NL46993.078.13 |
|-----------------------|----------------|

#### Additional study identifiers

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

Notes:

### Sponsors

|                              |                                                                               |
|------------------------------|-------------------------------------------------------------------------------|
| Sponsor organisation name    | Erasmus MC                                                                    |
| Sponsor organisation address | Dr. Molewaterplein 50, Rotterdam, Netherlands, PO 2040 3000CA                 |
| Public contact               | GBS workgroup Erasmus MC, Erasmus University Medical Center, 0031 0107043430, |
| Scientific contact           | GBS workgroup Erasmus MC, Erasmus University Medical Center, 0031 0107043430, |

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                       | 30 November 2016 |
| Is this the analysis of the primary completion data? | Yes              |
| Primary completion date                              | 25 October 2015  |
| Global end of trial reached?                         | Yes              |
| Global end of trial date                             | 25 October 2015  |
| Was the trial ended prematurely?                     | No               |

Notes:

## General information about the trial

Main objective of the trial:

The main objective of the study is to determine the PK and PD of IVIg during maintenance treatment in patients with CIDP. These data will be used to conduct a NONMEM analysis in relation to the dosage, frequency and batch of IVIg used.

Protection of trial subjects:

The canula for IV administration was left in place longer to obtain serum samples shortly after infusion in order to minimize blood drawings.

Background therapy: -

Evidence for comparator: -

|                                                           |               |
|-----------------------------------------------------------|---------------|
| Actual start date of recruitment                          | 30 April 2014 |
| Long term follow-up planned                               | No            |
| Independent data monitoring committee (IDMC) involvement? | No            |

Notes:

## Population of trial subjects

### Subjects enrolled per country

|                                      |                 |
|--------------------------------------|-----------------|
| Country: Number of subjects enrolled | Netherlands: 15 |
| Worldwide total number of subjects   | 15              |
| EEA total number of subjects         | 15              |

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)                      | 5  |
| From 65 to 84 years                       | 10 |
| 85 years and over                         | 0  |

## Subject disposition

### Recruitment

Recruitment details: -

### Pre-assignment

Screening details:

Screening criteria were: must be diagnosed with CIDP (EFNS/PNS), EMG findings compatible with this diagnosis, age 18 and older, be on maintenance treatment with IVIg, agree on and sign the informed consent.

### Period 1

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

### Arms

|                                        |                                 |
|----------------------------------------|---------------------------------|
| Arm title                              | Overall trial                   |
| Arm description: -                     |                                 |
| Arm type                               | Experimental                    |
| Investigational medicinal product name | Kiovig                          |
| Investigational medicinal product code | J06BA02                         |
| Other name                             |                                 |
| Pharmaceutical forms                   | Solution for injection/infusion |
| Routes of administration               | Intravenous use                 |

Dosage and administration details:

Highly variable and patient dependent: ranges from 15 to 60 (g). g gram(s)

| Number of subjects in period 1 | Overall trial |
|--------------------------------|---------------|
| Started                        | 15            |
| Completed                      | 15            |

## Baseline characteristics

### Reporting groups

|                                |               |
|--------------------------------|---------------|
| Reporting group title          | Overall trial |
| Reporting group description: - |               |

| Reporting group values                                | Overall trial | Total |  |
|-------------------------------------------------------|---------------|-------|--|
| Number of subjects                                    | 15            | 15    |  |
| Age categorical                                       |               |       |  |
| Units: Subjects                                       |               |       |  |
| In utero                                              |               | 0     |  |
| Preterm newborn infants<br>(gestational age < 37 wks) |               | 0     |  |
| Newborns (0-27 days)                                  |               | 0     |  |
| Infants and toddlers (28 days-23<br>months)           |               | 0     |  |
| Children (2-11 years)                                 |               | 0     |  |
| Adolescents (12-17 years)                             |               | 0     |  |
| Adults (18-64 years)                                  |               | 0     |  |
| From 65-84 years                                      |               | 0     |  |
| 85 years and over                                     |               | 0     |  |
| Age continuous                                        |               |       |  |
| Units: years                                          |               |       |  |
| median                                                | 69            |       |  |
| full range (min-max)                                  | 37 to 79      | -     |  |
| Gender categorical                                    |               |       |  |
| Units: Subjects                                       |               |       |  |
| Female                                                | 4             | 4     |  |
| Male                                                  | 11            | 11    |  |

## End points

### End points reporting groups

|                                |               |
|--------------------------------|---------------|
| Reporting group title          | Overall trial |
| Reporting group description: - |               |

### Primary: Serum IgG levels

|                        |                                 |
|------------------------|---------------------------------|
| End point title        | Serum IgG levels <sup>[1]</sup> |
| End point description: |                                 |

|                |         |
|----------------|---------|
| End point type | Primary |
|----------------|---------|

End point timeframe:

From first sample collected (30-04-2014) until last sample collected (25-10-2015)

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: Primary and secondary pharmacokinetic parameters were calculated using PKSolver's version 2.0 noncompartmental analysis tool for infusion (Linear up – Log down) or the two-compartmental option. There are no arms to compare, yet adding a statistical analysis with one arm keeps getting flagged as an error (despite the option is given). Hence to complete this form no statistical analyses were specified. For an overview of statistical analyses used please see the linked and/or enclosed publication.

|                             |                 |  |  |  |
|-----------------------------|-----------------|--|--|--|
| <b>End point values</b>     | Overall trial   |  |  |  |
| Subject group type          | Reporting group |  |  |  |
| Number of subjects analysed | 15              |  |  |  |
| Units: g/L                  |                 |  |  |  |
| number (not applicable)     | 15              |  |  |  |

### Statistical analyses

No statistical analyses for this end point

## Adverse events

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

Timeframe for reporting adverse events:

From start of the trial (30-04-2014) until the last blood sample was collected (25-10-2015).

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

### Dictionary used

|                 |       |
|-----------------|-------|
| Dictionary name | Excel |
|-----------------|-------|

|                    |      |
|--------------------|------|
| Dictionary version | 2010 |
|--------------------|------|

### Reporting groups

|                       |              |
|-----------------------|--------------|
| Reporting group title | All subjects |
|-----------------------|--------------|

Reporting group description: -

| Serious adverse events                            | All subjects   |  |  |
|---------------------------------------------------|----------------|--|--|
| Total subjects affected by serious adverse events |                |  |  |
| subjects affected / exposed                       | 0 / 15 (0.00%) |  |  |
| number of deaths (all causes)                     | 0              |  |  |
| number of deaths resulting from adverse events    | 0              |  |  |

Frequency threshold for reporting non-serious adverse events: 0 %

| Non-serious adverse events                            | All subjects   |  |  |
|-------------------------------------------------------|----------------|--|--|
| Total subjects affected by non-serious adverse events |                |  |  |
| subjects affected / exposed                           | 0 / 15 (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: SAEs during or after treatment with IVIg in general are rare, given all these patients were on maintenance treatment with IVIg before inclusion in the trial the occurrence of any AE was a priori very limited. After completion of the last blood sample collection no AE was objectified and/or reported.

## 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

None reported