

# TAK-771-4002: Evaluating the Safety of GAMMAGARD LIQUID for the Treatment of Patients With Chronic Inflammatory Demyelinating Polyradiculoneuropathy

**First published:** 11/03/2022

**Last updated:** 02/07/2024

Study

Finalised

## Administrative details

### **PURI**

<https://redirect.ema.europa.eu/resource/50068>

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### **EU PAS number**

EUPAS46101

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### **Study ID**

50068

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### **DARWIN EU® study**

No

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## Study countries

United States

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## Study description

This study evaluates the safety of GAMMAGARD LIQUID (GGL) in patients with chronic inflammatory demyelinating polyradiculoneuropathy (CIDP) in real-world healthcare delivery databases in the United States: IBM MarketScan Research Databases, and Optum Clinformatics Data Mart. The primary objectives are to evaluate rates of adverse events of special interest (thrombosis, acute kidney injury, hemolysis) among patients with CIDP initiating GGL compared with rates among patients with CIDP initiating comparator intravenous immunoglobulin (IVIG) products. IVIG initiation and use will be evaluated with medical procedure and pharmacy claims data. CIDP status and other patient demographic and clinical characteristics will be evaluated with medical diagnosis, procedure, and pharmacy dispensing coding and enrollment information on or before IVIG initiation. The analysis will be conducted separately in each data source, and pooled estimates will be calculated if appropriate. Primary outcomes (thrombosis, AKI, hemolysis) and other secondary outcomes will be evaluated in medical diagnosis claims data using claims-based algorithms validated in IVIG users, when available.

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## Study status

Finalised

## Research institutions and networks

### Institutions

[RTI Health Solutions \(RTI-HS\)](#)

- France
- Spain
- Sweden
- United Kingdom
- United Kingdom (Northern Ireland)
- United States

**First published:** 21/04/2010

**Last updated:** 13/03/2025

**Institution**

**Not-for-profit**

**ENCePP partner**

## Contact details

### Study institution contact

Study contact Takeda

**Study contact**

[TrialDisclosures@takeda.com](mailto:TrialDisclosures@takeda.com)

### Primary lead investigator

Study contact Takeda

**Primary lead investigator**

## Study timelines

### Date when funding contract was signed

Actual: 02/02/2022

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**Study start date**

Actual: 28/05/2022

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**Data analysis start date**

Actual: 28/05/2022

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**Date of final study report**

Actual: 21/03/2023

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Takeda

## Study protocol

[TAK-771-4002-protocol-original\\_redact.pdf](#)(1.52 MB)

## Regulatory

**Was the study required by a regulatory body?**

No

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**Is the study required by a Risk Management Plan (RMP)?**

Not applicable

## Methodological aspects

### Study type

### Study type list

**Study topic:**

Disease /health condition  
Human medicinal product

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**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

**Data collection methods:**

Secondary use of data

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**Main study objective:**

The objective is to study to evaluate the rates of adverse events of special interest (AESIs) (thrombotic events, acute kidney injury AKI, and hemolytic events) among participants with CIDP initiating GGL compared with rates among participants with CIDP initiating comparator intravenous immunoglobulin (IVIg) products.

## Study Design

**Non-interventional study design**

Cohort  
Other

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**Non-interventional study design, other**

Non-randomized, active-comparator, new-user, retrospective study

## Study drug and medical condition

## **Anatomical Therapeutic Chemical (ATC) code**

(J06BA02) immunoglobulins, normal human, for intravascular adm.

immunoglobulins, normal human, for intravascular adm.

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## **Medical condition to be studied**

Chronic inflammatory demyelinating polyradiculoneuropathy

## Population studied

### **Short description of the study population**

The study population included adult patients aged  $\geq 18$  years with chronic inflammatory demyelinating polyradiculoneuropathy (CIDP) received treatment with intravenous immunoglobulin in the years 2008 through 2019 identified from IBM MarketScan Research Databases, and Optum Clinformatics Data Mart.

Inclusion criteria

To be eligible for inclusion in either study cohort:

- Have a minimum of 6 months of continuous enrollment in the study database with medical and pharmacy coverage before the index date (to accurately define patient characteristics). Gaps in continuous enrollment  $\leq 31$  days are permitted.
- Fulfill the CIDP diagnosis algorithm on or before the index date using all available baseline data for each patient.

To be eligible for the Ig-naive (new-to-class) cohort:

- Be free of any previous recorded use of any Ig product (i.e., study IVIG products, nonstudy IVIG products, or subcutaneous Ig products) at any point before IVIG initiation

To be eligible for the Ig-experienced (new-to-drug) cohort:

- Have any previous recorded use of an Ig product (i.e., study IVIG products,

nonstudy IVIG products, or subcutaneous Ig products) at any point before the index date

Exclusion criteria:

Patients in both study cohorts will be excluded if they fulfill any of the following exclusion criteria:

- Having claims for  $\geq 2$  different IVIG products on the index date, as accurate categorization of the index IVIG product would not be possible
  - Recorded diagnosis of any of the following conditions on or before the index date, to reduce the potential for misclassification of CIDP status among patients using IVIG
    - PID, as PID is an approved indication for treatment with GGL
    - Evidence of secondary immunodeficiency (SID), including patients with recorded diagnoses of hematological malignancy (e.g., diagnosis of multiple myeloma or chronic lymphocytic leukemia) or treatment with rituximab, as short courses of IVIG may be used for SID treatment
    - Idiopathic thrombocytopenic purpura (ITP)
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### **Age groups**

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

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### **Special population of interest**

Other

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### **Special population of interest, other**

Chronic inflammatory demyelinating polyradiculoneuropathy patients

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## Estimated number of subjects

7000

# Study design details

## Outcomes

1. Hazard ratio of Thrombotic Events (TEs) between GGL and comparator IVIG products  
2. Hazard ratio of Acute kidney injury (AKI) between GGL and comparator IVIG products  
3. Hazard ratio of Hemolytic Events (HEs) between GGL and comparator IVIG products  
1. Hazard ratio of Anaphylaxis between GGL and comparator IVIG products  
2. Hazard ratio of Transfusion-related Acute Lung Injury (TRALI) between GGL and comparator IVIG products  
3. Hazard ratio of transfusion-associated Circulatory Overload (TACO) between GGL and comparator IVIG products

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## Data analysis plan

All analyses will be performed separately in the 2 data sources (MarketScan Research Databases, Optum Clinformatics Data Mart) and the data source-specific results will be reported separately. Pooling of the final results across data sources will be performed, if appropriate. Within each data source, the 2 cohorts (Ig naive and Ig experienced) will be analyzed and reported separately, except in secondary analyses combining the 2 cohorts.

# Documents

## Study results

[TAK-771-4002-clinical-study-report-redact.pdf\(1.38 MB\)](#)

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## Data management

## Data sources



**Data source(s), other**

IBM MarketScan Research Databases United States, Optum Clinformatics Data Mart United States

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**Data sources (types)**

[Administrative healthcare records \(e.g., claims\)](#)

## Use of a Common Data Model (CDM)

**CDM mapping**

No

## Data quality specifications

**Check conformance**

Unknown

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

## **Data characterisation conducted**

No