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

EU PAS number

EUPAS46101

Study ID

50068

DARWIN EU® study

No

Study countries

United States

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.

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

Primary lead investigator

Study contact Takeda

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 02/02/2022

Study start date
Actual: 28/05/2022

Data analysis start date
Actual: 28/05/2022

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

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

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Secondary use of data

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

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.

Medical condition to be studied

Chronic inflammatory demyelinating polyradiculoneuropathy

Population studied

Short description of the study population

The study population included adult patients aged ≥ 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 ≤ 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 ≥ 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)

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)

Special population of interest

Other

Special population of interest, other

Chronic inflammatory demyelinating polyradiculoneuropathy patients

---

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

---

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)

Data management

Data sources

Data source(s), other

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

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

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No