Effectiveness and Safety of Avacopan as Add-on to Standard of Care (SOC) Versus SOC Alone in ANCA-associated Vasculitis (LIBRA) (20240051)

First published: 08/05/2026

Last updated: 24/11/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000985

Study ID

1000000985

DARWIN EU® study

No

Study countries

United States

Study description

This retrospective, observational comparative-effectiveness study will assess the real-world effectiveness and safety of avacopan plus standard of care (SOC) versus SOC alone in adults with newly diagnosed or relapsing granulomatosis with polyangiitis (GPA) or microscopic polyangiitis (MPA). It uses longitudinal secondary data from US healthcare databases, including Optum Market Clarity and Komodo Healthcare Map, and a sequential nested-trial framework to emulate treatment comparisons in routine practice. Primary objectives are to compare time to first relapse, major adverse kidney events (MAKE) within 12 months, and the proportion of patients with a 30-day average prednisone-equivalent daily dose (PEDD) ≤7.5 mg at prespecified follow-up points. Secondary objectives include glucocorticoid-related complications, relapse incidence by 12 months, individual MAKE components, hepatotoxicity/drug-induced liver injury, and serious hypersensitivity reactions. Because few events are expected for the latter two outcomes, analyses will be descriptive: cumulative risk will be estimated by treatment arm, without between-arm comparisons. Primary and secondary analyses were prespecified before comparative analyses, except that two longitudinal glucocorticoid analyses were post hoc in Optum; they are prespecified secondary objectives for Komodo and any later database analyses. A staged analytic process with predefined checkpoints is used so that design refinements and protocol changes are not informed by comparative results. Each database will be analyzed separately using a common prespecified framework; results may be combined with the protocol’s overlap-aware meta-analytic approach if overlap can be sufficiently characterized. The Komodo analysis may be repeated after an upcoming data refresh. Medicare, Medicaid, or other databases may be considered after Komodo if fit for purpose and likely to provide a reasonable sample size; no such analyses are currently committed.

Study status

Ongoing

Research institutions and networks

Institutions

Amgen

United States

First published: 01/02/2024

Last updated: 27/03/2026

Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

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Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 10/02/2026

Actual: 10/02/2026

Study start date
Planned: 10/02/2026

Actual: 10/02/2026

Data analysis start date
Planned: 17/03/2026

Actual: 13/04/2026

Date of final study report
Planned: 30/12/2027

Sources of funding

- No external funding

Study protocol

Protocol-Published Amendment avacopan 20240051 1 .pdf (667.67 KB)

Protocol Amendment avacopan 20240051 4.pdf (1.61 MB)

Protocol-Published Amendment avacopan 20240051 3 .pdf (710.03 KB)

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 topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

This is a retrospective observational comparative effectiveness cohort study of avacopan + SOC vs SOC with sequential nested trials. Analyses will be performed separately by database. Results may be meta-analyzed pending assessment of cross-database overlap.

Main study objective:

The main objective of this study is to estimate the real-world effectiveness of avacopan + SOC versus SOC among adults with antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis (AAV).

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

TAVNEOS

Medicinal product name, other

Avacopan

Study drug International non-proprietary name (INN) or common name

AVACOPAN

Anatomical Therapeutic Chemical (ATC) code

(L04) IMMUNOSUPPRESSANTS

IMMUNOSUPPRESSANTS

Medical condition to be studied

Granulomatosis with polyangiitis

Microscopic polyangiitis

Population studied

Short description of the study population

Adults with newly diagnosed or relapsing GPA/MPA.

Age groups

- **Adult and elderly population (≥18 years)**

Estimated number of subjects

6.000

Study design details

Setting

This study will be conducted in multiple real-world data sources, including the Optum Market Clarity database and the Komodo Healthcare Map. Only closed claims will be used for this study. The study period will be from October 1, 2015, to the latest available date. The indexing period will be October 1, 2021, to 3 months prior to the latest available date.

Comparators

Patients starting treatment for new/relapsing GPA/MPA with rituximab (RTX) or cyclophosphamide (CYC).

Outcomes

The primary outcomes of this study are:

1. Time to first relapse.
2. Number of Major Adverse Kidney Events (MAKE) within 12 months (composite endpoint of mortality, dialysis, kidney transplant, end-stage kidney disease).
3. 30-day average prednisone-equivalent daily dose (PEDD) ≤ 7.5mg at 90, 120, 180, 270, and 365 days post- index.

The secondary outcomes of this study are:

1. Incidence of GC-related events within 12 months (composite of serious infection requiring hospitalization, initiation of new class of anti-hyperglycemic medication, major osteoporotic fractures [vertebra, pelvis, humerus, radius/ulna, hip, other femur]).
2. Incidence of relapse within 12 months.
3. Incidence of MAKE components within 12 months:

a. Mortality

b. Dialysis

c. Kidney transplant

d. End-stage kidney disease
4. Incidence within 12 months of hepatotoxicity and drug-induced liver injury, and serious hypersensitivity reactions, including angioedema and anaphylaxis.

Post-hoc Analyses

• 30-day average PEDD (and change from baseline 30-day average PEDD) by 30, 60, 90, 120, 150, 180, 210, 240, 270, 300, 330, 360 days
• Cumulative glucocorticoid exposure by 30, 60, 90, 120, 150, 180, 210, 240, 270, 300, 330, 360 days

The post-hoc glucocorticoid exposure analyses described below were motivated by findings from the Optum Market Clarity analysis, which indicated that additional characterization of glucocorticoid use over time would be useful for interpretation of the comparative effectiveness results. These analyses were added after review of Optum results but before creation of the Komodo cohort and before conduct of the corresponding analyses in Komodo. Accordingly, the two glucocorticoid analyses were post-hoc for Optum and prespecified secondary objectives for Komodo and any subsequent database analyses that are conducted.

Data analysis plan

Analyses will be conducted separately within Optum Market Clarity and Komodo Healthcare Map using the same prespecified analytic framework. Within each database, analyses will proceed in stages with prespecified checkpoints to (a) confirm comparability of treatment groups after weighting, and (b) review of results from negative control outcome analyses. Descriptive analyses will summarize baseline characteristics overall and by treatment group, including disease severity proxies and treatment patterns. Meta-analysis may be conducted only if sufficient independent observations remain following

assessment of cross-database overlap. The protocol’s overlap-aware meta-analytic approach may be used if overlap can be sufficiently characterized.

Data management

Data sources

Data source(s), other

Optum Market Clarity database: a linked dataset of electronic health records and
administrative claims.
Komodo Healthcare Map: a large-scale, longitudinal claims database capturing medical and pharmacy encounters across the United States.

Data sources (types)

Administrative healthcare records (e.g., claims)

Electronic healthcare records (EHR)

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