Retrospective Cohort Study Evaluating Clinical Outcomes in Patients with Granulomatosis with Polyangiitis (GPA)/Microscopic Polyangiitis (MPA) Treated with Avacopan (20230026)

First published: 09/04/2024

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Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/1000000065

EU PAS number

EUPAS1000000065

Study ID

1000000065

DARWIN EU® study

No

Study countries

United States

Study status

Ongoing

Research institutions and networks

Institutions

Amgen

United States

First published: 01/02/2024

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Institution

Mass General Brigham

Cleveland Clinic Foundation’s Center for Vasculitis Care and Research

Contact details

Study institution contact

Global Development Leader Amgen Inc.

Study contact

medinfo@amgen.com

Primary lead investigator

Global Development Leader Amgen Inc.

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 29/09/2023

Actual: 27/10/2023

Study start date

Planned: 02/04/2024

Actual: 02/04/2024

Data analysis start date

Planned: 01/04/2024

Actual: 02/04/2024

Date of final study report

Planned: 03/04/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Amgen Inc.

Study protocol

Redacted Protocol-Published Original avacopan 20230026 .pdf(603.86 KB)

Regulatory

Was the study required by a regulatory body?

No

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

Non-EU RMP only

Other study registration identification numbers and links

20230026

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Main study objective:

To describe characteristics associated with avacopan prescription and initiation, and outcomes observed among avacopan initiators. Among avacopan initiators: (1) investigate factors associated with glucocorticoid discontinuation and relapse risk; and (2) evaluate healthcare resource utilization.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

TAVNEOS

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

AVACOPAN

Anatomical Therapeutic Chemical (ATC) code

(L04AJ05) avacopan

avacopan

Medical condition to be studied

Granulomatosis with polyangiitis

Microscopic polyangiitis

Population studied

Age groups

Adults (18 to < 65 years)

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)

Estimated number of subjects

305

Study design details

Outcomes

Primary Outcomes:

• Number or participants achieving discontinuation of glucocorticoids for treatment of GPA or MPA 1 month before Month 6 and disease remission within 6 months of avacopan initiation.

• Number of participants achieving sustained remission at Month 12, defined as remission at Month 6 and remission at Month 12, with no glucocorticoid treatment for GPA/MPA 1 month before Month 12, and no relapse between Months 6 and 12.

Data analysis plan

There are three primary analyses. Analysis 1 will describe the baseline characteristics of participants by avacopan prescription status (yes/no), as well as the baseline characteristics of participants prescribed avacopan by initiation status (yes/no). Analyses 2 and 3 will examine the 6- and 12-month outcomes among initiators of avacopan, respectively. Analysis 1 will be performed at least six months after the study start date when the final cohort of avacopan initiators is identified and baseline data are collected. Analyses 2 and 3 will begin once at least 6 months after the last avacopan participant is identified during the inclusion period and at least 6 months of follow-up data are collected. The primary outcomes will be analysed using a descriptive approach. Remission will be based on the Birmingham Vasculitis Activity Score version 3 with/out glucocorticoids for the treatment of GPA/MPA in the 4 weeks prior to the month 6 or 12 visits. Summary statistics for continuous variables, including the number of patients, mean, standard deviation (SD), median, Q1, Q3, minimum and maximum, will be calculated after excluding missing/unknown values.

Data management

Data sources

Data source(s), other

Mass General Brigham (MGB) Electronic Data Warehouse (EDW)
Research patient data registry (RPDR)
Cleveland Clinic Foundation’s Center for Vasculitis Care and Research
Social Security Master Death File

NIH United States Renal Data Set

National Death Index

Data sources (types)

Electronic healthcare records (EHR)

Other

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

No

Check completeness

No

Check stability

No

Check logical consistency

No

Data characterisation

Data characterisation conducted

No