

# 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

**Last updated:** 19/10/2024

Study

Ongoing

## Administrative details

### 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

**Last updated:** 21/02/2024

**Institution**

[Mass General Brigham](#)

[Cleveland Clinic Foundation's Center for Vasculitis  
Care and Research](#)

### Contact details

#### Study institution contact

Global Development Leader Amgen Inc.  
[medinfo@amgen.com](mailto:medinfo@amgen.com)

**Study contact**

### **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

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### **Study start date**

Planned: 02/04/2024

Actual: 02/04/2024

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### **Data analysis start date**

Planned: 01/04/2024

Actual: 02/04/2024

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### **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

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**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

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**Study type:**

**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

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

AVACOPAN

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**Anatomical Therapeutic Chemical (ATC) code**

(L04AJ05) avacopan

avacopan

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## **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)

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### **Estimated number of subjects**

305

## Study design details

### **Outcomes**

Primary Outcomes:

- Number of 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.
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## 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

### ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

### 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

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**Data sources (types)**

[Electronic healthcare records \(EHR\)](#)

[Other](#)

## Use of a Common Data Model (CDM)

**CDM mapping**

No

## Data quality specifications

**Check conformance**

No

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**Check completeness**

No

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**Check stability**

No

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### **Check logical consistency**

No

## Data characterisation

### **Data characterisation conducted**

No