VASCULITIS FOUNDATION®

Early Diagnosis · Better Treatment · Improving Lives

June 9, 2026

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

**RE: Docket No. FDA-2026-N-1321** Comment on the Proposed Withdrawal of NDA 214487 for TAVNEOS® (Avacopan); Request for a Full Benefit-Risk Determination That Incorporates Patient Perspectives and the Risks of Available Alternatives

To the Dockets Management Staff and the Director of the Center for Drug Evaluation and Research:

## Who We Are

The Vasculitis Foundation (VF) is the leading patient-focused nonprofit organization in the world dedicated to improving the lives of people affected by vasculitis. Established in 1986, VF works to advance research, improve access to care, and support the more than 500,000 Americans living with all forms of vasculitis, including ANCA-associated vasculitis (AAV), specifically granulomatosis with polyangiitis (GPA) and microscopic polyangiitis (MPA).

We submit these comments not as regulatory or clinical experts, but as the primary organizational voice for the vasculitis patient community, a community that will bear the direct and lasting consequences of the FDA's decision in this matter.

## Conflict of Interest Disclosure

The VF discloses that both ChemoCentryx, Inc. (the original developer of TAVNEOS) and Amgen (which acquired ChemoCentryx in October 2022) have been corporate sponsors and partners of the Vasculitis Foundation.

The VF operates under a Board-approved Corporate Relations Policy and an Industry Interaction Policy, both adopted January 1, 2026, that govern these relationships and are designed to protect the independence of VF's positions, communications, and educational content. These policies ensure that corporate sponsorships do not influence VF's advocacy, that

VF does not endorse any company's specific products, and that VF retains editorial control of all its materials.

The comments submitted here reflect the independent judgment of the VF Board of Directors and are grounded in our obligation to the vasculitis patient community, not in the interests of any corporate partner.

## The VF's Position

The Vasculitis Foundation does not take a position on whether the FDA should or should not proceed with the proposed withdrawal of TAVNEOS. We recognize that the FDA's safety review process exists for important reasons, and we take seriously the concerns that have been raised regarding the assessment of clinical trial data and reports of liver injury.

What we do urge, clearly and emphatically, is this: the FDA's final benefit-risk determination must fully incorporate patient perspectives, must account for the serious risks of the available alternatives, and must reflect the profound unmet need of a community that has very few effective treatment options for a chronic, serious, and potentially fatal disease.

We ask the FDA to ensure its process is complete, transparent, and patient-centered before any final decision is made.

## The Unmet Need Is Serious and Well-Documented

AAV is a rare, chronic, relapsing disease with an estimated prevalence of approximately three cases per 100,000 people in the United States. It is characterized by inflammation of blood vessels that can restrict blood flow and damage vital organs, including the kidneys, lungs, sinuses, and trachea. More than 75 percent of patients with AAV have renal involvement characterized by rapidly progressive glomerulonephritis, which is a leading predictor of mortality in this population.

Patients with AAV are frequently misdiagnosed, and delays in establishing the correct diagnosis negatively affect clinical outcomes, increasing both morbidity and mortality. Even after diagnosis, treatment options are limited and their effectiveness varies considerably from patient to patient.

The VF Board of Directors wrote to the FDA in April 2021 in support of the original application for Avacopan. At that time, we emphasized the burden of existing therapies and the critical need for targeted treatment options. Those facts have not changed.

## The Risks of Available Alternatives Cannot Be Discounted

Any benefit-risk assessment of TAVNEOS must be conducted with clear-eyed recognition of what patients face in its absence. The existing standard of care for AAV carries well-established, serious, and sometimes fatal risks.

Current AAV treatment relies primarily on non-specific immunosuppressants (cyclophosphamide or rituximab) combined with prolonged courses of high-dose glucocorticoids. Glucocorticoid use is associated with a significant side-effect burden including serious infections (which can be fatal in immunocompromised patients), diabetes mellitus, osteoporosis, weight gain, adrenal suppression, and substantially reduced quality of life. These are not rare complications; they are the lived reality of the majority of patients with AAV.

TAVNEOS was the only FDA-approved targeted oral treatment for severe active GPA and MPA. Its removal from the market does not leave patients with safer options. It leaves them with fewer options, all of which carry their own serious and well-documented risks.

Rare disease patients, and the physicians who treat them, routinely and reasonably accept significant risk in exchange for meaningful clinical benefit. That calculus requires having options. The FDA's benefit-risk framework is designed to weigh exactly this kind of tradeoff, and we ask that it do so fully here.

## Patient Perspectives Must Be Part of the Process

The VF hears regularly from hundreds of individuals living with AAV. We hear about the burden of their illness, the isolation their symptoms create, and their deep frustration with the limited availability of effective treatments. We also hear from patients and physicians about the clinical impact of treatment options, including what is lost when those options are removed.

We urge the FDA to ensure that patient perspectives are formally incorporated into the benefit-risk determination for this decision, not as an afterthought, but as a substantive input that the agency's framework is designed to accommodate. The VF is prepared to facilitate access to patient voices in this community if the FDA finds that useful.

VASCULITIS FOUNDATION

Early Diagnosis · Better Treatment · Improving Lives

## Our Request

The Vasculitis Foundation respectfully asks the FDA to:

1. Ensure that the final benefit-risk determination for TAVNEOS fully and explicitly weighs the risks of the available alternatives for patients with GPA and MPA.
2. Formally incorporate patient perspectives and real-world experience into the benefit-risk analysis, consistent with the FDA's own patient-focused drug development guidance.
3. Conduct and communicate this process transparently, so that patients, caregivers, and clinicians can understand the basis for whatever final decision is reached.
4. Consider all available regulatory tools, including updated labeling, enhanced monitoring, and restricted prescribing conditions, before concluding that withdrawal is the only appropriate response.

The vasculitis community has waited a long time for treatment options that address the underlying mechanisms of this disease with fewer cumulative harms than the existing standard of care. We ask the FDA to honor the seriousness of that need in its process.

Respectfully submitted,

Vasculitis Foundation Board of Directors
www.vasculitisfoundation.org