VASCULITIS FOUNDATION®

Early Diagnosis · Better Treatment · Improving Lives

October 5, 2026

U.S. Food and Drug Administration
White Oak Campus
10903 New Hampshire Ave.
Silver Spring, MD 20993

Attention:

- Kyle Diamantas, J.D., Acting Commissioner of Food and Drugs, U.S. Food and Drug Administration
- Michael Davis, MD, PhD, Director, Center for Drug Evaluation and Research, U.S. Food and Drug Administration
- Nikolay P. Nikolov, MD, Director, Office of Immunology and Inflammation, Office of New Drugs, U.S. Food and Drug Administration

RE: Docket No. FDA-2026-N-1321 — Request for a Public Hearing Regarding the Proposed Withdrawal of NDA 214487 for TAVNEOS® (avacopan)

Dear Commissioner Diamantas, Dr. Davis, and Dr. Nikolov:

The undersigned patient advocacy organizations, professional organizations, and other stakeholders supporting patients living with antineutrophil cytoplasmic antibody-associated vasculitis (AAV) respectfully request that the U.S. Food and Drug Administration (FDA) convene a **public hearing regarding the proposed withdrawal of NDA 214487 for TAVNEOS® (avacopan)** before the Agency reaches a final determination.

The proposed withdrawal raises issues of significant consequence for individuals living with granulomatosis with polyangiitis (GPA) and microscopic polyangiitis (MPA), their caregivers, healthcare providers, researchers, and the broader vasculitis community. We believe these issues warrant a transparent opportunity for meaningful public discussion and consideration of the full range of relevant evidence and perspectives.

A public hearing would provide an important opportunity to:

1. Fully examine the benefit-risk profile of avacopan
The benefit-risk assessment should consider not only the identified risks associated with avacopan, but also the risks, toxicities, limitations, and individual treatment burdens associated with available alternative therapies for severe GPA and MPA.

2. Hear directly from patients and caregivers
Individuals living with GPA and MPA and their caregivers should have an opportunity to present their perspectives, real-world experiences, treatment priorities, and risk tolerances directly to the Agency. Patient experience can provide important information about the practical consequences of treatment decisions that may not be fully captured by clinical trial endpoints alone.

3. Hear directly from clinicians and vasculitis specialists
A public hearing would allow the FDA to hear from clinicians with experience managing severe GPA and MPA regarding current treatment practices, clinical decision-making, patient-specific considerations, and the potential consequences of removing an available therapeutic option in the United States.

4. Consider the full body of relevant evidence
The Agency should have the opportunity to consider the available clinical, safety, and postmarketing evidence concerning avacopan, including evidence arising from real-world clinical use, relevant monitoring practices, and regulatory assessments in other jurisdictions.

5. Consider whether risk-management approaches could address identified concerns

A public hearing could facilitate discussion of whether additional risk-management measures—including updated labeling, enhanced liver monitoring protocols, restricted prescribing conditions, or other appropriate safeguards—could address identified safety concerns while preserving access for appropriate patients.

6. Provide clarity and transparency regarding the Agency's final determination
Given the uncertainty currently affecting patients and healthcare providers, it is important that the Agency's final decision and underlying benefit-risk determination be communicated clearly and transparently to patients, caregivers, clinicians, researchers, and the public.

As a matter of background, we believe it is important to stand true to the patient at this time and would like to highlight the real, patient-facing issues that are a part of this conversation.

The consequences of withdrawal are significant for patients
AAV is a chronic and potentially life-threatening autoimmune disease characterized by inflammation of blood vessels that can cause irreversible damage to vital organs, including the kidneys, lungs, upper respiratory tract, and nervous system. Renal involvement can progress rapidly to kidney failure and contributes substantially to morbidity and mortality.

Treatment for severe GPA and MPA commonly relies on immunosuppressive therapies, including cyclophosphamide or rituximab, frequently in combination with glucocorticoids. These therapies can be highly effective and remain essential to the care of many patients. At the same time, they carry serious and well-established risks, including severe infections, metabolic complications, osteoporosis, weight gain, adrenal suppression, and other effects that can significantly affect health and quality of life.

For people with severe GPA and MPA, treatment decisions therefore require balancing the need to control a potentially life-threatening disease against the risks and burdens of immunosuppressive therapy. Individuals respond differently to treatment and may have distinct risk factors, comorbidities, prior treatment experiences, and adverse effects that affect the suitability of particular therapies.

For these reasons, the benefit-risk assessment of avacopan should consider not only the risks associated with the product itself, but also **the potential harms and treatment burdens patients may face if this therapeutic option is no longer available.**

Patient perspectives should be heard directly
The FDA has recognized the importance of incorporating patient experience and patient preferences into regulatory decision-making, particularly for serious and rare diseases in which patients may face substantial disease-related and treatment-related burdens.

People living with AAV and their caregivers can provide important perspectives on questions such as:
- What does meaningful disease control look like in daily life?
- What treatment burdens are patients willing to accept to reduce the risk of organ damage or relapse?
- Which adverse effects have had the greatest impact on patients' health and quality of life?
- How do patients weigh the risks of available treatments against the risks of uncontrolled disease?
- How important is maintaining multiple therapeutic options when existing treatments carry substantial risks and burdens?

These perspectives should not replace scientific and clinical evidence. Rather, they should complement that evidence and form part of a comprehensive benefit-risk assessment.

The current uncertainty is affecting patients and healthcare providers
A thorough and timely public hearing is also important because uncertainty surrounding the proposed withdrawal is having practical consequences for the AAV community.

Patients currently receiving avacopan may be concerned about losing access to a treatment that has helped control their disease. Newly diagnosed patients and those considering treatment face uncertainty about whether avacopan remains an available option and how the proposed withdrawal may affect their treatment decisions.

Healthcare providers are facing uncertainty about how to counsel patients while the regulatory process remains unresolved. Changes in treatment may have significant consequences for individual patients, including the potential need to transition to therapies with different efficacy, safety profiles, monitoring requirements, or treatment burdens.

The international regulatory landscape also contributes to uncertainty when regulatory authorities in other countries evaluate related evidence differently or reach different conclusions. A transparent opportunity for discussion would help provide greater clarity for U.S. patients, caregivers, and clinicians.

A public hearing would serve the interests of patients and the broader AAV community
The undersigned organizations have a strong interest in ensuring that decisions affecting treatment options for people living with severe GPA and MPA are based on a complete, transparent, and fully informed record.

The proposed withdrawal of an approved therapy raises questions that extend beyond the safety profile of a single product. It also affects the treatment landscape available to people living with a serious and potentially life-threatening disease.

A public hearing would provide the FDA with an opportunity to hear directly from individuals living with or affected by AAV, caregivers, clinicians, researchers, and other stakeholders who can speak to the patient experience, clinical realities, unmet needs, available evidence, and potential approaches for managing identified risks while preserving access where appropriate.

Such a forum would help ensure that the Agency's final determination reflects the full range of relevant scientific, clinical, regulatory, and patient-centered considerations.

We respectfully request that the FDA convene a public hearing before reaching a final determination regarding the proposed withdrawal of NDA 214487 for TAVNEOS® (avacopan).
We welcome the opportunity to assist the FDA in identifying and facilitating participation by members of the AAV community, particularly patients and caregivers, who can provide direct and meaningful perspectives during this process.

Thank you for your consideration of this request and for your continued attention to the needs of people living with vasculitis.

Respectfully submitted,

The undersigned organizations
AiArthritis
American Behcet's Disease Association
CGD Association of America
DADA2 Foundation
Eosinophilic & Rare Disease Cooperative
Give an Hour
Global Healthy Living Foundation
Hope For Stomach Cancer
Michigan Rare Coalition
Myasthenia Gravis Association
Myasthenia Gravis Holistic Society
National Kidney Foundation
The Mended Hearts, Inc.
Vasculitis Foundation

Related Submissions
September 26, 2026: RE: Docket No. FDA-2026-N-1321 — Request for a Public Hearing Regarding the Proposed Withdrawal of NDA 214487 for TAVNEOS® (avacopan)

June 9, 2026 — Comment Tracking Number: mq6-sxee-pvb9