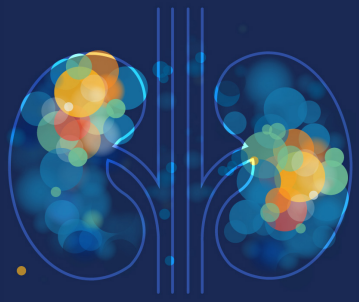
Case Study:

DELIVERING THE ONLY APPROVED THERAPY FOR ANCA-ASSOCIATED VASCULITIS



BACKGROUND

ANCA-associated vasculitis (AAV) is a rare autoimmune disease that causes inflammation in small to medium-sized blood vessels, often leading to damage across multiple organs such as the kidneys and lungs. Because symptoms vary widely – from fatigue and nosebleeds to respiratory issues – diagnosis is often delayed, and patients often require urgent treatment once identified. As a result, clinical trials in this space are notoriously difficult when it comes to patient recruitment and trial timing.

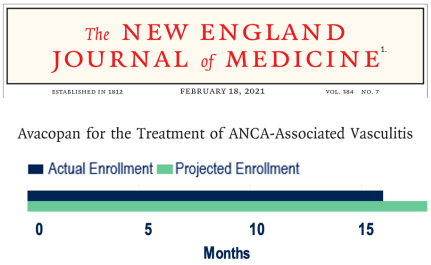
The following case study outlines the strategies Medpace took in a pivotal global Phase III trial that led to the FDA, EMA, MHRA and PMDA approval of the first and only small molecule therapy for AAV on the market.

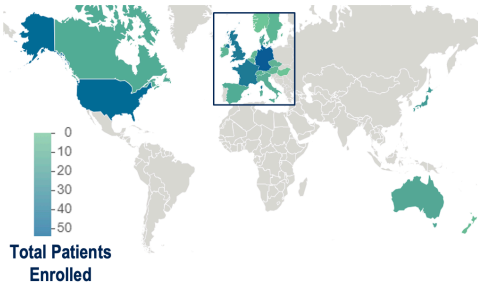
STUDY OVERVIEW

331
PATIENTS ENROLLED

20
COUNTRIES

239
SITES ACTIVATED





1. Jayne DRW, et al. Avacopan for the Treatment of ANCA-Associated Vasculitis. N Engl J Med. 2021;384:599-609. doi:10.1056/NEJMoa2023386.

RESULTS

- **Largest AAV Study to Date:** This Phase III trial remains the largest global study ever conducted in ANCA-associated vasculitis.
- **Early Enrollment Completion:** Despite the rarity and complexity of the disease, the study completed enrollment ahead of schedule across 20 countries and 239 sites.
- **Regulatory Success:** The study directly supported the FDA, EMA, MHRA and PMDA approval of Avacopan, making it the only therapy currently approved for AAV.

STUDY CHALLENGES

- **Rare and Acute Population:** AAV patients often require immediate in-patient treatment upon diagnosis or relapse, disqualifying them from study eligibility if treated with prohibited medications before enrollment. Many eligible patients became ineligible due to immediate administration of steroids or immunosuppressants by unaware providers.
- **Decentralized Referral Pathways:** Patients often see multiple specialists (e.g., primary care, pulmonology, nephrology, rheumatology) before receiving an accurate diagnosis, complicating site referral and recruitment.
- **Complex Clinical Assessments:** Primary endpoints relied on the Birmingham Vasculitis Activity Score (BVAS) and Vasculitis Damage Index (VDI), which are complex, subjective, and require standardized interpretation across all global sites.

MEDPACE SOLUTIONS

- **Enhanced Site Communication & Referral Pathways:** Medpace utilized their industry leading collaboration model with sites to ensure patients were enrolled before disqualifying treatments were administered. Educational materials were also provided to physicians to help improve their early recognition of AAV symptoms.
- **Close Medical Monitor/CRA & Site Collaboration:** Medpace in-house nephrologists and CRAs maintained real-time communication with sites and utilized our enhanced technology to flag potential patients and expedite screening and randomization before treatment delays or exclusions occurred.
- **Expert-Led BVAS/VDI Scientific Education:** Medpace collaborated with global KOLs to develop a continuous education strategy – ensuring consistent BVAS/VDI scoring across regions. Customized interactive case studies and certification ensured scoring accuracy and clinical rigor across all global sites – essential for endpoint protection.

AN INDUSTRY LEADER IN NEPHROLOGY CLINICAL RESEARCH

With decades of experience conducting phase I-IV nephrology clinical trials, Medpace is a globally recognized leader in nephrology clinical research. Our seasoned medical in-house nephrologists, operational, and regulatory teams are fully embedded throughout your project – providing unmatched support for your program. Combined with our strong site network and KOL relationships to accelerate development and enhance patient recruitment, we've successfully led Phase III studies (several to regulatory approval) in key indications such as acute kidney injury, chronic kidney disease, glomerulonephritis, genetic and rare kidney diseases, and renal transplantation.

MAKING THE COMPLEX SEAMLESS®