Efficacy and Safety Experience with Avacopan Beyond 52 Weeks in the Early Access Program (EAP)

To report on the safety and efficacy of avacopan in the treatment of ANCA-associated vasculitis (AAV) beyond 52 weeks using data from the Early Access Program (EAP)

INTRODUCTION

• Avacopan, a selective complement 5a receptor 1 (C5aR1) inhibitor, has demonstrated efficacy and safety over 52 weeks in patients with ANCA-associated vasculitis (granulomatosis with polyangiitis [GPA] and microscopic polyangiitis [MPA]) in the ADVOCATE Phase III trial (NCT02994927)

• Previously published experience in a real-world setting, based on 30 patients in the avacopan EAP (average treatment duration 11.5 months), has shown the safety profile of avacopan to be consistent with the clinical trial results. However, safety and efficacy data on avacopan beyond 52 weeks are limited

• Here, we describe the experience with avacopan beyond 52 weeks using data from the EAP

METHODS

• Safety data in patients with severe, active GPA or MPA within the EAP were recorded in a global pharmacovigilance database from Feb 2019 to Apr 2023

• All cases were received as solicited reports, which are defined as those obtained from an organized data collection method and were medically reviewed for clinical content, including medical history, concomitant medications, and possible causal relationship of adverse event (AE) to drug use

• All physicians agreed to report AEs and adverse drug reactions (ADRs) using the WHO-UMC system for standardized care causality assessment

• Definitions of AEs and serious adverse events (SAEs) were used according to the EMA guidelines, including lack of effect and relapse/worsening of disease

• Physicians agreed to report AEs and adverse drug reactions (ADRs) and the database received continuous updates on AEs from all avacopan studies as part of Good Clinical Practice (GCP) regulations

• Additional measures were undertaken to minimize the risk of under-reporting of AEs, including safety training and reminders

RESULTS

Figure 1. Study characteristics. Total patient number = 19

Table 1. Overview of Safety Events by System Order Class (preferred term)

<table>
<thead>
<tr>
<th>System Order Class</th>
<th>Preferred Term*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Musculoskeletal and connective tissue disorders</td>
<td>Psoriatic arthritis</td>
</tr>
<tr>
<td>Vascular disorders</td>
<td>Vasculitis</td>
</tr>
<tr>
<td>Surgical and medical procedures</td>
<td>Therapy interrupted</td>
</tr>
<tr>
<td>Infection, poisoning, and procedural complications</td>
<td>Product dose omission issue</td>
</tr>
<tr>
<td>Product issues</td>
<td>Product supply issue</td>
</tr>
<tr>
<td>General disorders and administration site conditions</td>
<td>Palp</td>
</tr>
<tr>
<td>General disorders and administration site conditions</td>
<td>Malaise</td>
</tr>
</tbody>
</table>

FAQ: Definition: a distinct descriptor (single medical concept) for a symptom, sign, disease diagnosis, therapeutic indication, investigation, surgical or medical procedure, and medical, social or family history characteristic

ADDITIONAL INFORMATION

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REFERENCES


ABBREVIATIONS

AAV, anti-neutrophil cytoplasmic antibody; ADR, adverse drug reaction; AEs, adverse events; ANCA, anti-neutrophil cytoplasmic antibody; BID, twice daily; C5aR1, C5a receptor 1; COVID-19; CV, cardiovascular; GPA, granulomatosis with polyangiitis; GCP, Good Clinical Practice; GPA, granulomatous anti-neutrophil cytoplasmic antibody; MPA, microscopic polyangiitis; SAE, serious adverse event; WHO-UMC, World Health Organization, Uppsala Monitoring Centre.

STUDY LIMITATIONS

• Low patient number
• Potential under-reporting
• Incomplete data

CONCLUSIONS

These results suggest that the continuation of avacopan treatment beyond the 52 weeks reported in the ADVOCATE trial is generally well-tolerated in patients with GPA and MPA

No treatment discontinuations due to AEs were reported across the study with 17 out of 19 patients (89.5%) reporting no AEs

Avacopan may be effective in terms of disease control beyond 52 weeks.