

Axatilimab: Adis Evaluation

Clinical Considerations

- **Anti-colony stimulating factor 1 receptor monoclonal antibody with a novel macrophage-driven mechanism**
- **Improves patient condition and reduces patient-reported symptom severity**
- **Generally manageable tolerability profile; infections are the most common adverse events**

Plain Language Summary

Background and rationale

- Chronic graft-versus-host disease (cGVHD) is a major cause of morbidity and non-relapse mortality in patients who have undergone allogeneic hematopoietic stem-cell transplantation
- Over half of cGVHD patients do not achieve durable responses with first-line corticosteroids, and response rates with newer agents vary, with many patients experiencing disease progression and end-organ damage
- Axatilimab (axatilimab-csfr; Niktimvo®), an anti-colony stimulating factor 1 receptor monoclonal antibody, was recently approved in the USA for treatment of cGVHD in patients weighing ≥ 40 kg who have failed $\geq$ two prior lines of systemic therapy

Clinical findings

- In a phase 2 clinical trial, axatilimab 0.3 mg/kg once every 2 weeks improved patient condition and reduced patient-reported symptom severity
- Improvements were reported across all affected organs, including those with difficult-to-treat fibrotic manifestations
- The tolerability of axatilimab at the approved dosage was generally manageable; infections were the most common adverse events
- Changes in laboratory abnormalities were temporary and not associated with end-organ damage

Conclusion

Axatilimab is a useful addition to treatment options in the USA for patients with recurrent or refractory cGVHD who have failed $\geq$ two prior lines of systemic therapy

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