

Adzynma™ (ADAMTS13 recombinant-krhn)

When requesting Adzynma (ADAMTS13 recombinant-krhn), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved indication

Adzynma is indicated for prophylactic or on-demand enzyme replacement therapy (ERT) in adult and pediatric patients with congenital thrombotic thrombocytopenic purpura (cTTP).

Coverage Guidelines

Congenital Thrombotic Thrombocytopenic Purpura

The individual must meet all of the following criteria for initial authorization:

- At baseline is ADAMTS13 activity is less than 10% (< 10 IU/dL);
Note: baseline refers to before any treatment, such as Adzynma or plasma-based therapies.
- Does not have anti-ADAMTS13 autoantibodies;
- Has a pathogenic variant or mutation in the ADAMTS13 gene;
Note: pathogenic variants or gene mutations are usually homozygous or compound heterozygous.
- Adzynma is prescribed by or in consultation with a hematologist.

Approval duration: 12 months

Dosing Recommendations

Prophylactic Therapy: Administer 40 IU/kg body weight once every other week intravenously. Dosing frequency may be adjusted to 40 IU/kg once weekly based on prior prophylactic regimen or clinical response.

On-Demand Therapy:

- Day 1: 40 IU/kg
- Day 2: 20 IU/kg
- Day 3 and beyond until two days after the acute event is resolved: 15 IU/kg

References

1. Adzynma™ intravenous infusion [prescribing information]. Lexington, MA: Takeda; November 2023.
2. Food and Drug Administration News Release. FDA approves first treatment for patients with rare inherited blood clotting disorder. November 9, 2023. Available at: <https://www.fda.gov/news-events/press-announcements/fda-approves-first-treatment-patients-rare-inherited-blood-clotting-disorder>. Accessed on November 26, 2023.

3. Kremer Hovingo JA, George JN. Hereditary thrombotic thrombocytopenic purpura. *N Engl J Med*. 2019;381:1653-1662.
4. Zheng XL, Vesely SK, Cataland SR, et al. International Society on Thrombosis and Haemostasis (ISTH) guidelines for the treatment of thrombotic thrombocytopenic purpura. *J Thromb Haemost*. 2020;18:2496-2502.
5. Scully M, Rayment R, Clark A, et al, on behalf of the BSH Committee. A British Society of Haematology Guideline: diagnosis and management of thrombotic thrombocytopenia purpura and thrombotic microangiopathies. *Br J Haematol*. 2023;203:546-563.

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