CARECONTINUUM™

UTILIZATION MANAGEMENT MEDICAL POLICY

POLICY: Hematology – Adzynma Utilization Management Medical Policy
- Adzynma® (ADAMTS13 recombinant-krhn intravenous infusion – Takeda)

REVIEW DATE: 08/26/2026

OVERVIEW

Adzynma, a human recombinant "A disintegrin and metalloproteinase with thrombospondin motifs 13" (ADAMTS13) product, is indicated for prophylactic or on demand enzyme replacement therapy for the management of congenital thrombotic thrombocytopenia purpura (TTP) in adults and pediatric patients.[1]

Disease Overview

Congenital TTP is a very rare, inherited blood clotting disorder (prevalence of 0.5 to 2 cases per million persons).[2-4] It is due to a mutation in the *ADAMTS13* gene that makes a key enzyme, also named ADAMTS13, that regulates blood clotting. The condition results from severe hereditary deficiency (< 10% of normal activity) of ADAMTS13. The deficiency in this enzyme causes blood clots to form in the small blood vessels throughout the body. Symptoms typically start in infancy or early childhood, but in some cases may develop in adulthood and can initially manifest during pregnancy. Patients with congenital thrombotic thrombocytopenic purpura may experience severe bleeding episodes, strokes, and damage to vital organs. The condition can be fatal if not managed properly.

Guidelines

The International Society on Thrombosis and Haemostasis (ISTH) performed a focused update of the 2020 ISTH guidelines for the management of TTP (2025).[5] For patients with congenital TTP who are in remission, Adzynma is recommended over plasma infusion to prevent acute episodes.

The British Society of Haematology published guidelines for the diagnosis and management of TTP and thrombotic microangiopathies (2023).[6] The diagnosis of congenital TTP is defined by ADAMTS13 activity < 10 IU/dL, no anti-ADAMTS13 antibodies, and confirmation of homozygous or compound heterozygous variants in the *ADAMTS13* gene. For an acute episode, solvent detergent plasma infusion is recommended. ADAMTS13 prophylaxis should be considered for all patients with an individualized approach to dose and frequency according to symptoms, whether overt or non-overt.

POLICY STATEMENT

Prior Authorization is recommended for medical benefit coverage of Adzynma. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indication. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). Because of the specialized skills required for evaluation and diagnosis of patients treated with Adzynma as well as the monitoring required for adverse events and long-term efficacy, approval requires Adzynma to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Documentation: Documentation is required for use of Adzynma as noted in the criteria as **[documentation required]**. Documentation may include but is not limited to chart notes, laboratory data, genetic test results, and/or other information. All documentation must include patient-specific identifying information.

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Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Adzynma is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Congenital Thrombotic Thrombocytopenic Purpura.** Approve for 1 year if the patient meets ALL of the following (A, B, C, and D):
   - **A)** At baseline (prior to therapy) ADAMTS13 activity is < 10% (< 10 IU/dL) **[documentation required]**; AND
     Note: Baseline refers to before any treatment was received, such as Adzynma or plasma-based therapies.
   - **B)** Patient does not have anti-ADAMTS13 autoantibodies as determined by a diagnostic test **[documentation required]**; AND
   - **C)** Patient has a pathogenic variant in the *ADAMTS13* gene **[documentation required]**; AND
     Note: Pathogenic variants are usually homozygous or compound heterozygous.
   - **D)** Medication is prescribed by or in consultation with a hematologist.

   Dosing. Approve the following dosing regimens (A and/or B):
   - **A)** Routine prophylaxis: approve up to 40 IU/kg by intravenous infusion once weekly; AND/OR
   - **B)** On demand therapy: approve up to 135 IU/kg by intravenous infusion per week as needed for the treatment of acute event(s).
     Note: On demand therapy is given as a daily dose until 2 days after the acute event resolves; however, the total weekly dose should not exceed 135 IU/kg.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Adzynma is not recommended in the following situations.

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Adzynma® intravenous infusion [prescribing information]. Lexington, MA: Takeda; June 2024.
2. Scully M, Antun A, Cataland SR, et al. Recombinant ADAMTS13 in congenital thrombotic thrombocytopenic purpura. *N Engl J Med*. 2024;390:1584-1596.
3. Rodgers GM. Update in treatment options for congenital and immune thrombotic thrombocytopenia purpura. *Front Pharmacol*. 2026;17:1841673.
4. Scully M, Carter MA, Subhan M. Thrombotic thrombocytopenic purpura: celebrating 25 years of ADAMTS13. *Blood*. 2026;147(22):2582-2591.
5. Zheng XL, Al-Housni Z, Cataland SR, et al, on behalf of the International Society on Thrombosis and Haemostasis. 2025 focused update of the 2020 ISTH guideline for management of thrombotic thrombocytopenic purpura. *J Thromb Haemost*. 2025;23(11):3711-3732.
6. 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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HISTORY

Type of Revision	Summary of Changes	Review Date
Annual Revision	No criteria changes.	12/04/2024
Annual Revision	No criteria changes.	12/10/2025
Early Annual Revision	**Congenital Thrombotic Thrombocytopenic Purpura:** Regarding the criterion which states that the patient has a pathogenic variant in the *ADAMTS13* gene (documentation required), the phrase "or a mutation" was removed. In the related Note, the phrase "or gene mutations" was also removed.	08/26/2026

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