

Clinical Policy: ADAMTS13, Recombinant-krhn (Adzynma)

Reference Number: PA.CP.PHAR.635

Effective Date: 02/2024

Last Review Date: 01/2026

Description

ADAMTS13, recombinant-krhn (Adzynma[®]) is a human recombinant “A disintegrin and metalloproteinase with thrombospondin motifs 13” (rADAMTS13).

FDA Approved Indication(s)

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

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness[®] that Adzynma is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Congenital Thrombotic Thrombocytopenic Purpura (must meet all):

1. Diagnosis of severe cTTP confirmed by all of the following (a, b, and c):
 - a. Genetic test confirming biallelic ADAMTS13 mutation;
 - b. ADAMTS13 activity < 10 % of normal, unless member is currently receiving prophylactic plasma therapy;
 - c. One of the following (i or ii):
 - i. Absence of ADAMTS13 functional inhibitor;
 - ii. Absence of anti-ADAMTS13 antibodies;
2. Prescribed by or in consultation with a hematologist;
3. Age ≥ 2 years;
4. For prophylaxis requests, member has TTP signs or symptoms that are persistent or recurrent (*see Appendix D*);
5. For acute (on demand) treatment requests, member has an acute TTP event defined by both the following (a and b):
 - a. Platelet count < 100,000/μL or a drop in platelet count ≥ 50% of the baseline platelet count;
 - b. Microangiopathic hemolytic anemia with a lactate dehydrogenase (LDH) elevation greater than two times the baseline or two times the upper limit of normal;
6. Documentation of member’s current body weight (in kg);
7. Dose does not exceed the following (a or b):
 - a. For prophylactic therapy: 40 IU/kg once weekly;
 - b. For acute treatment: 40 IU/kg on day 1, followed by 20 IU/kg on day 2, and 15 IU/kg/day until two days after the acute event is resolved.

Approval duration:

Prophylaxis: 12 months
Acute treatment: Up to 2 weeks per acute episode

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. Congenital Thrombotic Thrombocytopenic Purpura (must meet all):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies;
2. Member is responding positively to therapy as evidenced by, including but not limited to, improvement in any of the following parameters (a-c):
 - a. Thrombocytopenia;
 - b. Microangiopathic hemolytic anemia;
 - c. Symptom improvement (e.g., less headaches, lethargy, and/or abdominal pain);
3. Documentation of member's current body weight (in kg);
4. If request is for a dose increase, new dose does not exceed the following (a or b):
 - a. For prophylactic therapy: 40 IU/kg once weekly;
 - b. For acute treatment, both (i and ii):
 - i. 40 IU/kg on day 1, followed by 20 IU/kg on day 2, and 15 IU/kg/day until two days after the acute event is resolved;
 - ii. If request exceeds 2 weeks of treatment: provider justification for continued acute dosing.

Approval duration:

Prophylaxis: 12 months

Acute treatment: Up to 2 weeks per acute episode

B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies.

Approval duration: Duration of request or 12 months (whichever is less); or

2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

cTTP: congenital thrombotic thrombocytopenic purpura
 FDA: Food and Drug Administration
 LDH: lactate dehydrogenase

rADAMTS13: a disintegrin and metalloproteinase with thrombospondin motifs 13

Appendix B: Therapeutic Alternatives
 Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): life threatening hypersensitivity reactions to Adzynma or its components
- Boxed warning(s): none

Appendix D: General Information

- Examples of TTP signs and symptoms include, but are not limited to, persistent thrombocytopenia, recurrent microangiopathic hemolytic anemia, proteinuria, stroke, transient ischemic attack, lethargy, headaches, loss of concentration, and abdominal discomfort.
- Treatment for an acute TTP episode depends on the duration of an episode. Per the International Hereditary Thrombotic Thrombocytopenic Purpura Registry (van Dorland et al, 2019), the median duration of an acute episode was seven days. Per the Adzynma pivotal trial, the duration of an acute episode for Adzynma treatment ranged from 2-4 days.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
cTTP	<p><i>Prophylactic therapy</i> 40 IU/kg IV every 2 weeks. Dosing frequency may be adjusted to 40 IU/kg body weight once weekly based on prior prophylactic dosing regimen or clinical response.</p> <p><i>On-demand therapy</i> 40 IU/kg IV on day 1, followed by 20 IU/kg IV on day 2, and then 15 IU/kg/day on day 3 and beyond until two days after the acute event is resolved. Dose is based on body weight.</p>	<p>Prophylactic therapy: 40 IU/kg/week</p> <p>On-demand therapy: 40 IU/kg/day</p>

VI. Product Availability

Lyophilized powder in single-dose vials: 500 IU, 1,500 IU

VII. References

1. Adzynma Prescribing Information. Lexington, MA: Takeda Pharmaceuticals USA, Inc.; June 2024. Available at: <https://www.adzynma.com/>. Accessed October 23, 2025.
2. Scully M, Antun A, Cataland SR, et al. Recombinant ADAMTS13 in congenital thrombotic thrombocytopenic purpura. N Engl J Med. 2024;390(17):1584-1596.

3. Alwan F, Vendramin C, Liesner R, et al. Characterization and treatment of congenital thrombotic thrombocytopenic purpura. *Blood*. 2019;133(15):1644-1651.
4. Scully M, Cataland S, Coppo P, et al. Consensus on the standardization of terminology in thrombotic thrombocytopenic purpura and related thrombotic microangiopathies. *J Thromb Haemost*. 2017;15(2):312-322.
5. van Dorland HA, Taleghani MM, Sakai K, et al. The International Hereditary Thrombotic Thrombocytopenic Purpura Registry: Key findings at enrollment until 2017. *Haematologica*. 2019;104(10):2107-2115.
6. Kremer Hovinga JA, George JN. Hereditary thrombotic thrombocytopenic purpura. *N Engl J Med*. 2019;381(17):1653-1662.
7. Scully M, Rayment R, Clark A, et al. A British Society for Haematology Guideline: Diagnosis and management of thrombotic thrombocytopenic purpura and thrombotic microangiopathies. *Br J Haematol*. 2023;203(4):546-563.
8. Zheng XL, Al-Housni Z, Cataland SR, et al. 2025 focused update of the 2020 ISTH guidelines for management of thrombotic thrombocytopenic purpura. *J Thromb Haemost*. 2025;23(11):3711-3732.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J7171	Injection, adamts13, recombinant-krhn, 10 iu

Reviews, Revisions, and Approvals	Date
Policy created	01/2024
1Q 2025 annual review: no significant changes; added HCPCS code [J7171] and removed HCPCS codes [J3590, C9399]; references reviewed and updated.	01/2025
1Q 2026 annual review: removed requirement for plasma therapy failure per updated guideline; revised initial approval duration for prophylaxis to 12 months; references reviewed and updated.	01/2026