Adzynma® (ADAMTS13, recombinant-krhn)
(Intravenous)

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I. Length of Authorization [1]

Prophylaxis Therapy

- Coverage will be provided for 6 months and may be renewed annually thereafter.

On-Demand Therapy

- Coverage is provided for 3 months and is eligible for renewal.

  Note: The cumulative amount of medication the patient has on-hand, indicated for the acute treatment of TTP events, will be considered for authorizations. The authorization will provide a sufficient quantity in order for the patient to have a cumulative amount of medication on-hand in order to treat up to 4 acute attacks per 4 weeks for the duration of the authorization.

II. Dosing Limits

Max Units (per dose and over time) [HCPCS Unit]:

- 1800 billable units every 28 days

III. Initial Approval Criteria [1]

Coverage is provided in the following conditions:

- Patient is at least 2 years of age; **AND**
- Patient has not been diagnosed with other cTTP like disorders (e.g., acquired TTP, immune TTP, other primary thrombotic microangiopathies, immune thrombocytopenia, Evans Syndrome, etc.); **AND**
- Patient does not have a medical history or the presence of functional ADAMTS13 inhibitors prior to the start of therapy; **AND**

Universal Criteria [1-3]

- Patient does not have a known sensitivity to hamster protein; **AND**

Congenital Thrombotic Thrombocytopenic Purpura (cTTP) † Φ [1-9]

- Patient has a documented diagnosis of severe hereditary ADAMTS13 deficiency, defined as confirmed by molecular genetic testing, documenting biallelic pathogenic variants in *ADAMTS13*; **AND**
- Patient has an ADAMTS13 activity of < 10 % as measured by the fluorescent resonance energy transfer- von Willebrand factor73 (FRETS-VWF73) assay *(Note: Patients currently receiving prophylactic plasma infusion therapy may exceed 10% ADAMTS13 activity at start of therapy)*; **AND**
    - Treatment will be used as prophylactic therapy; **AND**
        - Patient has a history of at least one TTP event or is currently receiving prophylactic plasma infusion therapy *(Note: Patients who have been receiving prophylactic plasma-based therapies should discontinue routine use of those therapies after achieving a therapeutic response)*; **OR**
    - Treatment will be used as on-demand therapy and patient is at risk of a disease exacerbation

† FDA Approved Indication(s); **‡** Compendium Recommended Indication(s); **Φ** Orphan Drug

IV. Renewal Criteria [1]

Coverage may be renewed based upon the following criteria:

- Patient continues to meet the universal and other indication-specific relevant criteria identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: severe hypersensitivity or anaphylactic reactions, etc.; **AND**
- Patient has responded to therapy compared to pre-treatment baseline with the following:
    - If symptomatic, improvement in the signs and symptoms of disease (e.g., neurological symptoms including confusion, dysphonia, dysarthria, focal or general motor symptoms, seizures; renal dysfunction, TTP-related pain, etc.); **AND**

Prophylaxis:

- Patient has a reduction in or an absence of an acute TTP event, defined by a drop in platelet count (≥50% of baseline or a platelet count <100,000/µL) and an elevation of lactate dehydrogenase (LDH) (>2× baseline or >2× upper limit normal (ULN)); **OR**
- Patient has a reduction in or an absence of a sub-acute TTP event, defined by a thrombocytopenia event or a microangiopathic hemolytic anemia event; and organ-

specific signs and symptoms including but not limited to renal dysfunction events, neurological symptoms events, fever, fatigue/lethargy, and/or abdominal pain; **OR**

On-Demand*:

- Patient has responded to an acute TTP event with therapy as evidenced by improvement in thrombocytopenia *(defined as a drop in platelets ≥25% of baseline or a platelet count less than <150,000/mcg/L)* or in microangiopathic hemolytic anemia *(defined as an elevation of LDH > 1.5 times of baseline or >1.5 times ULN)*

  **Note: The cumulative amount of medication the patient has on-hand, indicated for the acute treatment of TTP events, will be considered for authorizations. The authorization will provide a sufficient quantity in order for the patient to have a cumulative amount of medication on-hand in order to treat up to 4 acute attacks per 4 weeks for the duration of the authorization (unless otherwise specified).*

V. Dosage/Administration [1]

Indication	Dose
Congenital Thrombotic Thrombocytopenic Purpura (cTTP)	**Prophylactic Therapy** The recommended prophylactic dosage regimen is as follows: – Administer 40 IU/kg body weight once every other week. *Note: The prophylactic dosing frequency may be adjusted to 40 IU/kg body weight once weekly based on prior prophylactic dosing regimen or clinical response.* **On-Demand Therapy** A guide for dosing Adzynma for on-demand treatment of an acute event is as follows: – Treatment Day 1: 40 IU/kg – Treatment Day 2: 20 IU/kg – Treatment Day 3 and beyond: 15 IU/kg once daily until two days after the acute event is resolved.
– Each vial of Adzynma is labeled with the actual rADAMTS13 activity, measured in terms of its potency in International Units (IU). – Calculate administration dose and volume based on the patient's body weight using the actual potency (and not the nominal potency) as printed on Adzynma vial. – For Intravenous (IV) Infusion at a rate of 2 to 4 mL per minute.	

VI. Billing Code/Availability Information

HCPCS code:

- J7171 – Injection, adamts13, recombinant-krhn, 10 iu; 1 billable unit = 10 units

NDC:

- Adzynma 500 IU single-dose vial for injection: 64764-0130-xx
- Adzynma 1500 IU single-dose vial for injection: 64764-0135-xx

VII. References

1. Adzynma [package insert]. Lexington, MA; Takeda Pharm. USA, Inc.; June 2024. Accessed February 2025.
2. Asmis, L. M., Serra, A., Krafft, A., Licht, A., Leisinger, E., Henschkowski-Serra, J., et al. 2022. Recombinant ADAMTS13 for Hereditary Thrombotic Thrombocytopenic Purpura. N Engl J Med, 387, 2356-2361.
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4. Sukumar, S., B. Lämmle and S. R. Cataland (2021). "Thrombotic Thrombocytopenic Purpura: Pathophysiology, Diagnosis, and Management." J Clin Med 10(3): 536.
5. Zheng XL, Vesely SK, Cataland SR, et al. ISTH guidelines for treatment of thrombotic thrombocytopenic purpura. J Thromb Haemost. 2020;18(10):2496-2502. Doi:10.1111/jth.15010.
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8. George JN, Cuker A(Feb 2025). Hereditary thrombotic thrombocytopenic purpura (hTTP). Crowther M, Tirnauer JS (Eds.). *UpToDate*. Accessed February 24, 2025. Available from: https://www.uptodate.com/contents/hereditary-thrombotic-thrombocytopenic-purpura-http
9. Scully M, Antun A, Cataland SR, et al. 2024 "Recombinant ADAMTS13 in Congenital Thrombotic Thrombocytopenic Purpura" N Engl J Med. 2024 May 2;390(17):1584-1596. doi: 10.1056/NEJMoa2314793.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
D69.42	Congenital and hereditary thrombocytopenia purpura

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC