RX.PA.115.MPC Adzynma (ADAMTS13, recombinant-krhn)

The purpose of this policy is to define the prior authorization process for Adzynma.

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

PROCEDURE
A. Initial Authorization Criteria
CLINICAL CRITERIA (*Use for ALL Drug Requests*)
Must meet all of the criteria listed under the respective product:

1. **Congenital Thrombotic Thrombocytopenic Purpura (cTTP)**

- Must be ≥ 2 years of age
- Must have a documented diagnosis of severe hereditary ADAMTS13 deficiency confirmed by the following:
    - Molecular genetic testing demonstrating biallelic pathogenic variants in ADAMTS13

      AND

    - ADAMTS13 activity of < 10 % as measured by the fluorescent resonance energy transfer- von Willebrand factor73 (FRETS-VWF73) assay
- Must have documentation to confirm the patient doesn't have a medical history or presence of a functional ADAMTS13 inhibitor prior to starting treatment
- Provider must attest that the patient does not have other diagnoses with other cTTP like disorders (e.g., acquired TTP, immune TTP, other primary thrombotic microangiopathies, immune thrombocytopenia, Evans Syndrome, etc.)
- Patient must have a documented trial and failure for at least 3 months or contraindication to fresh frozen plasma (FFP) Infusion prior to requesting treatment
- Must be prescribed by or in consultation with a hematologist

Prophylactic Therapy

- Must have documentation confirming patient has a history of at least 1 TTP event
- Must have documentation within the last 3 months confirming that the patient does not have signs of severe TTP defined as:
    - Platelet count < 100,000/µL
    - Lactate dehydrogenase (LD) > 2x upper limit normal (ULN)

On-Demand Therapy

- Must have documentation within the last 3 months confirming an acute TTP event defined by the following:
    - Drop in platelet count ≥ 50% of baseline or a platelet count of < 100,000/µL
    - Lactate dehydrogenase (LDH) > 2x baseline value or > 2x upper limit normal (ULN)

B. <u>Reauthorization Criteria:</u>
MPC Renewal: Coverage may be renewed based upon the following criteria:

- Documentation from the prescriber that patient had a clinically significant response to treatment compared to pre-treatment baseline
    - Improvement in signs and symptoms of the disease (confusion, dysphonia, dysarthria)
    - Improvement in general motor symptoms, renal dysfunction or TTP-related pain

AND

1. **Prophylaxis:**

- Patient has a reduction in or an absence of an acute TTP event
    - Note: acute TTP event is defined as a drop in platelet count (≥50% of baseline a platelet count 2× baseline or >2× upper limit normal (ULN)

OR

- Patient has a reduction in or an absence of a sub-acute TTP event

- Note: sub-acute TTP event is defined as a thrombocytopenia event, (i.e. platelet count < 150 × 10^3 per µL or a microangiopathic hemolytic anemia event (i.e. evidence of hemolysis and red blood cell fragmentation); and organ specific signs and symptoms including but not limited to renal dysfunction events, neurological symptoms events, fever, fatigue/lethargy, and/or abdominal pain

2. 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 1.5 times of baseline or >1.5 times ULN)

OR

C. <u>Renewal from Previous Insurer:</u>

- Members who have received prior approval (from insurers other than MPC), or have been receiving medication samples, should be considered under criterion A (Initial Authorization Criteria)

- Provider has a documented clinical response of the member's condition which has stabilized or improved based upon the prescriber's assessment

Dosing Table	
Indication	**Dosing**
Prophylactic Therapy	• Administer 40 IU/kg body weight once every other week intravenously at a rate of 2 to 4 mL per minute.
On-Demand Therapy	• 40 IU/kg body weight on day 1. • 20 IU/kg body weight on day 2. • 15 IU/kg body weight on day 3 and beyond until two days after the acute event is resolved.

Please note: The prophylaxis dosing frequency may be adjusted to 40 IU/kg body weight once weekly based on prior prophylactic dosing regimen or clinical response

<u>Limitations:</u>

Length of Authorization (if above criteria met)	
Initial Authorization	Prophylactic Therapy 3 Months On Demand Therapy 1 Month
Reauthorization	Prophylactic Therapy: 6 Months On Demand Therapy: 3 Months

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Applicable Codes:

CODE	DESCRIPTION
J7171	Injection, ADAMTS13, recombinant-krhn, 10 IU

REFERENCES

1. Adzynma [package insert]. Lexington, MA; Takeda Pharm. USA, Inc.; June 2024. Accessed September 2025.
2. Asmis LM, Serra A, Krafft A, et al. Recombinant ADAMTS13 for Hereditary Thrombotic Thrombocytopenic Purpura. N Engl J Med 2022; 387: 2356-2361.
3. ClinicalTrials.gov. A Phase 3, Prospective, Randomized, Controlled, Open-label, Multicenter, 2 Period Crossover Study With a Single Arm Continuation Evaluating the Safety And Efficacy of BAX 930 (rADAMTS13) in the Prophylactic And On-demand Treatment of Subjects With Severe Congenital Thrombotic Thrombocytopenic Purpura (cTTP, Upshaw-Schulman Syndrome [USS], Hereditary Thrombotic Thrombocytopenic Purpura [hTTP]). https://clinicaltrials.gov/study/NCT03393975?intr=tak-755&rank=4
4. ClinicalTrials.gov. A Phase 3b, Prospective, Open-label, Multicenter, Single Treatment Arm, Continuation Study of the Safety and Efficacy of TAK-755 (rADAMTS13, Also Known as BAX 930/SHP655) in the Prophylactic and On-demand Treatment of Subjects With Severe Congenital Thrombotic Thrombocytopenic Purpura (cTTP; Upshaw-Schulman Syndrome, or Hereditary Thrombotic Thrombocytopenic Purpura). https://clinicaltrials.gov/study/NCT04683003?intr=tak755&rank=1

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual Review	*02/2026*
New Policy	*11/2025*