

PHARMACY POLICY STATEMENT

Georgia Medicaid

DRUG NAME	Adzynma (ADAMTS13, recombinant-krhn)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Adzynma, approved by the FDA in 2023, is a human recombinant “A disintegrin and metalloproteinase with thrombospondin motifs 13” (rADAMTS13) indicated for prophylactic or on demand enzyme replacement therapy (ERT) in adult and pediatric patients with congenital thrombotic thrombocytopenic purpura (cTTP). Adzynma is the first treatment approved for cTTP. Hereditary TTP is much less common than acquired/immune TTP.

Endogenous ADAMTS13 regulates the activity of von Willebrand factor (VWF) by cleaving it into smaller units to reduce its platelet binding properties and microthrombi formation. TTP results from a deficiency of ADAMTS13 which leads to increased circulation of VWF causing clotting and red blood cell destruction. Signs and symptoms can include thrombocytopenia, hemolytic anemia, neurological symptoms, and a skin rash. Plasma infusions have been the main treatment for cTTP, to provide sufficient ADAMTS13.

Adzynma (ADAMTS13, recombinant-krhn) will be considered for coverage when the following criteria are met:

Congenital Thrombotic Thrombocytopenic Purpura (cTTP)

For **initial** authorization:

1. Medication must be prescribed by or in consultation with a hematologist; AND
2. Member has a diagnosis of cTTP confirmed by BOTH of the following:
 - a) Genetic test results demonstrating *ADAMTS13* gene mutation and
 - b) Plasma ADAMTS13 activity level <10% of normal (<10 IU/dL).
3. **Dosage allowed/Quantity limit:**
 Prophylaxis: 40 IU/kg IV once every other week; may adjust to once weekly.
 On-Demand: 40 IU/kg on day 1, 20 IU/kg on day 2, 15 IU/kg on day 3 and beyond until two days after the acute event is resolved.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show improvement or stabilized signs and symptoms of disease such as fewer acute TTP events, increased ADAMTS13 activity, or increased platelet count with treatment.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Adzynma (ADAMTS13, recombinant-krhn) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
12/13/2023	New policy for Adzynma created.

References:

1. Adzynma [prescribing information]. Takeda Pharmaceuticals U.S.A., Inc.; 2023.
2. Zheng XL, Vesely SK, Cataland SR, et al. ISTH guidelines for the diagnosis of thrombotic thrombocytopenic purpura [published correction appears in *J Thromb Haemost.* 2021 May;19(5):1381]. *J Thromb Haemost.* 2020;18(10):2486-2495. doi:10.1111/jth.15006
3. 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
4. Jain N, Oldenburg J, Ozelo MC, Sun SX, Tang L, Tzivelekis S. Recent advances in therapeutic options for rare hemostatic disorders: selected poster extracts of recent research in hemophilia A, congenital hemophilia with inhibitors, von Willebrand disease, and thrombotic thrombocytopenic purpura presented at the 29th congress of the International Society on Thrombosis and Haemostasis (ISTH 2021, Jul 17-21; virtual congress). *Expert Rev Hematol.* 2022;15(sup1):1-18. doi:10.1080/17474086.2022.2074395
5. Nusrat S, Beg K, Khan O, Sinha A, George J. Hereditary Thrombotic Thrombocytopenic Purpura. *Genes (Basel).* 2023;14(10):1956. Published 2023 Oct 18. doi:10.3390/genes14101956
6. 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. doi:10.1111/bjh.19026
7. Alwan F, Vendramin C, Liesner R, et al. Characterization and treatment of congenital thrombotic thrombocytopenic purpura. *Blood.* 2019;133(15):1644-1651. doi:10.1182/blood-2018-11-884700

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