Adzynma (ADAMTS13, Recombinant-Krhn)

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➲ Instructions for Use

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Community Plan Policy

- Adzynma (ADAMTS13, Recombinant-Krhn)

Application

UnitedHealthcare Commercial

This Medical Policy applies to UnitedHealthcare Commercial benefit plans.

UnitedHealthcare Individual Exchange

This Medical Policy applies to Individual Exchange benefit plans.

Coverage Rationale

➲ See Benefit Considerations

Adzynma (ADAMTS13, recombinant-krhn) is proven and medically necessary for prophylactic treatment of congenital thrombotic thrombocytopenic purpura (cTTP) in patients who meet all of the following criteria:

- For **initial therapy**, **all** of the following:
  - Diagnosis of congenital thrombotic thrombocytopenic purpura (cTTP); **and**
  - ADAMTS13 mutation is confirmed by molecular genetic testing; **and**
  - Adzynma is being prescribed for routine prophylactic treatment to prevent thrombotic thrombocytopenic purpura (TTP) events; **and**
  - Adzynma dosing is in accordance with the United States Food and Drug Administration approved labeling; **and**
  - Prescribed by or in consultation with a hematologist; **and**
  - Authorization will be for no more than 12 months
- For **continuation of therapy**, **all** of the following:
  - Patient has previously received routine prophylactic treatment with Adzynma; **and**
  - Documentation of positive clinical response to Adzynma therapy; **and**
  - Adzynma dosing is in accordance with the United States Food and Drug Administration approved labeling; **and**
  - Prescribed by or in consultation with a hematologist; **and**
  - Authorization will be for no more than 12 months

Adzynma (ADAMTS13, recombinant-krhn) is proven and medically necessary for on demand treatment of an acute thrombotic thrombocytopenic purpura (TTP) event in patients with congenital thrombotic thrombocytopenic purpura (cTTP) who meet all of the following criteria:

- Diagnosis of congenital thrombotic thrombocytopenic purpura (cTTP); **and**

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- ADAMTS13 mutation is confirmed by molecular genetic testing; **and**
- Adzynma is being prescribed for on demand treatment of an acute thrombotic thrombocytopenic purpura (TTP) event; **and**
- Adzynma dosing is in accordance with the United States Food and Drug Administration approved labeling; **and**
- Prescribed by or in consultation with a hematologist; **and**
- Authorization will be for no more than 3 months

Applicable Codes

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Listing of a code in this policy does not imply that the service described by the code is a covered or non-covered health service. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other policies and guidelines may apply.

HCPCS Code	Description
J7171	Injection, ADAMTS13, recombinant-krhn, 10 IU

Diagnosis Code	Description
D69.42	Congenital and hereditary thrombocytopenia purpura

Background

Adzynma (ADAMTS13, recombinant-krhn)is a recombinant form of the endogenous ADAMTS13. ADAMTS13 is a plasma zinc metalloprotease that regulates the activity of von Willebrand factor (VWF) by cleaving large and ultra large VWF multimers to smaller units and thereby reducing the platelet binding properties of VWF and its propensity to form microthrombi.

Benefit Considerations

Some Certificates of Coverage allow for coverage of experimental/investigational/unproven treatments for life-threatening illnesses when certain conditions are met. The member specific benefit plan document must be consulted to make coverage decisions for this service. Some states mandate benefit coverage for off-label use of medications for some diagnoses or under some circumstances when certain conditions are met. Where such mandates apply, they supersede language in the benefit document or in the medical or drug policy.

Clinical Evidence

Proven

Congenital Thrombotic Thrombocytopenic Purpura (cTTP)

ADAMTS13, recombinant-krhn was studied in a global, prospective, randomized, active-controlled, open-label, multicenter, two-period crossover study followed by a single arm continuation period (Study 1) evaluating the efficacy and safety of the prophylactic and on demand ERT with ADAMTS13, recombinant-krhn compared to plasma-based therapies in patients with cTTP. The efficacy of ADAMTS13, recombinant-krhn in the prophylactic treatment of patients with cTTP was evaluated in Study 1, in 46 patients who were randomized to receive 6 months of treatment with either 40 IU/kg of ADAMTS13, recombinant-krhn or plasma-based therapies (Period 1), then crossed over to the other treatment for 6 months (Period 2). Thirty-five patients have entered the 6-month single arm period with ADAMTS13, recombinant-krhn (Period 3). The median (min-max) age of patients was 32.5 years (range 3-58 years), with a mean weight of 67.6 kg. Most patients were white (65.2%), not Hispanic or Latino (80.4%) and were female (58.7%). Twenty of the 27 female patients (74.1%) were of child-bearing potential.

The efficacy of prophylactic treatment with ADAMTS13, recombinant-krhn in patients with cTTP was demonstrated based on the incidence of protocol defined acute and subacute TTP events and TTP manifestations, as well as the incidence of supplemental doses prompted by subacute TTP events over a 6-month time period. No patients receiving ADAMTS13, recombinant-krhn had an acute TTP event throughout the study, including Period 3 (with a median duration of exposure to ADAMTS13, recombinant-krhn of 14 months for patients 12 to < 18 years of age and patients ≥ 18 years of age; and 4

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and 1 months in patients 6 to < 12 and < 6 years of age, respectively). One acute TTP event occurred in a patient receiving plasma-based therapies (FFP) prophylactically during Period 1.

No subacute TTP events were reported in patients receiving ADAMTS13, recombinant-krhn during Periods 1 and 2. In Period 3, two patients receiving ADAMTS13, recombinant-krhn prophylaxis had two subacute events of which one was treated with four supplemental doses, 2 of FFP and 2 of ADAMTS13, recombinant-krhn. Four patients receiving plasma based therapies had five subacute TTP events in Periods 1 and 2. A total of seven supplemental doses, 2 of FVIII-VWF concentrate, 1 of FFP and 4 of ADAMTS13, recombinant-krhn were given to three of these patients.

The efficacy of the on-demand (OD) enzyme replacement therapy was evaluated based on the proportion of acute TTP events responding to ADAMTS13, recombinant-krhn in both the Prophylactic and the OD cohorts throughout the duration of the study. An acute TTP event responding to ADAMTS13, recombinant-krhn was defined as a resolved TTP event when platelet count was ≥ 150,000/μL or platelet count was within 25% of baseline, whichever occurs first, and LDH ≤ 1.5 x baseline or ≤ 1.5 x ULN, without requiring the use of another ADAMTS13-containing agent. Five adult patients (≥ 18 years of age) enrolled in the OD cohort and had a total of six acute TTP events. Of these five patients, two patients were randomized to receive on-demand treatment with ADAMTS13, recombinant-krhn and three patients were randomized to receive plasma-based therapies. All 6 acute TTP events resolved after treatment with either ADAMTS13, recombinant-krhn or plasma-based therapies.

U.S. Food and Drug Administration (FDA)

This section is to be used for informational purposes only. FDA approval alone is not a basis for coverage.

Adzynma (ADAMTS13, recombinant-krhn) 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).

References

1. Adzynma [package insert]. Lexington, MA: Takeda Pharmaceuticals USA, Inc., June 2024.
2. Alwan F, Vendramin C, Liesner R, et. al. Characterization and treatment of congenital thrombotic thrombocytopenic purpura. Blood. 2019 Apr 11;133(15):1644-1651.
3. Asmis LM, Serra A, Krafft A, et. al. Recombinant ADAMTS13 for Hereditary Thrombotic Thrombocytopenic Purpura. N Engl J Med. 2022 Dec 22;387(25):2356-2361.

Policy History/Revision Information

Date	Summary of Changes

07/01/2026	**Template Update**
	• Transferred content to shared policy template that applies to both UnitedHealthcare Commercial and Individual Exchange benefit plans
	• Added *Application* section to indicate this policy applies to:
	  ○ UnitedHealthcare Commercial benefit plans
	  ○ Individual Exchange benefit plans
	Supporting Information
	• Archived previous policy versions 2025D0131D and IEXD00131.03

Instructions for Use

This Medical Benefit Drug Policy provides assistance in interpreting UnitedHealthcare standard benefit plans. When deciding coverage, the member specific benefit plan document must be referenced as the terms of the member specific benefit plan may differ from the standard plan. In the event of a conflict, the member specific benefit plan document governs. Before using this policy, check the member specific benefit plan document and any applicable federal or state mandates. UnitedHealthcare reserves the right to modify its policies and guidelines as necessary. This Medical Benefit Drug Policy is provided for informational purposes. It does not constitute medical advice.

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This Medical Benefit Drug Policy may also be applied to Medicare Advantage plans in certain instances. In the absence of a Medicare National Coverage Determination (NCD), Local Coverage Determination (LCD), or other Medicare coverage guidance, CMS allows a Medicare Advantage Organization (MAO) to create its own coverage determinations, using objective evidence-based rationale relying on authoritative evidence (Medicare IOM Pub. No. 100-16, Ch. 4, §90.5).

UnitedHealthcare may also use tools developed by third parties, such as the InterQual® criteria, to assist us in administering health benefits. UnitedHealthcare Medical Benefit Drug Policies are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice.

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