Adzynma (ADAMTS13, Recombinant-Krhn)

Policy Number: CS2026D0131G
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Application

This Medical Benefit Drug Policy does not apply to the states listed below; refer to the state-specific policy/guideline, if noted:

State	Policy/Guideline
Arizona	Refer to the state's Medicaid clinical policy
Florida	Refer to the state's Medicaid clinical policy
Kansas	Refer to the state's Medicaid clinical policy
North Carolina	None
Ohio	Adzynma (ADAMTS$_{13}$, Recombinant-Krhn) (for Ohio Only)

Coverage Rationale

Adzynma (ADAMTS$_{13}$, 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**
  - ADAMTS$_{13}$ 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 (ADAMTS$_{13}$, 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**
- $ADAMTS_{13}$ 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 federal, state, or contractual requirements 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 ($ADAMTS_{13}$, recombinant-krhn) is a recombinant form of the endogenous $ADAMTS_{13}$. $ADAMTS_{13}$ 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.

Clinical Evidence

Proven

Congenital Thrombotic Thrombocytopenic Purpura (cTTP)

$ADAMTS_{13}$, 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 $ADAMTS_{13}$, recombinant-krhn compared to plasma-based therapies in patients with cTTP. The efficacy of $ADAMTS_{13}$, 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 $ADAMTS_{13}$, 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 $ADAMTS_{13}$, 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 $ADAMTS_{13}$, 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 $ADAMTS_{13}$, recombinant-krhn had an acute TTP event throughout the study, including Period 3 (with a median duration of exposure to $ADAMTS_{13}$, recombinant-krhn of 14 months for patients 12 to < 18 years of age and patients ≥ 18 years of age; and 4 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 $ADAMTS_{13}$, recombinant-krhn during Periods 1 and 2. In Period 3, two patients receiving $ADAMTS_{13}$, recombinant-krhn prophylaxis had two subacute events of which one was treated with four supplemental doses, 2 of FFP and 2 of $ADAMTS_{13}$, 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 $ADAMTS_{13}$, 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 ADAMTS$_{13}$, recombinant-krhn in both the prophylactic and the OD cohorts throughout the duration of the study. An acute TTP event responding to ADAMTS$_{13}$, 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 ADAMTS$_{13}$-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 ADAMTS$_{13}$, recombinant-krhn and three patients were randomized to receive plasma-based therapies. All 6 acute TTP events resolved after treatment with either ADAMTS$_{13}$, 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 (ADAMTS$_{13}$, recombinant-krhn) is a human recombinant, "A disintegrin and metalloproteinase with thrombospondin motifs 13" (rADAMTS$_{13}$) 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
04/01/2026	**Application** ***Indiana*** • Removed language indicating this Medical Benefit Drug Policy does not apply to the state of **Indiana** ***Louisiana*** • Removed content/language pertaining to the state of **Louisiana** **Supporting Information** • Archived previous policy versions CS2026D0131F and CSIND00131.03

Instructions for Use

This Medical Benefit Drug Policy provides assistance in interpreting UnitedHealthcare standard benefit plans. When deciding coverage, the federal, state, or contractual requirements for benefit plan coverage must be referenced as the terms of the federal, state, or contractual requirements for benefit plan coverage may differ from the standard benefit plan. In the event of a conflict, the federal, state, or contractual requirements for benefit plan coverage govern. Before using this policy, check the federal, state, or contractual requirements for benefit plan coverage. 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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