CARECONTINUUM™

UTILIZATION MANAGEMENT MEDICAL POLICY

POLICY: Hematology – Cablivi Utilization Management Medical Policy
- Cablivi® (caplacizumab-yhdp intravenous infusion and subcutaneous injection – Genzyme/Sanofi)

REVIEW DATE: 08/26/2026

Overview
Cablivi, a von Willebrand factor (vWF)-directed antibody fragment, is indicated for the treatment of **acquired thrombotic thrombocytopenic purpura** (TTP) in adults and pediatric patients ≥ 12 years of age, in combination with plasma exchange and immunosuppressive therapy.[1]

Disease Overview
Acquired TTP is a rare but potentially fatal blood disorder.[2-4] The annual incidence worldwide is 2 to 6 cases per million; acquired TTP is more common in adults. Most cases of acquired TTP are due to autoantibodies formed against the ADAMTS13 (A Disintegrin And Metalloproteinase with ThromboSpondin-1 motif, member 13) protein. Reduced ADAMTS13 activity (< 10%) leads to accumulation of ultra-large vWF multimers in the blood, which bind to platelets and lead to excessive platelet clumping in the microvasculature, resulting in multi-organ failure and death. Also, red blood cells break apart which leads to anemia. Cablivi is a nanobody that targets the ultra-large vWF and inhibits the interaction between vWF and platelets, thereby preventing vWF platelet adhesion and consumption.[1] When given concurrently with immunosuppression and therapeutic plasma exchange, Cablivi reduces the time to the normalization of the platelet count and decreases the risk of early recurrence compared with placebo.[1,6-8] Risks associated with Cablivi involve bleeding, which may be serious.

Dosing Information
Two doses of Cablivi are given on the first day of plasma exchange, followed by one dose of Cablivi per day during plasma exchange; treatment is continued for 30 days after the last plasma exchange session.[1] If, after the initial treatment course, there are signs of persistent underlying disease such as suppressed ADAMTS13 levels, Cablivi therapy may be extended for a maximum of 28 days. Cablivi should be discontinued if the patient experiences more than two recurrences of acquired TTP while on Cablivi. The first dose of Cablivi is given as a bolus intravenous injection by a healthcare provider; additional doses are administered by subcutaneous (SC) injection. The SC injections may be self-administered in adults or by a caregiver. In pediatric patients ≥ 12 years of age, SC Cablivi may be given by a healthcare provider or an adult caregiver.

Guidelines/Recommendations
The International Society on Thrombosis and Hemostasis (ISTH) has guidelines for the diagnosis of TTP (2020).[4] For patients with a high clinical suspicion of acquired TTP, ADAMTS13 testing is recommended for selected patients.[4] The ISTH also has guidelines for the treatment of TTP (2020).[9] For patients with acquired TTP experiencing an acute event (first event or relapse), the panel suggests therapy with Cablivi over not using Cablivi.[9] This was reaffirmed by ISTH in 2025.[10] The panel stressed that Cablivi should only be given under the guidance of an experienced clinician, ideally a TTP expert (e.g., a hematologist or pathologist specialized in transfusion medicine with previous experience in treating the disease).[9]

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POLICY STATEMENT

Prior Authorization is recommended for medical benefit coverage of Cablivi. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indication. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). Because of the specialized skills required for evaluation and diagnosis of patients treated with Cablivi as well as the monitoring required for adverse events and long-term efficacy, approval requires Cablivi to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

Documentation: Documentation is required for use of Cablivi as noted in the criteria as **[documentation required]**. Documentation must provide the date of the most recent administration of plasma exchange or the scheduled date of plasma exchange if it has not yet occurred. All documentation must include patient-specific identifying information.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Cablivi is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Acquired Thrombotic Thrombocytopenic Purpura.** Approve for up to 60 days following the last plasma exchange session if the patient meets ALL of the following (A, B, C, D, and E):
   - **A)** Patient is ≥ 12 years of age; AND
   - **B)** Patient meets BOTH of the following (i and ii):
     - **i.** Cablivi will be or was administered on the same day as initiation of plasma exchange therapy **[documentation required]**; AND
     - **ii.** Cablivi use will not exceed, or has not exceeded, 60 days after the most recent plasma exchange session **[documentation required]**; AND
   - **C)** Patient is currently receiving at least one immunosuppressive therapy; AND
     Note: Examples include systemic corticosteroids, rituximab, cyclosporine, cyclophosphamide, mycophenolate mofetil, hydroxychloroquine, bortezomib.
   - **D)** According to the prescriber, Cablivi will be discontinued if the patient has more than two recurrences of acquired thrombotic thrombocytopenic purpura while receiving Cablivi during this current treatment course; AND
   - **E)** The medication is prescribed by or in consultation with a hematologist.

   Dosing. Approve two doses of Cablivi (11 mg intravenous bolus prior to plasma exchange followed by an 11 mg subcutaneous [SC] dose after completion of plasma exchange), then 11 mg SC once daily.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Cablivi is not recommended in the following situations.

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

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10. Zheng XL, Al-Housni Z, Cataland S, et al, on behalf of the International Society on Thrombosis and Haemostasis. 2025 focused update of the 2020 ISTH guidelines for management of thrombotic thrombocytopenia purpura. *J Thromb Haemost*. 2025;23:3711-3732.

HISTORY

Type of Revision	Summary of Changes	Review Date
Annual Revision	No criteria changes.	02/21/2024
Annual Revision	No criteria changes.	02/19/2025
Selected Revision	**Acquired Thrombotic Thrombocytopenic Purpura:** The age of approval was changed from ≥ 18 years of age to ≥ 12 years of age.	01/14/2026
Annual Revision	No criteria changes.	02/11/2026
Early Annual Revision	**Policy Statement:** The statement that all approvals are provided for one course of treatment was removed. The statement that noted that one course of treatment consists of Cablivi to be administered in conjunction with plasma exchange and Cablivi is to be administered for up to 60 days (one dose per day) following the last plasma exchange was also removed. Documentation was added to this section as well. It was noted that documentation must provide the date of the most recent administration of plasma exchange or the scheduled date of plasma exchange if it has not occurred yet. In addition, the following changes were made: **Acquired Thrombotic Thrombocytopenic Purpura:** The requirement that Cablivi was initiated in the inpatient setting in combination with plasma exchange was changed to the following: 1) Cablivi will be or was administered on the same day as initiation of plasma exchange therapy (with documentation required), AND 2) Cablivi use will not exceed, or has not exceeded, 60 days after the most recent plasma exchange (with documentation required). The requirement that if the patient has previously received Cablivi, he/she has not had more than two recurrences of acquired thrombotic thrombocytopenic purpura while on Cablivi was changed to the requirement that according to the prescriber, Cablivi will be discontinued if the patient has more than two recurrences of acquired thrombotic thrombocytopenic purpura while receiving Cablivi during this current treatment course. Regarding Dosing, the wording to not exceed 60 days following the last plasma exchange session was removed. Dosing was changed to state "Approve two doses of Cablivi (11 mg intravenous bolus prior to plasma exchange followed by an 11 mg subcutaneous dose after completion of plasma exchange), then 11 mg by subcutaneous injection once daily; the phrase stating "Day 1 of treatment with plasma exchange" was removed, as well as "up to" (referencing to once daily).	08/26/2026

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