Reference number(s)
6251-A

STANDARD MEDICARE PART B MANAGEMENT

ADZYNMA (ADAMTS13, recombinant-krhn)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

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

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
A. Initial requests: ADAMTS13 enzyme assay and ADAMTS13 genetic testing results supporting the diagnosis.
B. Continuation of therapy requests: Medical records (e.g., chart notes, lab reports) documenting a response to therapy (e.g., reduction or maintenance of number of thrombotic thrombocytopenic purpura [TTP] events, increase in platelet count, decrease in lactate dehydrogenase [LDH] level).

III. CRITERIA FOR INITIAL APPROVAL

Congenital thrombotic thrombocytopenic purpura (cTTP)
Authorization of 6 months may be granted for the treatment of congenital thrombotic thrombocytopenic purpura (cTTP) when both of the following criteria are met:
A. The diagnosis of cTTP has been confirmed by genetic testing with biallelic mutations in the ADAMTS13 gene.
B. Member has an ADAMTS13 activity level of less than 10% at the time of diagnosis.

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization of 12 months may be granted for continued treatment of congenital thrombotic thrombocytopenic purpura (cTTP) when both of the following criteria are met:
A. The member is currently receiving therapy with the requested medication.

B. The member is receiving benefit from therapy (e.g., reduction or maintenance of number of thrombotic thrombocytopenic purpura [TTP] events, increase in platelet count, decrease in lactate dehydrogenase [LDH] level).

V. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:

1. The prescribing information for Adzynma.
2. The available compendium
    a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    b. Micromedex DrugDex
    c. American Hospital Formulary Service- Drug Information (AHFS-DI)
    d. Lexi-Drugs
    e. Clinical Pharmacology

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Adzynma are covered.

VI. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for using an enzyme assay and genetic testing to confirm the diagnosis of cTTP prior to initiating treatment with Adzynma can be found in the clinical trials cited in the prescribing information. To be included in the trial, the patient must have had a documented diagnosis of severe hereditary ADAMTS13 deficiency, defined as: A) Confirmed by molecular genetic testing, documented in participant history or at screening, and B) ADAMTS13 activity < 10 % as measured by the fluorescent resonance energy transfer- von Willebrand factor73 (FRETS-VWF73) assay, documented in participant history or at screening (participants currently receiving standard of care prophylactic therapy may exceed 10% ADAMTS13 activity at screening). Additionally, an article from The New England Journal of Medicine, cited in the prescribing information, indicates that hereditary TTP is caused by biallelic mutations in the gene ADAMTS13 that lead to a severe ADAMTS13 deficiency (ADAMTS13 activity <10% of that in normal plasma).

VII. REFERENCES

1. Adzynma [package insert]. Lexington, MA: Takeda Pharmaceuticals U.S.A., Inc.; November 2023.
2. Asmis LM, Serra A, Krafft A, et al. Recombinant ADAMTS13 for Hereditary Thrombotic Thrombocytopenic Purpura. N Engl J Med 2022; 387: 2356-2361.