Reference number(s)
4868-A

STANDARD MEDICARE PART B MANAGEMENT

VONVENDI (von Willebrand factor [recombinant])

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 Indications
Vonvendi is indicated for use in adults (age 18 and older) diagnosed with von Willebrand disease (VWD) for:
1. On-demand treatment and control of bleeding episodes
2. Perioperative management of bleeding
3. Routine prophylaxis to reduce the frequency of bleeding episodes in patients with severe Type 3 VWD receiving on-demand therapy

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. CRITERIA FOR INITIAL APPROVAL

Von Willebrand Disease
Authorization of 12 months may be granted for members with VWD when any of the following criteria is met:
A. Member has type 1, 2A, 2M, or 2N VWD and has had an insufficient response to desmopressin or a documented clinical reason for not using desmopressin (see Appendix).
B. Member has type 2B or type 3 VWD.

III. CONTINUATION OF THERAPY

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

Authorization for 12 months may be granted when all of the following criteria are met:
A. The member is currently receiving therapy with Vonvendi.
B. Vonvendi is being used to treat an indication enumerated in Section II.
C. The member is receiving benefit from therapy (e.g., reduced frequency or severity of bleeds).

IV. APPENDIX
Clinical Reasons for Not Utilizing Desmopressin in Patients with Type 1, 2A, 2M and 2N VWD

A. Age < 2 years
B. Pregnancy
C. Fluid/electrolyte imbalance
D. High risk for cardiovascular or cerebrovascular disease (especially the elderly)
E. Predisposition to thrombus formation
F. Trauma requiring surgery
G. Life-threatening bleed
H. Contraindication or intolerance to desmopressin
I. Severe type 1 von Willebrand disease
J. Stimate Nasal Spray is unavailable due to backorder/shortage issues (where applicable)

IV. SUMMARY OF EVIDENCE

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

1. The prescribing information for Vonvendi.
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
3. The diagnosis, evaluation, and management of von Willebrand disease.
4. World Federation of Hemophilia (WFH) Guidelines for the Management of Hemophilia, 3rd edition.
5. MASAC recommendations concerning products licensed for the treatment of hemophilia and other bleeding disorders.

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

V. EXPLANATION OF RATIONALE

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

Support for using Vonvendi to treat von Willebrand syndrome can be found in the National Institutes of Health publication called the "Diagnosis, Evaluation, and Management of von Willebrand Disease". Type 2B and type 3 VWD do not respond consistently to DDAVP therapy and therefore DDAVP is not considered clinically useful in these patients.
The guideline from the National Bleeding Disorders Foundation (previously the National Hemophilia Foundation) also recommends using Vonvendi in VWD. Vonvendi is used to treat patients with type 2B and type 3 VWD; it can also be used in patients with types 1, 2A, 2M, and 2N VWD who are not responsive to DDAVP and in children under 2 years of age regardless of VWD type. Vonvendi is approved for use as routine prophylaxis only in individuals with severe type 3 VWD who were previously treated with VWF (recombinant or plasma derived) on-demand.

VI. REFERENCES

1. Vonvendi [package insert]. Lexington, MA: Baxalta US Inc.; January 2022.
2. National Hemophilia Foundation. MASAC recommendations concerning products licensed for the treatment of hemophilia and other bleeding disorders. Revised March 2022. MASAC Document #272.

https://www.hemophilia.org/sites/default/files/document/files/272_Treatment.pdf. Accessed October 4, 2022.
3. National Hemophilia Foundation. MASAC recommendations regarding the treatment of von Willebrand disease. Revised February 2021. MASAC Document #266. https://www.hemophilia.org/sites/default/files/document/files/266.pdf. Accessed October 4, 2022.
4. National Institutes of Health. The diagnosis, evaluation, and management of von Willebrand disease. Bethesda, MD: US Dept of Health and Human Services, National Institutes of Health; 2007. NIH publication No. 08-5832.
5. Stimate [package insert]. King of Prussia, PA: CSL Behring LLC; June 2021.
6. Leissinger C, Carcao M, Gill JC, et al. Desmopressin (DDAVP) in the management of patients with congenital bleeding disorders. *Haemophilia*. 2014;20:158-167.