Von Willebrand factor (Vonvendi®)

EOCCO POLICY

Policy Type: PA/SP Pharmacy Coverage Policy: EOCCO073

Description

Vonvendi is a recombinant von Willebrand factor indicated for use in adults (age 18 and older) diagnosed with von Willebrand disease for on-demand treatment and control of bleeding episodes, and perioperative management.

Length of Authorization

- Initial: 6 months (for on-demand); 1 month (for perioperative)
- Renewal: 6 months (for on-demand)

Quantity limits

Product Name	Dosage Form	Indication/ FDA Labeled Dosing	Quantity Limit
Vonvendi, von Willebrand factor (recombinant)	650, 1300 IU	**On-demand treatment and control of bleeding episodes:** • *Minor:* Up to 50 IU/kg for the initial dose, subsequent doses of up to 50 IU/kg every eight to 24 hours as clinically required • *Major:* Up to 80 IU/kg for the initial dose, subsequent doses of up to 60 IU/kg every eight to 24 hours for approximately two to three days, as clinically required **Perioperative management of bleeding:** A dose may be given 12 to 24 hours prior to surgery to allow the endogenous factor VIII levels to increase to at least 30 IU/dL (minor surgery) or 60 IU/dL (major surgery)	**On-demand treatment and control of bleeding episodes:** Up to the number of doses requested every 28 days **Perioperative management of bleeding:** Up to the number of doses requested every 28 days

Initial Evaluation

I. Vonvendi may be considered medically necessary when the following criteria below are met:
   A. Treatment is prescribed by or in consultation with a hematologists; **AND**
   B. A diagnosis of von Willebrand disease (vWD) has been confirmed by blood coagulation and von Willebrand factor testing; **AND**
   C. Use is planned for one of the following indications:
      1. On-demand treatment and control of bleeding when one of the following is met:

i. Member has severe vWD; **OR**
ii. Member has mild or moderate vWD and the use of desmopressin is known or suspected to be ineffective or contraindicated; **OR**

2. Perioperative management of bleeding

II. Vonvendi is considered investigational when used for any other condition.

Renewal Evaluation

I. Documentation of clinical benefit, including decreased incidence of bleeding episodes or stability of bleeding episodes relative to baseline

Supporting Evidence

I. Von Willebrand disease (vWD) is the most common of the inherited bleeding disorders. Although vWD is common, only a fraction of patients seek medical attention due to bleeding symptoms due to the mild nature of the disease in many patients, and to the lack of bleeding challenges.

II. There are three types of inherited vWD:

- Type 1 – The most common type that accounts for about 70% of cases. It reflects a quantitative deficiency of von Willebrand factor (vWF). The clinical presentation varies from mild to moderately severe.
- Type 2 – Accounts for 25-30% of cases and is characterized by several qualitative abnormalities of vWF (e.g. altered size rations or biologic properties).
- Type 3 – The most severe type of disease with very low or undetectable levels of vWF. Patients typically present with severe bleeding involving both the skin and mucous membrane surfaces and soft tissues and joints. Replacement therapy with vWF is usually required.

III. Choice of therapy begins with an accurate and complete diagnosis of vWD, plus patient-specific factors must be taken to account (e.g. history of bleeding, response to prior therapies).

IV. A trial of desmopressin (DDAVP) should be considered in all patients with type 1 and most with type 2, but not in patients with type 3 vWD. Typically, minor bleeding episodes can be treated with DDAVP without further therapeutic intervention. Major surgery typically requires replacement with vWF.

V. Patients with type 3 vWD, those with more severe type 1, and many of those with certain subtypes of type 2 disease often require replacement therapy with a vWF-containing product to control bleeding. However, vWF is not generally given as long-term prophylaxis like is done in patients with hemophilia A.

VI. The safety and efficacy of Vonvendi was established based on a series of 22 patients with vWD over the age of 18 years of age who experienced 192 bleeding episodes (mostly mucosal, seven major). Results showed the Vonvendi was highly effective in restoring hemostasis. Most episodes were treated with a single infusion.

Investigational or Not Medically Necessary Uses

There is no evidence to support the use of Vonvendi in any other condition.

References

1. Vonvendi® [Prescribing Information]. Westlake Village, CA: Baxalta US Inc; February 2019
2. National Hemophilia Foundation. MASAC Recommendations Concerning products Licensed for the Treatment of Hemophilia and Other Bleeding Disorders. Available from: https://www.hemophilia.org/Researchers-Healthcare-Providers/Medical-and-Scientific-Advisory-Council-MASAC/MASAC-Recommendations. Accessed July 5, 2019.
3. UpToDate, Inc. Treatment of von Willebrand disease. UpToDate [database online]. Last updated July 19, 2019.

Policy Implementation/Update:

Date Created	August 2019
Date Effective	August 2019
Last Updated	August 2019
Last Reviewed	08/2019

Action and Summary of Changes	Date
New policy created for von Willebrand factor (Vonvendi)	08/2019