Vonvendi® (von Willebrand factor [recombinant]) – New indication

- On January 31, 2022, Takeda announced the FDA approval of Vonvendi (von Willebrand factor [recombinant]), for use in adults (age 18 and older) diagnosed with von Willebrand disease (VWD) for routine prophylaxis to reduce the frequency of bleeding episodes in patients with severe Type 3 VWD receiving on-demand therapy.

- Vonvendi is also approved for use in adults diagnosed with VWD for:

    - On-demand treatment and control of bleeding episodes
    - Perioperative management of bleeding.

- VWD is the most common inherited bleeding disorder, affecting up to 1% of the U.S. population. VWD is caused by a deficiency or dysfunction of von Willebrand factor, one of several types of proteins in the blood that are needed to facilitate proper blood clotting.

- The approval of the expanded indication was based on a single arm, open-label study evaluating Vonvendi prophylactic treatment in reducing the frequency of bleeding episodes in adult patients diagnosed with VWD. There were 12 patients who previously received on-demand (OD) treatment (Prior OD Group) and 10 patients who previously received prophylactic treatment with plasma derived von Willebrand factor (pdVWF) (Switch Group) prior to enrolling into this study. Efficacy was assessed based on the median annualized bleeding rate (ABR) for all bleeds, spontaneous bleeds, and joint bleeds.

    - Data is provided in the table below for patients with Type 3 VWD receiving on-demand treatment prior to study entry. The median percentage change of ABRs from historical to on-study were: for all bleeds -54.7%, for spontaneous bleeds -75.9%, and for joint bleeds -100.0%.

Type or site of bleeding event	Number of bleeding episodes historical/ on-study	Historical median ABR (min, max)	On-study median ABR (min, max)
All bleeds	201/38	5.0 (3.0, 159.0)	2.3 (0, 157.9)
Spontaneous bleeds	195/33	3.5 (3.0, 158.0)	1.0 (0.0, 157.9)
Joint bleeds	23/3	2.0 (0.0, 7.0)	0.0 (0.0, 1.9)

    - The study did not adequately demonstrate efficacy of Vonvendi as prophylactic treatment to reduce bleeding events in patients with Type 3 VWD receiving prophylactic therapy prior to study entry (Switch Group).

- For initiation of prophylactic treatment, Vonvendi should be administered as 40 to 60 IU per kg of body weight twice weekly. The prophylaxis dose should be adjusted up to 60 IU/kg twice weekly if breakthrough bleeding occurs in joints or if severe bleeding occurs. Refer to the drug label for dosing for treatment of breakthrough bleeding.

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— Refer to the Vonvendi drug label for complete dosing and administration information for its other uses.

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