

# FDA Grants Expanded Approval to Wilate as the First VWF Concentrate for Prophylaxis in All Types of VWD

Paramus, NJ 07/12/2023 Octapharma USA announced the expansion of the U.S. Food and Drug Administration (FDA) approval for wilate®, von Willebrand Factor/Coagulation Factor VIII Complex (Human) Lyophilized Powder for Solution for Intravenous Injection. The approved label now includes routine prophylaxis aimed at reducing the frequency of bleeding episodes in adults and children aged 6 and older with any type of von Willebrand disease (VWD), the most prevalent bleeding disorder in the United States. Wilate® is the first von Willebrand factor (VWF) concentrate indicated for prophylactic treatment across all forms of VWD, marking a significant milestone in the field.

“Long-term prophylaxis with VWF concentrate, as compared to on-demand treatment for bleeding, is recommended for patients with severe VWD,” according to Shveta Gupta, MD, a specialist in pediatric hematology and oncology with The Haley Center for Children's Cancer and Blood Disorders at Orlando Health Arnold Palmer Hospital for Children. “The approval of wilate® for VWD prophylaxis is a welcome new treatment option that can be life-saving for many patients. Increased use of VWF prophylaxis in VWD patients may lead to improved patient care and a reduced burden of disease.”

## **WIL-31 Study: A Breakthrough in VWD Treatment**

The FDA approval is supported by Octapharma's WIL-31 study, a prospective, non-controlled, international, multicenter phase 3 trial that investigated the efficacy and safety of wilate® prophylaxis over 12 months in people aged 6 and older with severe VWD of any type.

All WIL-31 patients received on-demand treatment with wilate® during a previous six-month, prospective, observational study (WIL-29). Patients who experienced at least six bleeding episodes (BEs), excluding menstrual bleeds, with at least two of these BEs treated with a VWF-containing product, were eligible to enter WIL-31. Patients in WIL-31 received wilate® prophylaxis two to three times per week at a dose of 20-40 IU/kg, for 12 months.

The clinical trial's primary purpose was to investigate whether prophylaxis with wilate® lowered the mean total annualized bleeding rate (ABR) by more than 50% compared to the six months of on-demand treatment. Secondary goals were to measure spontaneous ABR and treatment-emergent adverse events.<sup>1</sup>

Researchers reported an 84% reduction in the mean total ABR compared with on-demand treatment during the prior study. The median spontaneous ABR decreased by 95%. Importantly, no serious drug-related adverse events or thrombotic events were observed during the study.

## **Addressing the Prevalence of VWD in the U.S. Population**

Von Willebrand disease affects up to 1% of the U.S. population, equating to about 3.3 million individuals. The disease, occurring equally among men and women, is often noticed more by women due to heavy or abnormal bleeding during menstrual periods and after childbirth. VWD includes three major types – Type 1, Type 2, and Type 3, the most severe form. The expanded wilate® label provides a treatment option for a significant patient population.<sup>2</sup>

“Wilate® prophylaxis for adults and children aged 6 and older with VWD promises to be life-changing for many patients,” stated Octapharma USA President Flemming Nielsen. “Patients have been forced to live with far too many bleeding episodes while receiving on-demand treatment. Octapharma is pleased to offer patients a new therapy option that can greatly improve their quality of life.”

## **Factor My Way: Empowering VWD Patients**

In tandem with this approval, Octapharma USA extends its commitment to patient care through the Factor My Way patient support program. Tailored for those living with VWD and hemophilia A, this free membership program provides access to caregivers, educational programs, a resource library, and more. For additional information, please visit [FactorMyWay.com](http://FactorMyWay.com).

## **About wilate®**

Wilate®, von Willebrand Factor/Coagulation Factor VIII Complex (Human) Lyophilized Powder for Solution for Intravenous Injection is indicated in children and adults with von Willebrand disease for:

- On-demand treatment and control of bleeding episodes
- Perioperative management of bleeding
- Routine prophylaxis to reduce the frequency of bleeding episodes

Wilate® is indicated for routine prophylaxis in children 6 years of age and older and adults with von Willebrand disease.

Wilate® is indicated in adolescents and adults with hemophilia A for:

- Routine prophylaxis to reduce the frequency of bleeding episodes
- On-demand treatment and control of bleeding episodes

## **CONTRAINDICATIONS**

Do not use in patients with known hypersensitivity reactions, including anaphylactic or severe systemic reaction, to human plasma-derived products, any ingredient in the formulation, or components of the container.

## **WARNINGS AND PRECAUTIONS**

Anaphylaxis and severe hypersensitivity reactions are possible. Thromboembolic events may occur. Monitor plasma levels of FVIII activity. Development of neutralizing antibodies to FVIII and to VWF, especially in VWD type 3 patients, may occur. Wilate® is made from human plasma and carries the risk of transmitting infectious agents.

For complete prescribing information, please visit [wilateusa.com/pi](http://wilateusa.com/pi).

### **About the Octapharma Group**

Headquartered in Lachen, Switzerland, Octapharma is one of the largest human protein manufacturers in the world, developing and producing human proteins from human plasma and human cell lines.

Octapharma employs more than 11,000 people worldwide to support the treatment of patients in 118 countries with products across three therapeutic areas: Hematology, Immunotherapy and Critical Care.

Octapharma has seven R&D sites and five state-of-the-art manufacturing facilities in Austria, France, Germany and Sweden, and operates more than 190 plasma donation centers across Europe and the US. The company's American subsidiary, Octapharma USA, is located in Paramus, N.J. For more information, please visit [octapharmausa.com](http://octapharmausa.com)

## **REFERENCES**

1 – Robert F. Sidonio, Jr., Ana Boban, Leonid Dubey, Adlette Inati, Csongor Kiss, Toshko Lissitchkov, Dzmitry Novik, Elina Peteva, Ali T. Taher, Margarita Arkadevna Timofeeva, Kateryna V. Vilchevska, Vladimir Vdovin, Sylvia Werner, Sigurd Knaub, Claudia Khayat; Efficacy and Safety of Prophylaxis with a Plasma-Derived Von Willebrand Factor/ Factor VIII Concentrate in Previously Treated Patients with Von Willebrand Disease. *Blood* 2022; 140 (Supplement 1): 8438–8439. doi: <https://doi.org/10.1182/blood-2022-162358>.

2 – Centers for Disease Control and Prevention website, What is von Willebrand Disease, accessed Nov. 27, 2023.

Posted: December 2023

## **Related articles**

- FDA Approves Octapharma's Wilate® for Hemophilia A in Adult and Adolescent Patients - October 8, 2019
- FDA Approves Wilate License Supplement - Perioperative Management of Bleeding in Patients with von Willebrand Disease - August 13, 2015

- Octapharma USA Announces FDA Approval of Wilate - the First Replacement Therapy Developed Specifically for von Willebrand Disease - December 8, 2009

Wilate (von Willebrand Factor/Coagulation Factor VIII Complex (Human)) FDA Approval History

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