**Vonvendi® (recombinant von Willebrand factor) – New Orphan Drug Approval**

- On December 8, 2015, the [FDA](https://www.fda.gov) announced approval of Baxalta’s Vonvendi (recombinant von Willebrand factor [rVWF]) for on-demand treatment and control of bleeding episodes in adults diagnosed with von Willebrand disease (VWD).

- According to the Centers for Disease Control and Prevention (CDC), VWD is the most common inherited bleeding disorder, affecting approximately 1% of the U.S. population. VWD occurs equally in men and women and is caused by a deficiency or defect in VWF resulting in impaired clotting. The disease can manifest through a variety of bleeding events, including mucosal bleeds, gastrointestinal bleeds or menorrhagia.

- Vonvendi is an innovative recombinant protein treatment that supports clot formation. The treatment contains only trace amounts of Factor VIII (FVIII), offering the flexibility to administer additional FVIII only in required patients.
  - Vonvendi was granted orphan drug designation by the FDA.

- Vonvendi’s approval was based on data from an open-label trial comparing Vonvendi with and without recombinant FVIII (rFVIII) for 192 bleeding episodes in 22 adults diagnosed with VWD. The primary endpoint was the number of patients with treatment success for control of bleeding episodes.
  - The trial demonstrated that Vonvendi was safe and effective for the on-demand treatment and control of bleeding episodes from a variety of different sites in the body.
  - 100% (95% CI: 81.5, 100) of patients demonstrated treatment success of bleeding episodes, with an efficacy rate of excellent (96.9%) or good (3.1%).

- Warnings and precautions for Vonvendi include embolism and thrombosis, hypersensitivity reactions, neutralizing antibodies, and monitoring laboratory tests.

- The most common adverse event (≥ 2%) with Vonvendi use was generalized pruritus.

- For each bleeding episode, the first dose of Vonvendi should be administered with an approved rFVIII (non-VWF containing), if FVIII baseline levels are < 40% or unknown.
  - If rFVIII is required, rFVIII should be given within 10 minutes of completing Vonvendi infusion at a ratio of 1.3:1.
  - rFVIII medications include Advate®, Adynovate®, and Nuwiq®.

- The initial intravenous dose of Vonvendi is 40 – 80 international units (IU)/kg body weight.
  - For a minor hemorrhage, the initial dose is 40 – 50 IU/kg. The subsequent dose is 40 – 50 IU/kg every 8 to 24 hours (as clinically required).
  - For a major hemorrhage, the initial dose is 50 – 80 IU/kg. The subsequent dose is 40 – 60 IU/kg every 8 to 24 hours for approximately 2 – 3 days (as clinically required).
- Baxalta plans to launch Vonvendi in late 2016. Vonvendi will be available as a lyophilized powder in single-use vials containing nominally 650 IU or 1300 IU VWF Ristocetin Cofactor (VWF:RCo).