# VWD Connect Foundation Severe Von Willebrand Disease Patient Registry

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## PURPOSE / OBJECTIVES

Severe Von Willebrand Disease (sVWD) patients (estimated over 1500 in the U.S.) are an under-studied population with severe bleeding not well-characterized in literature. VWD Connect Foundation (VCF) has sponsored the design and launch of a severe Von Willebrand Disease online longitudinal natural history and outcomes patient registry.

The primary objective is to characterize the sVWD patient population, reporting on prevalence, genotypes, phenotypes, and management. The secondary objective is to provide a convenient online platform for participants (or caregivers) to self-report clinical outcomes in real time. This critical endeavor also aims to provide researchers and other stakeholders with actionable data describing the sVWD population leading to better treatments, management, and ultimately a cure.

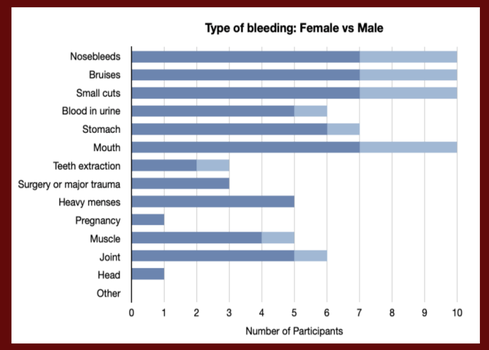
## MATERIALS & METHODS

August 2021: VWD Connect Foundation (VCF) received IRB approval for protocol VWD-001, The Severe Von Willebrand Disease Patient Registry: A Longitudinal Natural History and Patient Outcomes Study. The co-principal investigators are Christopher Walsh, MD, PhD, Mrinal Gounder, MD, Alice Arapshian and S. Christina Morgenthaler, MS, MBA.

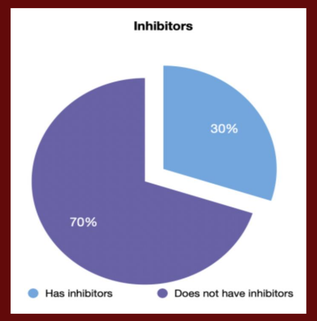
In December 2021, the Registry opened to a small cohort of participants. For the purposes of this Registry, sVWD patients may include Type 3 VWD and Severe Types 1, 1C, 2A, 2B, 2M, and 2N, excluding acquired VWD. Patients with unknown/other types may be considered severe if they have Von Willebrand factor levels <20%. Following informed consent and screening, participants complete modules, with continued participation as new modules are released.

Future modules planned include quality of life, family history, laboratory data, genetic data, reproductive bleeding, joints, treatments, prophylaxis and inhibitors. Participants complete the Self-Administered Bleeding Assessment Tool (Self-BAT; Deforest et al, 2015) upon initial enrollment.

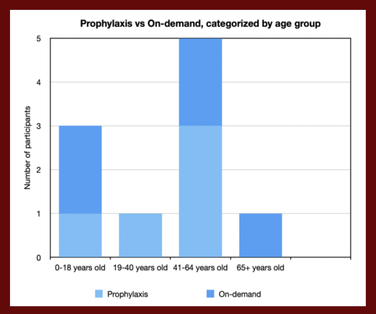
n=10 sType 3 patients



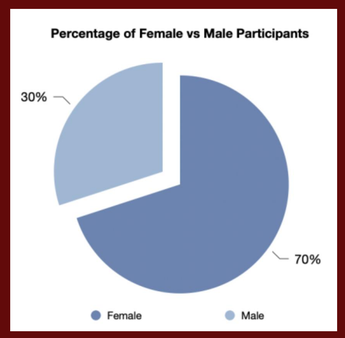

**Compiled results from Self-Administered Bleeding Tool reflecting types of bleeding by gender**




**History of Inhibitors**




**Prophylaxis vs On-demand categorized by age group**




**Percentage of female vs male participants**

## RESULTS

Preliminary data has been collected on a small cohort of 10 participants, with enrollment and participation ongoing. Data includes patient demographics, medical history, prior and concomitant medication history, and Self-BAT results.

All (100%) of the ten participants have a diagnosis of Type 3 Von Willebrand Disease and receive their care at a U.S. Hemophilia Treatment Center. The participants represent 7 states within the U.S. and 70% are female. Participant ages range from 5–67 years old, with a median age of 43. 30% of patients reported having developed inhibitors to replacement factors. 50% of responders reported being on prophylaxis treatment. Patients reported (Self-BAT) muco-cutaneous bleeding (100%) and other sites as shown.

The VCF Medical and Scientific Advisory Board, a seventeen-member international group of clinicians and scientists, has been engaged to review and comment on the sVWD Patient Registry and the initial data. The Patient Registry will be publicly open for enrollment in July 2022 to all eligible U.S. patients.

## SUMMARY/CONCLUSION

VWD Connect Foundation, as a National Non-Profit Patient Organization, has been able to utilize the skill of its membership along with dedicated community professionals, to undertake a significant research endeavor that will impact the advancement of the understanding and management of severe Von Willebrand Disease.

A primary focus for the Foundation will be to supply critical data that will aid in better defining sVWD and determining its prevalence in the population.