VWD Connect Foundation
U.S Severe Von Willebrand Disease Patient Registry

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Introduction and Objectives

The estimated 1,500 patients with severe Von Willebrand Disease (sVWD) in the U.S. are an under-studied population with severe bleeding that is not well characterized in literature. This demonstrates a need for patients to register and self-report natural history information on their diagnosis, bleeding history and management, quality of life, and medical data (e.g., laboratory values and genetic testing results). To respond to this need, VWD Connect Foundation (VCF) , the exclusive U.S. national patient organization for sVWD, undertook the design and launch of an online natural history patient registry for U.S. patients with sVWD.

Methods

VWD Connect Foundation established a Patient Registry Sub-Committee (PRSC) to design and launch an online natural history patient registry for those with sVWD. The PRSC members include three of the co-Principal Investigators of the study; two who have severe Type 3 VWD and one who is a father of a severe Type 3 VWD child. Additional PRSC members include a genetic counselor, a hematologist, a research coordinator and the Executive Director of the Foundation. Patient Foundation member volunteers contributed in the testing and critiquing of patient registry software platform.

For purposes of the study, sVWD patients may include, but are not limited to, those with Type 1 Severe, Type 1C, Type 2A, severe Type 2B, severe Type 2M, severe Type 2N, and Type 3. Additionally, patients with unknown type or with other types may be considered severe if they have Von Willebrand factor levels < 20%.

Working under advisement and guidance of the VCF Medical and Scientific Advisory Board (MSAB), a 16-member international Board of clinicians and researchers, VCF submitted the protocol to IRB in August 2021. The Protocol calls for a modular, prospective, longitudinal study on the natural history, biology and patient reported outcomes in severe Von Willebrand Disease. The Committee adopted the slogan, “For Patients, By Patients, Advancing Together” for the study.

Results and Conclusions

In August 2021, VWD Connect Foundation received IRB approval for VWD-001, The Severe Von Willebrand Disease Patient Registry: A Longitudinal Natural History and Patient Outcomes Study.

In December 2021, the Registry opened for a small cohort of sVWD Patients. The resulting data is being tested and the final registry process being reviewed by MSAB members.

The Registry is scheduled for full public open enrollment in June of 2022.

A National Patient Organization, utilizing the skill of its membership along with dedicated community professionals, has the resources to impact the advancement of its disease state by undertaking research endeavors, such as a Patient Registry. The Foundation, by sponsoring this registry, will be able to provide critical data to researchers describing the patient population to aid in their research for better treatments, management and ultimately a cure.

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