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CLINICAL STUDY TO INVESTIGATE THE EFFICACY AND SAFETY OF WILATE DURING PROPHYLAXIS IN PREVIOUSLY TREATED PATIENTS WITH VON WILLEBRAND DISEASE (VWD)

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Clinical Research/Clinical Trials

Abstract

Clinical Study to Investigate the Efficacy and Safety of Wilate during Prophylaxis in Previously Treated Patients with von Willebrand Disease (VWD) Objectives: This study has a primary objective to determine the efficacy of VWF/FVIIII concentrate (Wilate) in the prophylactic treatment of previously treated patients with type 3, type 2 (except 2N), or severe type 1 VWD. Secondary objectives of this study will be to collect data to 1) Assess the VWF:Ac and VWF:Ag incremental IVR of VWF/FVIIII concentrate over time, 2) Assess the safety and tolerability of VWF/FVIIII concentrate in this indication. Also the study will examine, the efficacy of VWF/FVIIII concentrate in the treatment of breakthrough bleeding episodes (BEs), and in surgical prophylaxis, as well as the quality of life (QoL) during prophylaxis with VWF/FVIIII concentrate. Methods: The study is planned to enrol 28 PTPs aged ≥6 years and with VWD type 1, 2A, 2B, 2M, or 3.Eligible patients must be receiving on-demand treatment with a VWF-containing product, with at least 1, and an average of ≥2, documented spontaneous BEs per month in the preceding 6 months requiring treatment with a VWF-containing product. This will be assessed as part of a run in observational study to collect bleeding rate prior to the start of prophylaxis. From the beginning of the study, patients will receive prophylactic treatment with VWF/FVIIII concentrate for 12 months and record all BEs in a patient diary. Based on these data, the frequency of BEs and the annualized bleeding rate (ABR) under prophylactic treatment will be calculated. Treatment efficacy of BEs will be assessed by the patient (together with the investigator in case of on-site treatment) using a 4-point scale (excellent, good, moderate, none)In case patients undergo surgeries, efficacy of VWF/FVIIII concentrate will be assessed at the end of surgery by the surgeon and at the end of the postoperative period by the haematologist. In both cases, predefined assessment criteria will be used. In addition, an overall assessment of efficacy will be made at the end of the postoperative period by the investigator. Summary/conclusions: Prophylactic treatment in other congenital bleeding disorders is widely accepted as the standard of care to prevent bleeding and preserve quality of life in patients. This form of treatment in VWD is not well characterized prospectively as yet. This study will provide data on the efficacy of prophylactic treatment in reducing the rate of bleeding and on the impact of prophylaxis on the quality of life in VWD patients.