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Understanding the Disease Burden of Severe Von Willebrand Disease in a Resource Poor Country: An Indian Cohort Study

Shrinath Kshirsagar, MDMBBS[1], Nimish Kulkarni[1], Magdalne D'silva[1], Savita Rangarajan, s.rangarajan@soton.ac.uk[1], Shrimati Shetty[1]

[1] Advanced Center for Research in Oncology, Hematology and Rare Disorders (ACOHRD), K J Somaiya Medical College and Research Centre, Mumbai, India

Background : The national economic status impacts identification of rare diseases which also reflects access to standard healthcare infrastructure.Though VWD is the commonest bleeding disorder, the disease remains under-recognized and underdiagnosed in India due to its phenotypic and clinical heterogeneity and the need for multiple and complex diagnostic tests. The hospital based data shows a contrasting prevalence to that of the west, in that type 3 is the commonest subtype followed by type 1 and type 2 subtype. The treatment patterns of VWD too differs between countries and geographical regions. Unlike hemophilia, the bleed patterns and treatment details are not very well defined. There is a need to identify the unmet needs in the area of diagnosis and management.

Aim : The study is aimed to increase understanding of clinical manifestations, treatment practices along with Quality of life (qoL) in severe VWD patients from India

Methods : This is a cross-sectional, patient reported retrospective analysis of severe VWD patients from India. The eligibility criteria is confirmed diagnosis of VWD with documented laboratory reports. An informed consent was taken from each patient before participation in the study and the study was initiated after obtaining approval from Institute Ethics Committee. The EQ-5D instrumentswere used to measure patient reported health related QoL.

Results : A total of sixty two severe VWD patients were included in the present study. Mean age of the patients was 23.8 years (SD + 13.2 years); median 22 years (range 5-56 years) and the mean age of diagnosis was 4.9 years (SD + 6.9 years). The mean ABR was 12.6 (SD + 8.9). Twenty five patients (40%) had a positive family history and 37 (60%) of the patients reported parental consanguinity. Common bleeding symptoms were of mucocutaneous nature like epistaxis, ecchymoses and gingival bleed .Prolonged bleed from cuts was reported by 46 (74%) of patients and 14 out of 27 women (52%) in the reproductive age group complained of severe menorrhagia (Table 1). In approximately 76% of the patients, the major treatment product was intermediate purity FVIII whereas 3 patients were still being treated with either cryprecipitate or fresh frozen plasma. The EQ-5D data reported a worse health state in the EQ-5D-5L dimensions of pain/discomfort (34% moderate and 8% moderate to severe problems),usual activities (21% moderate problems and 3% moderate to severe problems), self-care (21% moderate problems) and anxiety (moderate 21% and 2% moderate to severe).There were two deaths due to bleeding in the previous 10 years .

Conclusion : In a resource limited country like India, type 3 is the predominant subtype presenting in the treatment centers. All patients had mucocutaneous bleeding with epistaxis being the predominant manifestation, but joint bleeding is also not uncommon. The study also reveals much lower than optimal QoL in all patients, mobility being the least affected parameter. Despite India being the pharmacy of the world, these conditions are neglected by stakeholders.

Disclosures No relevant conflicts of interest to declare.

Table 1. Bleeding manifestations in 62 severe VWD patients

Type of bleed	Number	Percentage
Epistaxis	25	40.30%
Ecchymosis	20	32.20%
Gingival Bleeding	37	59.60%
Bleeding from minor cuts	25	40.30%
GI Bleed	10	16.12%
Hemathrosis	10	16.12%
Hematuria	12	19.35%
Menorrhagia	14	53.84%

Figure 1

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