

DIAGNOSIS AND DISEASE BURDEN OF VON WILLEBRAND DISEASE IN A LARGE US POPULATION-BASED DATASET

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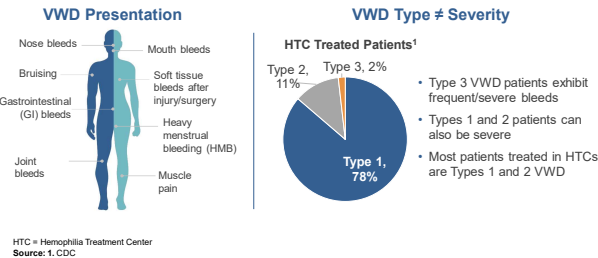
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INTRODUCTION

VWD is the most common inherited bleeding disorder

- Von Willebrand Disease (VWD) affects up to 1% of the population
- Patients experience excessive bleeding; bleed type, severity, and frequency vary
- However, diagnosis is often delayed, which interferes with effective management

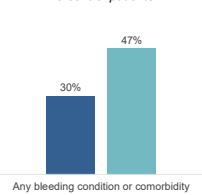


OBJECTIVE

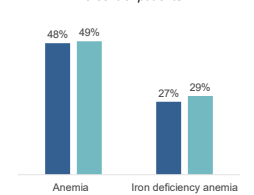
To further understand the VWD disease burden in the United States, we evaluated the largest, single-source, US VWD administrative and laboratory dataset to date

RESULTS

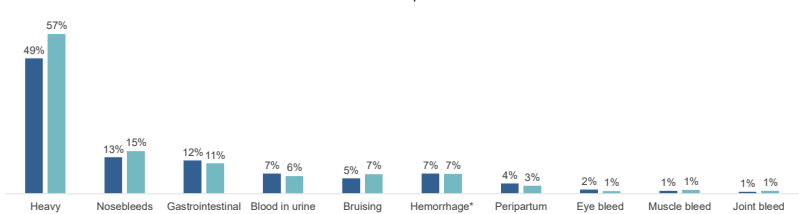
VWD PATIENTS WITH ANY BLEEDING COMORBIDITY
Percent of patients



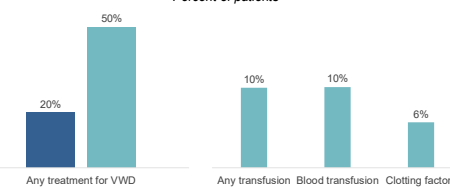
ANEMIA IN VWD PATIENTS WITH BLEEDING COMORBIDITY
Percent of patients



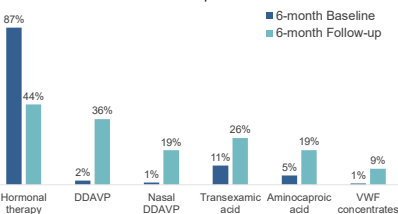
BLEED TYPES IN PATIENTS WITH BLEEDING COMORBIDITY
Percent of patients



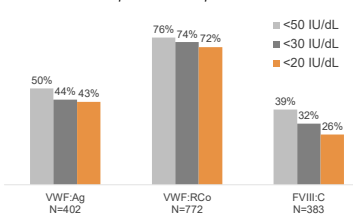
VWD PATIENTS TREATED WITH ANY TREATMENT AND/OR TRANSFUSION
Percent of patients



VWD TREATMENTS PRESCRIBED
Percent of patients



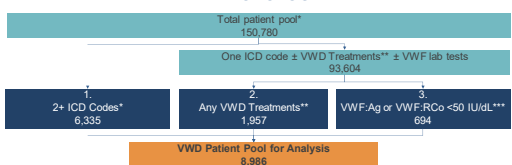
VWD LABORATORY VALUES
Percent of patients with reported test values



METHODS

VWD patients were identified from July 2007 to April 2023 in the Optum Claims Database using three methods. Patients were evaluated during a 6-month baseline period prior to first VWD claim and a 6-month follow-up period after first VWD claim

METHODOLOGY



Numbers presented differ from those in the abstract due to refined patient cohort and updated analyses

* Patient pool identification criteria: ≥1 medical claim with a VWD diagnosis (ICD-9-CM or ICD-10-CM) or ≥1 record with a VWD-specific lab result (i.e., VWF:Ag, VWF:RCO, and FVIII:C) or ≥1 medical claim for a VWD-specific lab test or ≥1 medical or pharmacy claim for VWD-specific medication. Continuous enrollment criteria of 6 months before and after index date (first claim date) were applied.

** VWD-specific treatments included: desmopressin, tranexamic acid, aminocaproic acid, recombinant VWF, and plasma-derived VWF/FVIII concentrates.

*** ± laboratory result for VWF tests taken during 6-month follow-up period.

PATIENT DEMOGRAPHICS

Demographics	Patients (n=8,986)	Demographics	Patients (n=8,986)
Age (mean)	38.8 years (SD 23.0 years)	Gender	
Age (median)	36.0 years (p25 18.0 years) (p75 57.0 years)	Female	72.9%
Age group		Male	27.1%
0-17	24.8%	Insurance	
18-44	35.5%	Commercial	79.3%
45-64	21.4%	Medicare	20.7%
65+	18.3%	Region	
		Northeast	20.2%
		Midwest	27.2%
		South	38.5%
		West	14.1%

LIMITATIONS

- We analyzed 6-month periods before and after first VWD-related claim over a 14-year timeframe in this analysis
 - Not all products currently used to treat VWD were available during the time span of the study
 - Some products may be initiated >6 months after diagnosis, not reported in the current analysis
 - Longitudinal analyses and patient journey will be shared in the future
- Both heavy menstrual bleeding and nosebleeds may be under captured in patient claims data and may precede and lead to a VWD diagnosis

CONCLUSIONS

- VWD patients experience varied bleeding types, severity, and frequency, which may result in delayed diagnosis and suboptimal management
- A large proportion of patients had VWF:Ag or VWF:RCO laboratory values <20 IU/dL, suggesting the prevalence of severe VWD (Wynn, ISTH 2024) may be higher than previously reported
- Almost half of VWD patients with bleeding comorbidities presented with anemia with a substantial proportion requiring blood transfusions
- While VWD diagnosis resulted in increased use of VWD therapies, treatment with VWF replacement remained low, likely due to availability and high burden of treatment
- There exists a high unmet need for better diagnostic vigilance and disease burden understanding to enable proper management of VWD patients