# Characterising the UK Population Living with von Willebrand Disease: Foundational Data for Health Economics and Outcomes Research

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Background & Aims: von Willebrand Disease (vWD) is a rare, inherited, heterogenous, and under-researched bleeding disorder. The **PIVOT-vWD** UK dataset aims to **characterise the patient community in the United Kingdom (UK),** informing **health economics and outcomes research (HEOR),** particularly in burden of illness, healthcare resource use, and patient preference.

Methods: Data were drawn from the UK cohort of the **PIVOT-vWD study** (collected Oct 24–Jan 25), a cross-sectional study co-developed with key opinion leaders within the vWD community to capture **the impact, voice, and outcomes** of people living with **vWD.** Invitations were distributed via a network of patient organisations. Participants (patients and/or caregivers) completed an encrypted online questionnaire. Individuals self-reported **demographic, clinical, and treatment characteristics, bleed impact, healthcare resource use,** outcome measures including **quality of life,** visual analogue scales (VAS), and treatment-focused **preference ranking** exercises. Descriptive statistics were used to summarise the cohort.

Figure 3 : vWD Type

Self-reported **vWD types** included: **Type 1** (28%), **Type 2** including subtypes (51%), **Type 3** (10%), **Acquired** and **Unreported** (11%). (Figure 3)

Figure 4: Location of Participants

Respondents were geographically dispersed **across the UK,** with the highest representation from **South East England** (22%). (Figure 4)

Treatment use was reported by 69%, and 76% had **accessed hospital-based care** in the past 12 months.

Figure 1: Gender Split

- Male
- Female

Sample includes 116 individuals (83% self-complete; 17% proxy-complete), with a mean age of 41.9 years (SD: 21.4), and **66% female.** (Figure 1)

Figure 2: Hospital Satisfaction

Mean satisfaction with hospital care was **7.2 out of 10** (SD: 2.6) on the VAS. (Figure 2)

Real-world patient and caregiver voices providing the foundation for future burden, preference and HRQoL research in vWD.

HEOR Use Cases utilising PIVOT-vWD
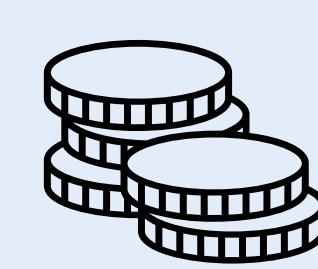
Economic Burden
Linking real-world healthcare resource use with unit costs to estimate direct medical costs

Patient Preference Insights
Unique ranking & rating preference data from people with vWD and caregivers
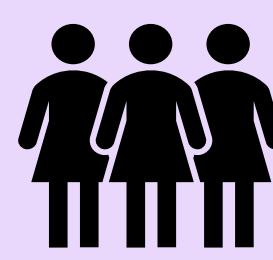
Health State Utilities
Dual generic utility measures (EQ-5D, AQoL-4D) to inform QALY modelling
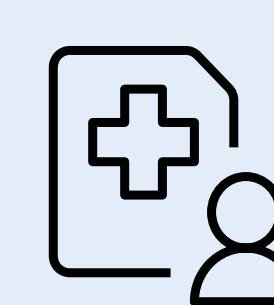
Work & Caregiver Impact
Capturing productivity loss (WPAI) and caregiver spillover effects, including time, QoL, and decision-making burden

Multi Country Expansion
Currently collecting data in the US, France, Spain, Germany and Italy

Diagnosis Insights
Patient reported diagnostic experience and gaps using qualitative questions.

Treatment & Management
Treatment satisfaction and adherence, and novel treatment perspectives

Community Engagement
Community based data capture, reaching those not regularly engaging with care settings

Conclusion: This dataset offers a comprehensive, real-world, **patient reported** resource characterising people living with vWD the UK, including perspectives from both **patients and caregivers.** Findings reveal variation in treatment exposure and hospital interaction,. PIVOT-vWD supports **future HEOR** by enabling robust analysis of service use, patient preference, and burden, grounded in the real-world patient voice of the vWD community.