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Unmet Needs of Patients with Severe Von Willebrand Disease & their Caregivers: A Qualitative Study on Current Standard of Care in Canada

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INTRODUCTION

Von Willebrand disease (VWD) is a hereditary bleeding disorder caused by a deficiency of von Willebrand factor[1]. While there is no cure for VWD, several treatment options, such as plasma-derived VWD (pdVWF), are available to manage the disorder.[2]

The impact of living with VWD and the associated burden of managing the disease are poorly understood.

This study was registered on ClinicalTrials.gov (NCT05695560).

OBJECTIVE

This qualitative research aimed to examine key areas of unmet needs and explore the experiences of patients living with severe VWD and their caregivers in Canada.

METHODS

Eligibility criteria

Patients:

- Adults age ≥ 18 years
- Severe VWD (self-BAT ≥ 10)
- Received pdVWF within the last 5 years

Caregivers: Current caregivers of VWD patients

Self-administered online questionnaire

Sociodemographics, comorbidities, VWD characteristics, quality of life.

Semi-structured telephone interview

Qualitative data on patients and caregivers collected by a research nurse

Virtual focus groups

Two focus groups of patients took place. Focus groups with caregivers did not take place due to small sample size.

Thematic analysis

Data analysis based on grounded theory using the constant comparison technique.

RESULTS

The study included 10 VWD patients and 2 caregivers.

Table 1. Sociodemographic characteristics of patients

Sex assigned at birth	n (%)
Female	7 (70.0)
Male	3 (30.0)
Employment status	**n (%)**
Employed full-time	1 (10.0)
Self-employed	4 (40.0)
Unemployed	1 (10.0)
Retired	4 (40.0)
Number of comorbidities	**n (%)**
None	1 (10.0)
1	1 (10.0)
2	3 (30.0)
≥ 3	4 (40.0)
Unknown	1 (10.0)
Geographical region in Canada	**n (%)**
Western	3 (30.0)
Central	7 (70.0)

Both caregivers (1 spouse, 1 parent) were males age > 50 years. One resided in Central Canada and the other in Western Canada.

Overall, patients scored least favourably for SF-36 items related to limitations due to physical health and pain (Table 2).

Table 2. HRQoL scores of individual domains of SF-36

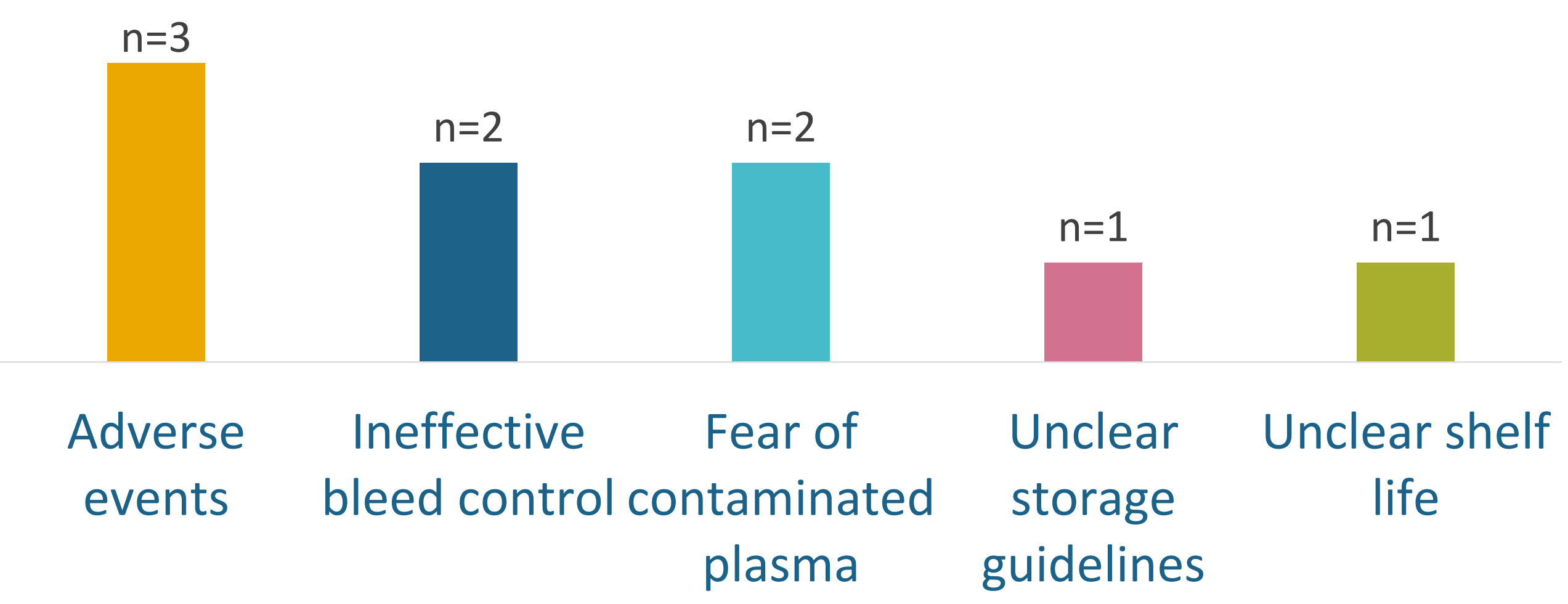
Domain	Number of items	Mean score (SD)	Range
Physical functioning	10	74.0 (25.5)	15-100
Emotional well-being	5	70.8 (17.4)	52-100
Health change	1	70.0 (30.7)	25-100
Social functioning	2	65.0 (26.9)	13-100
Role limitations due to emotional problems	3	60.0 (43.9)	0-100
General health perceptions	5	58.5 (24.8)	5-100
Energy/fatigue	4	50.5 (23.9)	10-80
Pain	2	47.0 (24.9)	0-80
Role limitations due to physical health	4	32.5 (42.6)	0-100

Female patients aged 18-49 years (n=4) scored the lowest for the domains of role limitations due to physical health: 0 (SD: 0) and pain: 29.0 (SD: 28.3) compared to other patients, likely due to menorrhagia

Patient Satisfaction with pdVWF therapies

Based on interviews and focus groups, 7 of the 10 patients recounted feelings of satisfaction toward pdVWF, mainly driven by treatment effectiveness. However, 6 patients also described feelings of dissatisfaction, with reasons shown below.

Figure 1. Reasons for dissatisfaction with pdVWF*

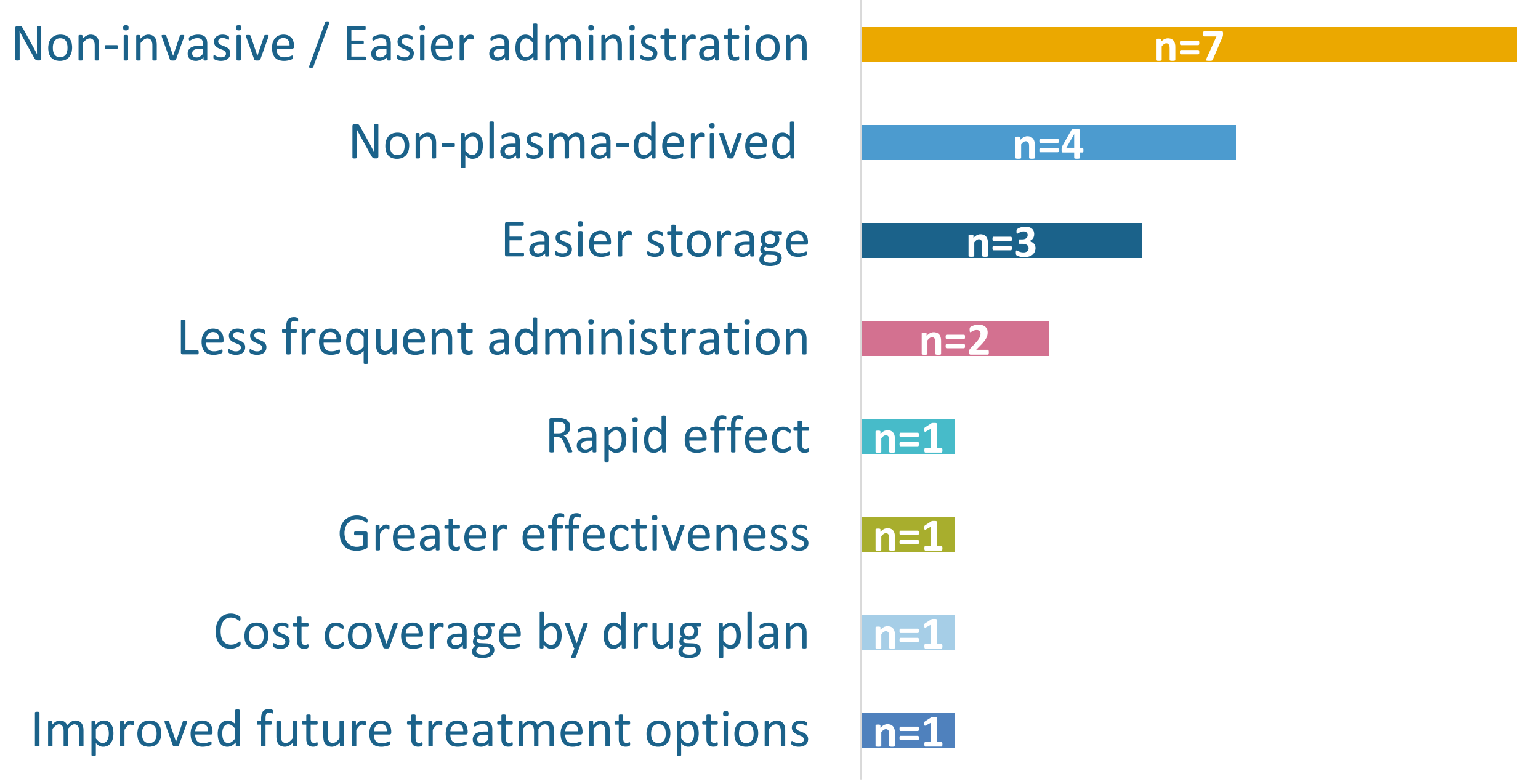


Responses are not mutually exclusive

Patient Expectations for New Treatments

Across the interviews and focus groups, 8 patients expressed several different expectations and needs for new VWD treatments.

Figure 2. Patient expectations for new treatments*



**Responses are not mutually exclusive*

Mental Health Burden

The majority of patients and both caregivers experience negative impacts to mental health due to VWD.

Among patients:

Anxiety, stress, reduced emotional well-being, dismissal, depression

Among caregivers:

Stress and anxiety

All female patients aged 18-49 years (n=4) experienced important impact on mental health and quality of life due to menorrhagia.

Unmet Needs among Patients and Caregivers

Improved education on VWD among health care providers

The most common theme among patients was the perceived lack of knowledge on VWD among healthcare providers, resulting in long delays in treatment.
This was echoed by caregivers.

> *Most [doctors] still have no idea what this [VWD] is. [They] still don't know how to treat it, and I think that's appalling [...] I've met too many medical [professionals] who don't have a clue [...] And don't take me seriously.*
>
> *Female patient*

Improved access to healthcare and treatments

- Better health insurance coverage
- Reduced delay to treatment initiation
- Financial aid
- Support with transportation to medical services

Greater awareness of VWD

Another common theme among patients and caregivers was the need for greater awareness of VWD in the general population and increased research initiatives.

In particular, among female patients or patients who menstruate, a greater awareness and understanding of menorrhagia is needed.

CONCLUSIONS

Mental health burdens due to VWD were common among all participants, especially among female patients of reproductive age.

Most patients were satisfied with current pdVWF treatments. However, there is a clear preference for less invasive treatment options.

There was a universal call for enhanced VWD education and awareness among healthcare professionals and the population.

Despite the small sample size, this qualitative study suggests that VWD imposes a burden on the daily lives and well-being of both patients and caregivers in Canada.

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DISCLOSURES

Tiffany Cristarella & Yola Moride: employees of YolaRX Consultants, which received consulting fees for the conduct of this study
David Lillicrap: No conflicts of interest to declare
Julia Hews-Girard: received honoraria from Takeda and research funding from CSL Behring
Debbie Lim: employee of Takeda Canada Inc., and shareholder of Takeda
Abhinav Wadhwa: employee of Takeda Canada Inc.

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