

Redefining Treatment Satisfaction and Its Impact on Treatment Adherence and Value for Persons with Hemophilia: Findings from the HemACTIVE Study

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CONCLUSIONS

- Treatment adherence appears to be linked to treatment satisfaction--which persons with hemophilia (PwH) are more likely to define according to tolerability and/or efficacy of a product than impact on quality of life
- By redefining treatment satisfaction to encompass freedom and flexibility in lifestyle, there is an opportunity to improve adherence, thus improving value of treatment

OBJECTIVE

The HemACTIVE patient survey was used to gain insight on how PwH define treatment satisfaction and its impact on adherence

INTRODUCTION

- Prophylaxis with replacement FVIII is standard of care for hemophilia but frequent injections contribute to a treatment burden that can lead to poor adherence resulting in increased bleeding risk, pain, and poor health-related quality of life¹⁻³
- Adherence to prescribed replacement factor prophylaxis regimens is prognostic of several outcomes (bleeding, arthropathy, quality of life) in persons with hemophilia (PwH), including reduced acute and chronic pain, yet rates of nonadherence range from 30% to 73%⁴
- Adherence is an important determinant in cost-effective analyses, which often assume perfect adherence when establishing value. There is growing interest in understanding the value of adherence-enhancing interventions for improving health outcomes⁵
- The HemACTIVE patient survey was designed to evaluate the impact of hemophilia and its treatments on daily activities in PwH. The survey included questions on satisfaction with current treatment and treatment adherence behavior, thus enabling an analysis on the relationship between the two

METHODS

Participants and Study Design

- Participants were adult PwH or caregivers (for children <18 years), diagnosed with moderate or severe hemophilia A and receiving prophylaxis or on-demand treatment with no clinically significant inhibitors
- HemACTIVE was a 25-minute cross-sectional internet survey administered to PwH (hemophilia A) and their caregivers
- Participants were recruited from rare disease panels from France, Italy, Germany, US, and Canada from March 2018 to April 2019
- The study protocol and questionnaire were reviewed and approved by respective local ethics committees

Questionnaire and Statistical Analysis

- The questionnaire was developed by the authors and other key opinion leaders. It included sections on:
 - Current and aspirational activity level;
 - Influence of hemophilia on daily activities;
 - Impact of treatment on daily activities; and
 - Demographics
- Questions consisted of yes/no, multiple choice, and rating scale questions. A 5-level scale was used to report activity levels
- Planned sample size was based on feasibility estimation and expectation of enrollment
- No formal statistical hypotheses were tested, and all analyses were descriptive in nature only
- Adherence behavior was evaluated in PwH whose participation in activities was impacted by pain

RESULTS

Demographics and Patient Characteristics

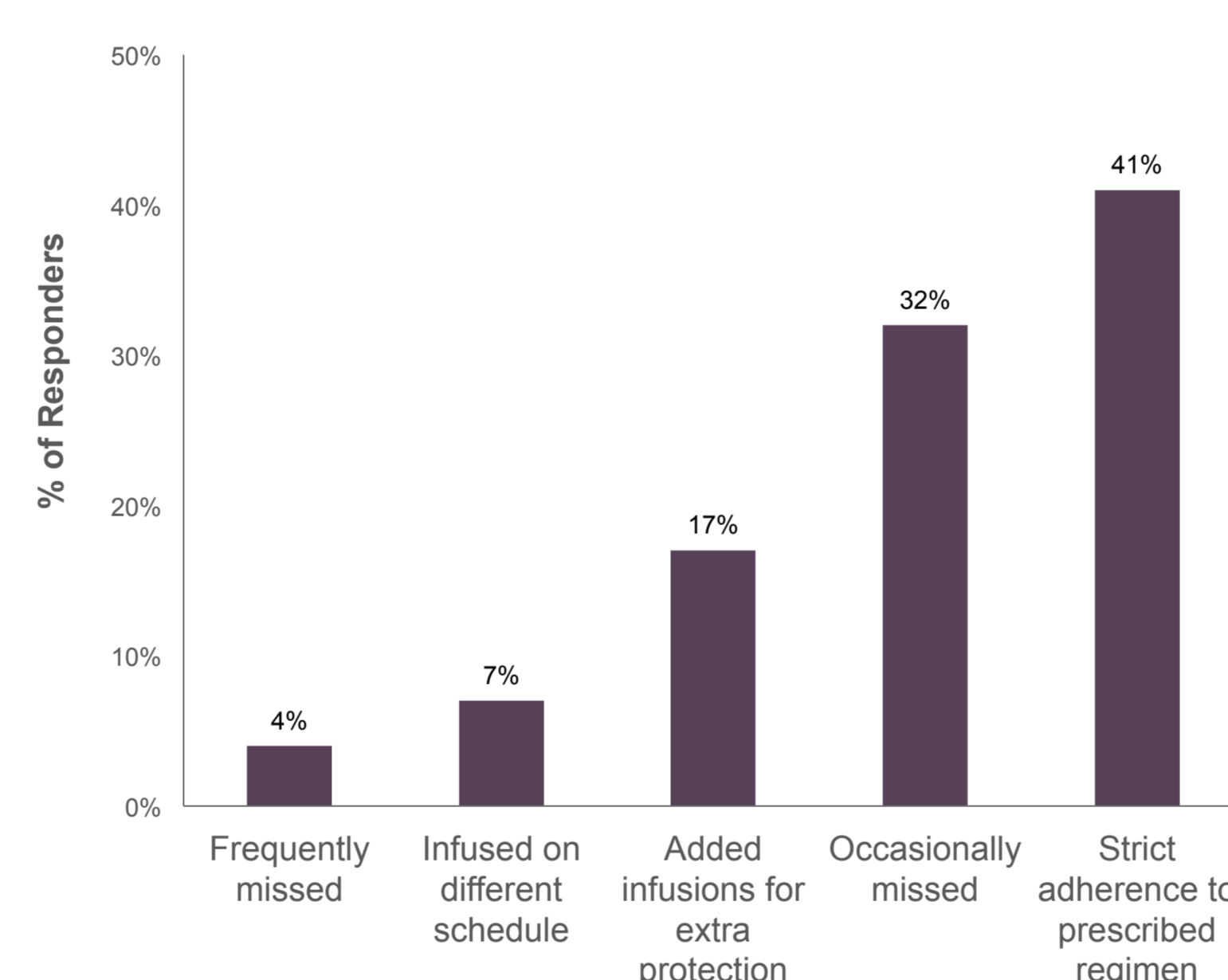
Table 1: DEMOGRAPHICS AND PATIENT CHARACTERISTICS

	France	Italy	US	Germany	Canada	TOTAL
Participants, n	39	60	110	26	41	275
Age ranges, n (%)						
2-17 (Caregiver)	13 (33)	4 (7)	41 (37)	5 (20)	18 (44)	81 (29)
18-30	8 (21)	19 (32)	34 (31)	11 (44)	8 (20)	80 (29)
31+	18 (46)	37 (62)	35 (32)	9 (36)	15 (37)	114 (41)
Severity, n (%)						
Moderate	15 (38)	23 (38)	13 (12)	NA	32 (78)	83 (30)
Severe	24 (62)	37 (62)	97 (88)	NA	9 (22)	167 (70)
Treatment, n (%)						
Prophylaxis	27 (69)	47 (78)	97 (88)	23 (92)	34 (83)	185 (67)
On-demand	12 (31)	13 (22)	13 (12)	2 (8)	7 (17)	47 (33)

Treatment Adherence

- Across 231 responses, 41% adhered strictly to prescribed treatments, while 4% frequently missed and 32% occasionally missed infusions; 7% infused on a different schedule and 17% added infusions for extra protection (Figure 1)

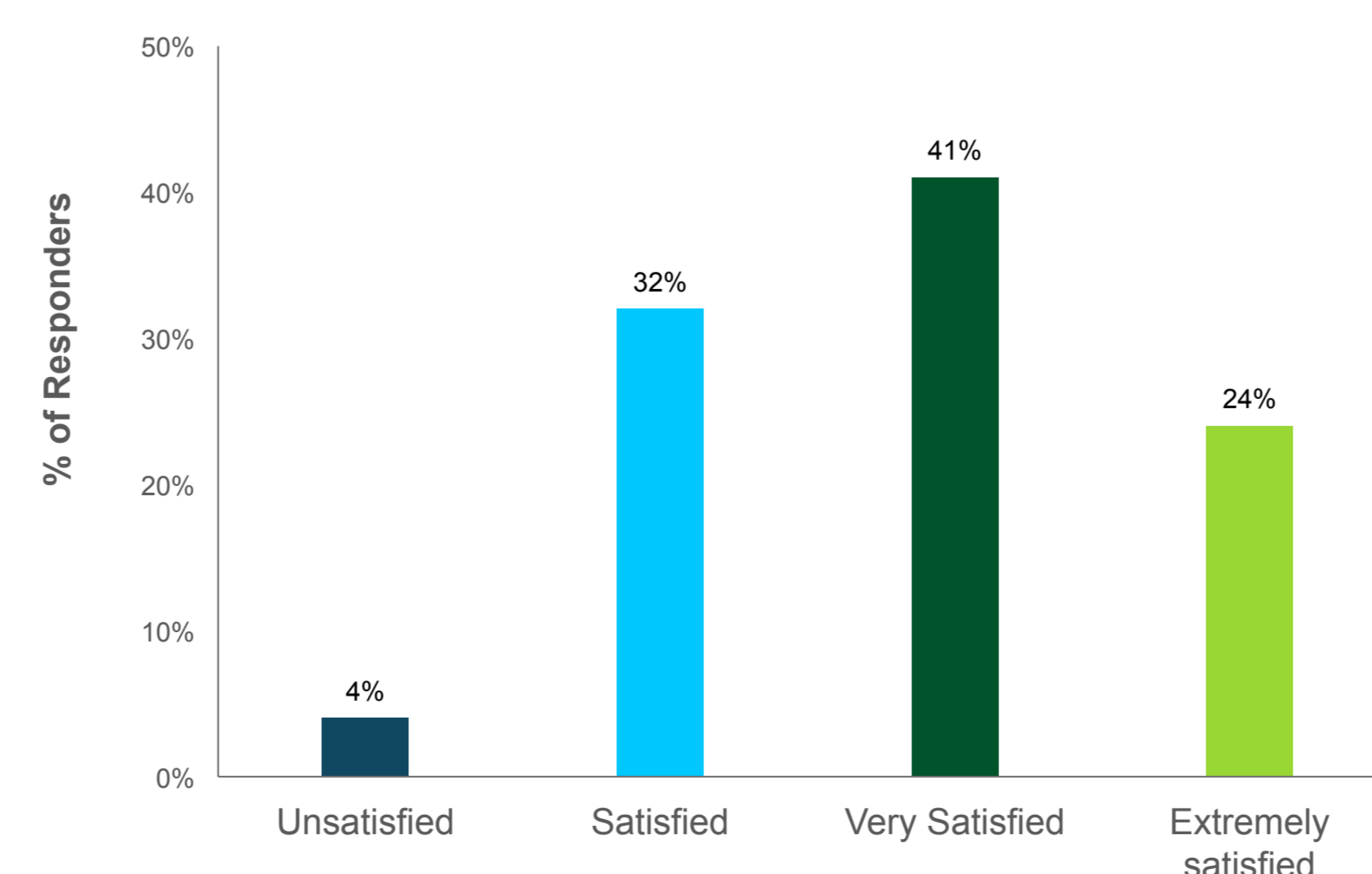
Figure 1: Treatment Adherence Behavior



Treatment Satisfaction

- Most were satisfied with current treatment, with varying degrees of satisfaction (24% extremely satisfied, 41% very satisfied, 32% satisfied, 4% unsatisfied) (Figure 2)

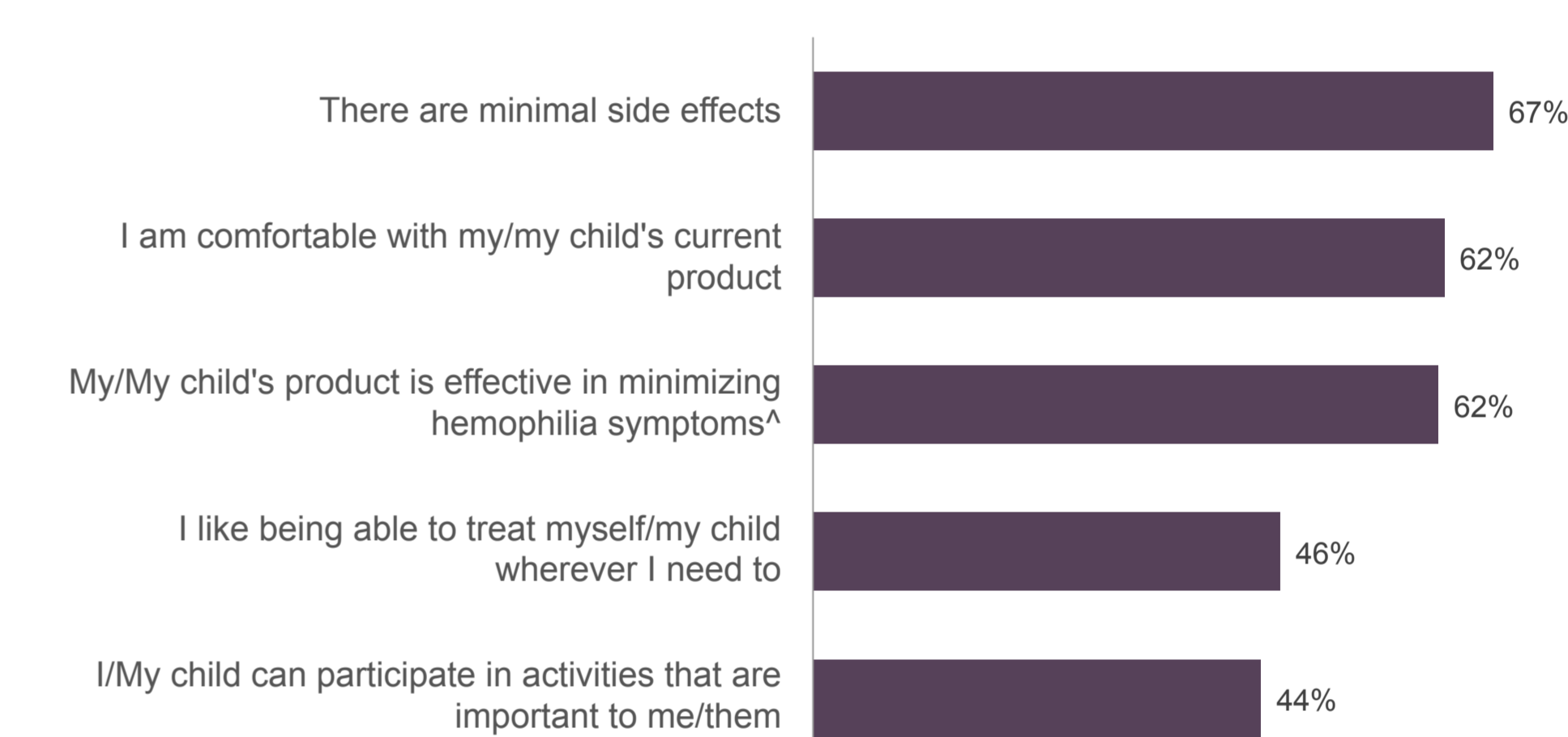
Figure 2: Most Survey Participants Were Satisfied With Their Current Treatment



Treatment Adherence and Treatment Satisfaction

- PwH were more likely to cite tolerability or efficacy as reasons for treatment satisfaction than its impact on freedom from restrictions (Figure 3)
 - Tolerability: minimal side effects 67% and comfort 62%
 - Efficacy: symptom minimization 62%
 - Freedom from restrictions: ability to treat whenever needed 46% and ability to participate in activities 44%

Figure 3: Reasons Given for Satisfaction with Product



Note: Survey participants could select more than one response.

Impact of Treatment Satisfaction on Adherence

- Strict adherence to prescribed regimens improved with degree of treatment satisfaction (Figure 4)
 - Strict adherence was reported by 18% of unsatisfied PwH, compared with 36% vs 41% vs 52% for satisfied, very satisfied, and extremely satisfied PwH, respectively
- PwH who were unsatisfied with their treatment were more likely to add infusions or frequently miss scheduled infusions
 - 28% of PwH who were unsatisfied reported adding more infusions than prescribed, compared with 23%, 15%, and 8% of satisfied, very satisfied, and extremely satisfied PwH, respectively
 - 18% of PwH who were unsatisfied reported frequently missing scheduled infusions, compared with 4%, 4%, and 2% of satisfied, very satisfied, and extremely satisfied PwH respectively
- Occasional missed infusions were similar across PwH regardless of treatment satisfaction (range: 28%-36%)

Figure 4: Adherence Behavior Improves With Treatment Satisfaction

