A real-world study of haemophilia treatment satisfaction, bleeds and health-related quality of life as reported by patients and physicians to demonstrate gaps

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Objective

- This study aimed to explore patient and physician satisfaction with current prophylactic treatments, including bleeding rates and health-related quality of life (HRQoL).

Conclusions

- Prophylactic treatment adherence was greater among patients where physicians reported higher treatment satisfaction.
- The reported Haem-A-QoL "treatment" domain mean total score suggests there is a remaining unmet need for haemophilia treatments.
- Patient and physician discordance in the reporting of treatment satisfaction indicates that increased communication could facilitate better disease management and understanding of treatment efficacy.
- Future research could look at reasons for treatment satisfaction and perceptions of disease management.

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Disclosures:

- ST, VM, LW and AYS are employees of Pfizer Inc., New York, United States of America.
- EM, NB, SL ,CB, RS and KWC are employees of Adelphi Real World, Bollington, United Kingdom.

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Introduction

- Haemophilia is an inherited genetic disorder due to a defect in factors VIII or IX, respectively.
- The hallmarks of haemophilia include bleeds and joint problems[1].
- Treatments consist of prophylactic and on-demand options, including standard and extended half-life factor and non-factor therapies[2].
- For treatment to be effective in improving quality of life, adherence is crucial.
- Adherence to treatment can be impacted by multiple variables including form of administration[3].
- There is a lack of comprehensive data examining both physicians and patient perspectives that can be used to give insight into current treatment adherence, satisfaction and patient quality of life.

Methods

- Data were drawn from the Adelphi Real World Haemophilia Disease Specific Programme (DSP), a cross-sectional survey with elements of retrospective data collection from physicians and 10 consulting haemophilia A and B patients in France, Germany, Italy, Spain, and the United States (September 2023–April 2024).
- The DSP™ has been previously published, validated, and proven to be consistent over time[4,5,6,7].
- Physicians provided information on patient demographics, satisfaction with treatment, and bleeding episodes.
- Patients independently self-reported their quality of life via the Haem-A-Qol[8], a ten domain HRQoL patient-reported outcome measure. The "treatment" domain was analysed, with scores transformed to a scale of 0 (no impairment) to 100 (the most/extreme impairment to HRQoL). Analyses were descriptive.

Limitations

- Physicians completed surveys for their next consecutively consulting patients, meaning patients with more severe disease, who may consult more frequently, are more likely to be captured within the DSP.
- Recall bias, a common limitation of surveys may have affected results. This was minimised by physicians' ability to refer to the patients' records.
- Physician participation was limited to inclusion criteria, was voluntary, and influenced by their willingness to participate.

Results

- Overall, 45 physicians provided data for 222 patients with self-reported data, 93% White and mean [standard deviation (SD)] age 27.5 [9.9]. These same patients provided self-reported data, allowing for matched comparisons between the physician and patient. Patient's haemophilia type and current prophylactic treatment split by patient- and physician-reported prophylactic treatment satisfaction are shown in **Table 1**.
- Of all patients, 64% reported they were not completely satisfied (NCS) with their prophylaxis and 36% were completely satisfied (CS). Of all patients, 64% also reported they were not completely satisfied with how their medication prevents bleeds.
- In contrast, physicians reported they were NCS with 35% of their patients' prophylaxis and CS with 65%.
- Patients and physicians reported percentage of prophylactic treatment doses taken on time. **Figure 1** shows the percentage reporting that they take all doses on time, split by physician reported satisfaction.
- Patients reported a mean [SD] Haem-A-QoL "treatment" domain impairment score of 38.5 [19.7]. Further splits based on reported levels of prophylactic treatment satisfaction and total transformed scores are shown in **Figure 2**.
- In total, 65% of patients reported they 'always' tell their physician when they experience a bleed (patient reported - CS: 72%, NCS: 61%)
- In the 12 months prior to survey, patients reported a mean [SD] of 2.9 [2.9] bleeds, while in the same time period, physicians for these patients' reported bleeds of 2.4 [2.9].
- **Figure 3** shows number of physician reported bleeds split by prophylactic treatment satisfaction.

Table 1: Haemophilia type and treatment overview

		Patient-reported prophylactic treatment satisfaction		Physician-reported prophylactic treatment satisfaction	
	Overall (n=222)	**Completely satisfied (n=80)**	**Not completely satisfied (n=142)**	**Completely satisfied (n=144)**	**Not completely satisfied (n=78)**
Haemophilia type, n (%) Haemophilia A	182 (82)	62 (78)	120 (85)	115 (80)	67 (86)
Current prophylactic, n (%)					
SHL	134 (60)	52 (65)	82 (58)	97 (67)	37 (47)
EHL	105 (47)	35 (44)	70 (49)	65 (45)	40 (51)
NFT	56 (25)	19 (24)	37 (26)	41 (28)	15 (19)
Other	15 (7)	6 (8%)	9 (6)	12 (8)	3 (4)

SD; standard deviation, SHL; Standard half-life factor replacement, EHL; Extended half-life factor replacement, NFT; Non-factor therapies

Figure 1: Patient and physician-reported % of patients who take all their treatment doses on time, split by physician prophylactic treatment satisfaction

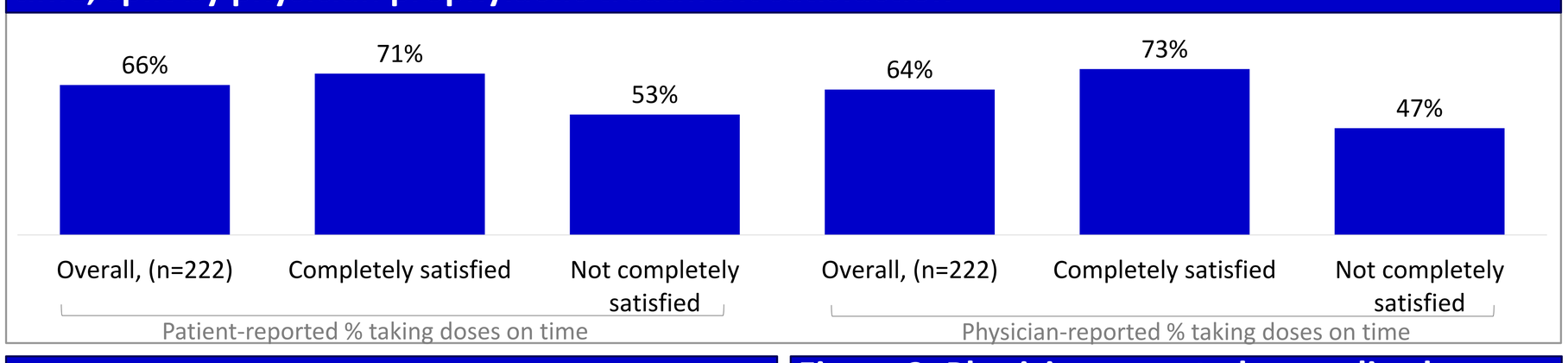

Figure 2: Patient-reported Haem-A-Qol scores, split by prophylactic treatment satisfaction

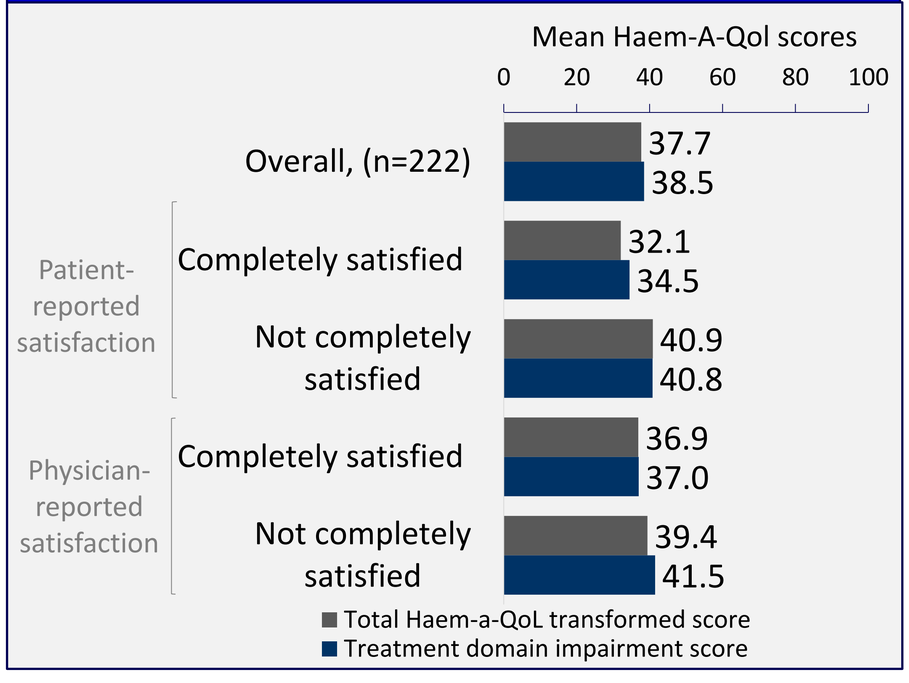

Figure 3: Physician-reported annualised bleeding rate, split by prophylactic treatment satisfaction