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Using the Hemophilia Functional Ability Scoring Tool (Hemo-FAST) to describe the joint health status in adults with hemophilia

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CONCLUSIONS

- Despite advances in hemophilia treatment, the burden of disease of a representative European hemophilia population was demonstrated to be high in persons with hemophilia A and B.
- Hemo-FAST is a newly developed quick and easy-to-use tool to evaluate joint status contributing to assessing the burden of disease in persons with hemophilia by capturing both patient and clinician perspectives.

BACKGROUND

- Despite increased availability of treatment options, persons with hemophilia A (PwHA) and B (PwHB) still experience a significant burden of joint disease.[1]
- Standard tools for joint health assessment are often time consuming, complex, and do not capture both the perspectives of persons with hemophilia and clinicians.[2,3]
- The Hemophilia Functional Ability Scoring Tool (Hemo-FAST), consisting of a patient-reported outcome (PRO) part and a clinician-reported outcome (ClinRO) part, is a newly developed quick and easy-to-use tool to evaluate joint status in clinical practice.[4]
- A recent study validated the psychometric properties of Hemo-FAST for assessment of joint health in persons with hemophilia.[4]

AIMS

- The aim of this analysis was to describe the burden of disease in a population of PwHA and PwHB, including joint health, using data collected through the Hemo-FAST validation study (NCT04731701, funded by Sobi).

METHODS

- For the Hemo-FAST validation study, between May 2021 and July 2022 adult PwHA and PwHB were recruited in French study sites where:
  - Patients completed the PRO part of Hemo-FAST and the short-form 36 health survey questionnaire (SF-36).
  - Clinicians assessed the participants' joint health using the ClinRO part of Hemo-FAST and the Haemophilia Joint Health Score (HJHS).
- Demographics (gender, age, and body mass index) and medical history (disease severity, hemophilia type, treatment regimen, and joint bleeding episodes ≤12 months prior to enrollment) were also collected. Descriptive analyses by hemophilia type are presented.

RESULTS

Demographics

- Of the 180 adults with hemophilia recruited into the study, 149 (82.8%) were PwHA and 31 (17.2%) were PwHB. The baseline demographics and characteristics were largely comparable between PwHA and PwHB (**Table 1**).

Table 1. Baseline demographics and characteristics

	Hemophilia A (n=149)	Hemophilia B (n=31)	Total population (N=180)
Male/female, % (n)	99.3 (148)/ 0.7 (1)	100.0 (31)/ 0.0 (0)	99.4 (179)/ 0.6 (1)
Age (years), median (range)	39.0 (18.0–78.0)	34.0 (18.0–77.0)	38.0 (18.0–78.0)
BMI (kg/m²), median (range)	24.5 (15.4–41.8)*	25.5 (17.6–35.4)†	24.7 (15.4–41.8)‡
Severity of hemophilia,§ % (n)			
Severe	66.4 (99)	51.6 (16)	63.9 (115)
Moderate	14.1 (21)	29.0 (9)	16.7 (30)
Mild	19.5 (29)	19.4 (6)	19.4 (35)
Current treatment regimen, % (n)			
Prophylaxis	63.1 (94)	54.8 (17)	61.7 (111)
On-demand treatment	36.9 (55)	45.2 (14)	38.3 (69)
Type of prophylaxis, % (n)			
Primary	7.4 (7)‖	23.5 (4)¶	9.9 (11)**
Secondary	63.8 (60)‖	47.1 (8)¶	61.3 (68)**
Tertiary	27.7 (26)‖	23.5 (4)¶	27.0 (30)**
Unknown	1.1 (1)‖	5.9 (1)¶	1.8 (2)**
Number of joint bleedings in the 12 months prior to enrollment, % (n)			
No bleeding	58.4 (87)	64.5 (20)	59.4 (107)
1–5 bleeding(s)	28.9 (43)	29.0 (9)	28.9 (52)
≥6 bleedings	4.0 (6)	0.0 (0)	3.3 (6)
Unknown	8.7 (13)	6.5 (2)	8.3 (15)

*n=133; †n=28; ‡n=161; §Severe hemophilia, <1% basal factor VIII or IX; moderate hemophilia, 1–5% basal factor VIII or IX; mild hemophilia, >5% to <40% basal factor VIII or IX; ‖n=94; ¶n=17; **n=111
BMI, body mass index

Table 2. Hemo-FAST, HJHS and SF-36 PCS scores of the total study population by type of hemophilia and further stratified by severity or current regimen

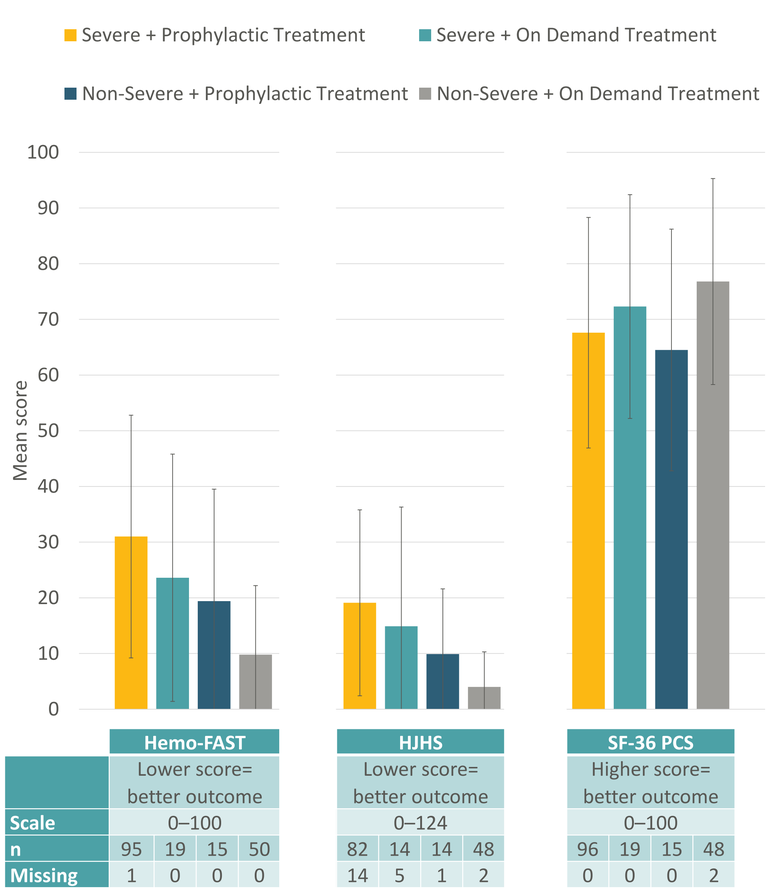
	Hemo-FAST		HJHS		SF-36 PCS	
	Hemophilia A (n=149)	Hemophilia B (n=31)	Hemophilia A (n=149)	Hemophilia B (n=31)	Hemophilia A (n=149)	Hemophilia B (n=31)
	n=148*	n=31	n=131†	n=27‡	n=148*	n=30*
Mean (SD)	26.0 (23.1)	20.8 (22.4)	14.4 (16.3)	8.3 (12.3)	69.5 (20.0)	74.2 (21.9)
Median (range)	19.8 (0.0–84.0)	8.3 (0.0–72.2)	10.0 (0.0–82.0)	2.0 (0.0–45.0)	72.5 (13.0–98.8)	80.6 (12.3–100.0)
Severe hemophilia	n=98*	n=16	n=82§	n=14‖	n=99	n=16
Mean (SD)	30.4 (21.7)	25.7 (23.5)	19.3 (17.7)	13.5 (14.9)	68.0 (20.3)	70.5 (22.9)
Median (range)	31.7 (0.0–78.2)	20.2 (1.9–66.7)	14.0 (0.0–82.0)	7.5 (0.0–45.0)	72.3 (13.0–98.0)	79.8 (12.3–94.3)
Non-severe hemophilia	n=50	n=15	n=49*	n=13‖	n=49*	n=14*
Mean (SD)	11.9 (15.0)	12.5 (15.1)	6.1 (8.8)	2.6 (4.2)	72.6 (19.3)	78.4 (20.8)
Median (range)	4.8 (0.0–71.8)	6.4 (0.0–48.1)	2.0 (0.0–39.0)	0.0 (0.0–14.0)	73.3 (30.5–98.8)	83.4 (24.0–100.0)
Prophylaxis	n=93*	n=17	n=81¶	n=15‖	n=94	n=17
Mean (SD)	29.6 (21.8)	28.2 (22.8)	18.5 (16.6)	13.7 (14.3)	67.3 (20.1)	66.5 (24.2)
Median (range)	28.9 (0.0–78.2)	31.4 (1.9–66.7)	14.0 (0.0–65.0)	12.0 (0.0–45.0)	70.3 (13.0–98.0)	71.0 (12.3–94.3)
On demand	n=55	n=14	n=50**	n=12‖	n=54*	n=13*
Mean (SD)	14.9 (17.7)	8.5 (11.0)	7.7 (13.3)	1.5 (2.6)	73.4 (19.4)	84.2 (13.8)
Median (range)	6.7 (0.0–60.3)	5.1 (0.0–38.5)	3.0 (0.0–82.0)	0.0 (0.0–8.0)	78.1 (29.5–98.8)	87.8 (55.3–100.0)

*One missing; †18 missing; ‡Four missing; §17 missing; ‖Two missing; ¶13 missing; **Five missing. A lower Hemo-FAST (scale 0–100) and HJHS (scale 0–124) score corresponds to better outcomes, whereas a higher SF-36 PCS (scale 0–100) score corresponds to better outcomes. Non-severe hemophilia includes both mild and moderate hemophilia
Hemo-FAST, Hemophilia Functional Ability Scoring Tool; HJHS, Haemophilia Joint Health Score; SD, standard deviation; SF-36 PCS, short-form 36 health survey physical component summary

Burden of disease stratified by hemophilia type, severity, and treatment regimen

- The burden of disease in PwHA and PwHB was demonstrated by the mean (standard deviation) scores for Hemo-FAST of 26.0 (23.1) and 20.8 (22.4), HJHS of 14.4 (16.3) and 8.3 (12.3), and SF-36 physical component summary (SF-36 PCS) of 69.5 (20.0) and 74.2 (21.9), respectively (**Table 2**).
- For both hemophilia types, severe hemophilia was associated with higher Hemo-FAST and HJHS scores compared with non-severe hemophilia, and, unexpectedly, prophylactic treatment was associated with higher scores compared with on-demand treatment (**Table 2**). This might reflect the burden of disease in this adult population, which is mostly on secondary or even tertiary prophylaxis.
- When stratifying the whole population by severity and treatment type, Hemo-FAST and HJHS scores, but not SF-36 PCS, appear to display similar trends across categories, although the high variability and lack of follow-up measurements limit the interpretation of these results (**Figure 1**).

Figure 1. Hemo-FAST, HJHS, and SF-36 PCS scores of the total study stratified by severity and current regimen



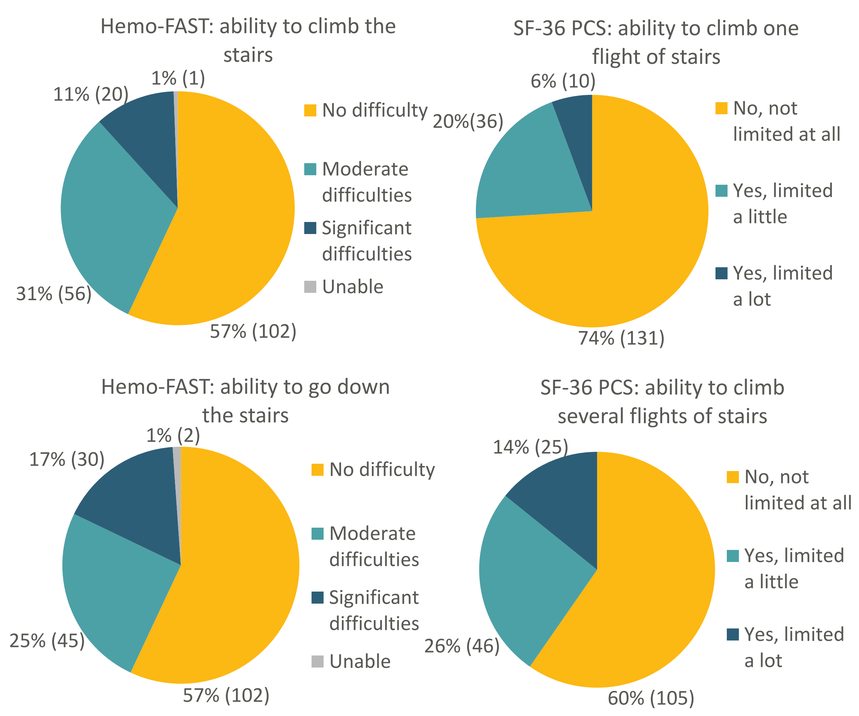
Non-severe hemophilia includes both mild and moderate hemophilia. Error bars depict ± SD
Hemo-FAST, Hemophilia Functional Ability Scoring Tool; HJHS, Haemophilia Joint Health Score; SD, standard deviation; SF-36 PCS, short-form 36 health survey physical component summary

Comparison of specific questions in Hemo-FAST and SF-36 PCS

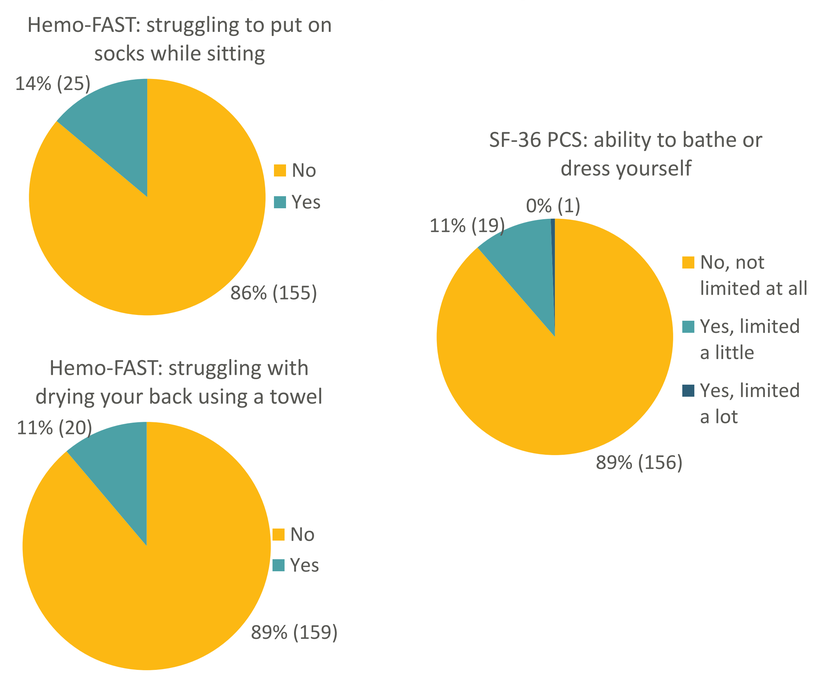
- Results from similar questions used in Hemo-FAST and SF-36 PCS have been grouped together relating to ability to use stairs (**Figure 2A**) and ability to dress oneself and bathe (**Figure 2B**). Overall, the results show similar trends across the two tools.

Figure 2. Specific questions in Hemo-FAST and SF-36 PCS

A. Questions relating to stairs [% (n)]



B. Questions relating to dressing and bathing [% (n)]



Hemo-FAST, Hemophilia Functional Ability Scoring Tool; SF-36 PCS, short-form 36 health survey physical component summary

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