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Clinical, Psychosocial, Quality of Life Outcomes and Patient-Physician Discrepancies in Joint Damage Reporting in a cohort of People with Haemophilia A: real-world insights from the CHESS III Study

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INTRODUCTION

- Joint health deterioration is a burdensome consequence of haemophilia A (HA), as recurrent joint bleeding can lead to progressive joint damage and functional limitations [1,2].
- If joint health is not routinely assessed in clinical practice, misinterpretation of joint symptoms and perception discrepancies between people with HA (PwHA) and their health care providers (HCPs) may lead to under-detection, as well as suboptimal management.
- PwHA who perceive more joint issues than are detected/reported by HCPs may experience challenges in clinical outcomes, quality of life, and psychosocial well-being, potentially leading to an increased disease burden.
- This analysis investigated clinical, health-related quality of life (HRQoL), and psychosocial outcomes in PwHA recruited as part of the CHESS III (Cost of Haemophilia: a socioeconomic survey III) study, based on the agreement or lack thereof between PwHA and HCPs, in reports of joint damage.

METHODS

- The CHESS III study was a burden-of-illness study that recruited adult male individuals diagnosed with congenital haemophilia A or B of any severity, with or without inhibitors, from France, Germany, Spain, Italy and United Kingdom, between 2022-2023.
- This current analysis focused on an anonymized sample of individuals with haemophilia A without inhibitors from this dataset.
- The CHESS III study employed two linked questionnaires for data collection (each questionnaire completed by HCP for a PwHA was linked to a questionnaire completed by the same PwHA):
  - A web-based case report form (CRF), completed by the HCP, capturing information such as medical history and consultations, clinical information, joint health, and healthcare resource use of each participant.
  - A patient and public involvement and engagement (PPIE) questionnaire completed by the PwHA, which captured clinical information, impact on daily life/compromise due to haemophilia, as well as health-related quality of life (HRQoL), via the following patient-reported outcome (PRO) tools:
    - EuroQoL EQ-5D-5L tool, assessing overall quality of life through five dimensions of health across five severity levels and including a 0–100 health rating scale [3],
    - Generalised Anxiety Disorder 7-item scale (GAD7): an anxiety-assessment instrument consisting of 7 items rated from 0-3 based on severity, with higher scores indicating worse outcomes [4], and
    - Patient Health Questionnaire-8 (PHQ-8): a depression-assessment instrument measuring symptom severity across eight items (total 0–24), with higher scores indicating worse outcomes [5].
- Cross-sectional data on adult PwHA without inhibitors were extracted from the dataset and were analysed descriptively: continuous and categorical variables were reported as mean (SD) or n (%). Subgroup differences were tested for significance via t-test for continuous variables and chi2 test for categorical variables.
- Reporting agreement between PwHA and their HCPs was assessed by comparing PJ reports, with three situations emerging: PwHA reporting less PJs, the same number of PJs, or more PJs than their HCP.
- While for a small sample (n=10) it was noted PwHA reported less PJs than the HCP, in this case the clinician would be aware of their situation, and therefore it may be considered "appropriately assessed". As such, data on PROs and key clinical outcomes are presented for two mutually exclusive groups:
  - PwHA reporting less or the same number of PJs as their HCP (the "concordance" group) and,
  - PwHA reporting more PJs than their HCP (the "discordance" group).

Problem Joints (PJ):

Joints with chronic pain and/or limited range of movement due to compromised joint integrity, with or without persistent bleeding [2]

AIM

To investigate clinical, health-related quality of life, and psychosocial outcomes in PwHA recruited as part of the CHESS III study, based on the agreement between PwHA and HCPs in reports of joint damage.

RESULTS

- The analysis was conducted for a sample of **133 PwHA** from the CHESS III dataset; out of which, **35/133 (26.3%)** were in the **discordance group** and **98/133 (73.7%)** in the **concordance group** (see **Table 1**). Age was similar across groups (averaging approximately 41.8 (16.4) years in the concordance group and 43.2 (14.8) in the discordance group), and **more PwHA in the discordance group had severe haemophilia A** (27/35, 77.1% vs. 57/98, 58.2%).
- **Mean annual bleed rate (ABR)** was **4.9 (6.7)** in the **discordance group**; this was **significantly higher than** what was reported in the **concordance group**: **1.9 (4.1)** (p-value of p<0.01).
- Within the sample with PwHA-reported problem joints, **chronic pain in at least one joint** was reported in **85.7% (30/35) individuals** in the **discordance group**, and in **75.6% (31/41)** of those in the **concordance group** (p-value of 0.27).

Table 1. Clinical characteristics, by PJ alignment

Variables	PJ alignment groups		P-value
	Concordance group N=98	Discordance group N=35	
Age (years), mean (SD)	41.84 (16.45)	43.20 (14.82)	0.67
Severity, n (%)			0.14
Mild	15 (15.3%)	3 (8.6%)	
Moderate	26 (26.5%)	5 (14.3%)	
Severe	57 (58.2%)	27 (77.1%)	
Problem joints (HCP-reported), mean (SD)	1.00 (1.07)	0.57 (0.92)	0.04*
Problem joints (Patient-reported), mean (SD)	0.71 (1.06)	2.37 (1.68)	<0.01**
Chronic joint pain[a], n (%)	31 (75.6%)	30 (85.7%)	0.27

[a]Patient-reported chronic joint pain, as a symptom of PJ (only available in PwH with ≥1 patient reported PJs, n=41)
** p-value <0.05; ** p-value <0.01*

- Overall HRQoL was **significantly lower in the discordance group**, as measured by the **EQ-5D** tool (EQ-5D-5L mean [SD]: 0.67 [0.22] compared to 0.80 [0.21], p-value <0.01) – see **Figure 1**.
- Anxiety (as measured via the **GAD-7** tool) and depression (as measured via the **PHQ-8** tool) scores **were significantly higher in the discordance group**, with mean (SD) GAD-7 score of 6.9 (5.7) compared to 4.6 (4.0), and mean (SD) PHQ-8 score of 8.5 (6.1) compared to 5.0 (4.6), respectively (p<0.01, for both tool scores) – see **Figure 2**.
- When comparing impact on daily life and compromise associated with haemophilia, a noticeably **higher proportion of the discordance group** reported they had to reduce or stop physical activities, when compared to the concordance group (21/35, 60% vs. 46/98, 47%), with similar proportions of PwHA reporting missed opportunities due to Haemophilia (see **Figure 3**).
- In addition, 51% (18/35) of the discordance group shared having **frustrations about the impact of the disease**, compared to 37% (36/98) in the concordance group reporting this.

Figure 1. EQ-5D-5L index score, by PJ alignment

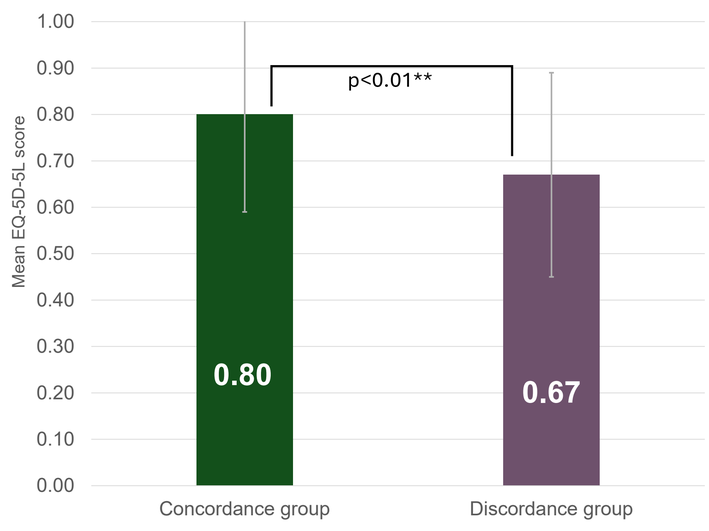


Figure 2. PHQ-8 and GAD-7 score, by PJ alignment

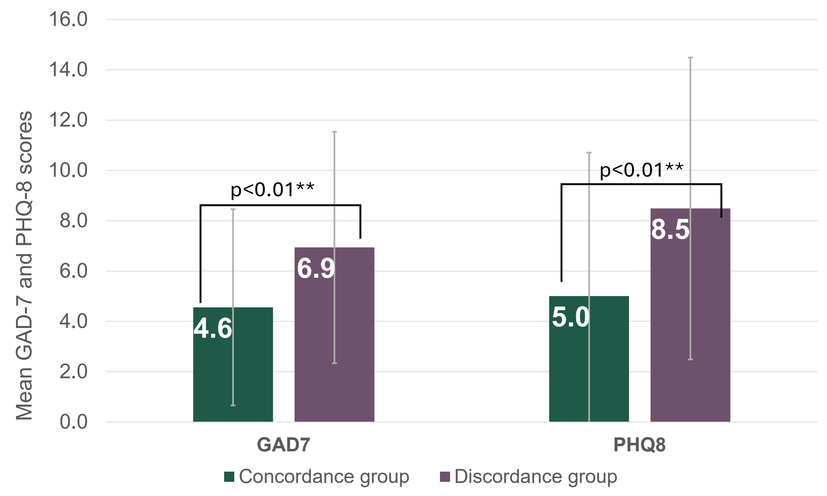
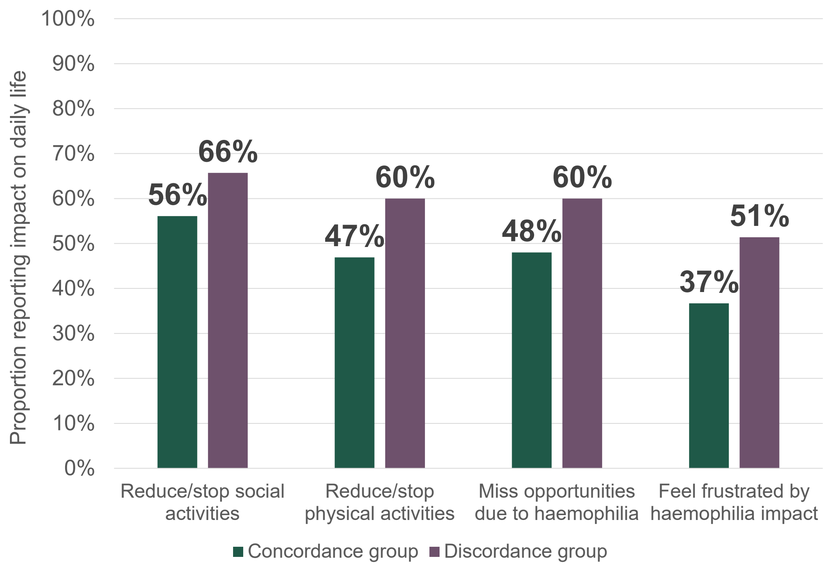


Figure 3. Impact on daily life/compromise, by PJ alignment



CONCLUSIONS

- **PwHA who reported more joint problems** than their healthcare providers consistently showed **worse clinical outcomes**, **poorer HRQoL**, and **greater psychosocial burden**.
- These findings highlight a critical gap in the recognition of joint deterioration and its consequences, suggesting that **under-detection** may allow **disease burden to increase over time.**
- **Strengthening routine assessment and management**, as well as ensuring that patient-reported joint symptoms are fully acknowledged in clinical practice could play a key role in **improving overall outcomes** for people living with haemophilia.

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