

An Insight into the Impact of Hemophilia A on Daily Life According to Disease Severity: A Preliminary Analysis of the CHES II Study

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Key takeaways

- There is a paucity of data on the impact of hemophilia A (HA) on daily life
- This analysis demonstrates that although the impact of HA on daily life is most pronounced in individuals with severe disease, it is also apparent in mild and moderate HA

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Disclosures

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The CHES II study collects real-world data on the burden-of-illness in adult PwHA

Background

- There are currently **limited data on the impact of HA** on daily life, particularly according to disease severity
- The Cost of Haemophilia in Europe: a Socioeconomic Survey II (CHES II) is a **retrospective, cross-sectional, burden-of-illness** study in adults with **mild, moderate, and severe HA**
- In this preliminary analysis of the CHES II study, we examine **the impact of HA on the daily life of adult PwHA without current FVIII inhibitors** according to disease severity

Methods

Male participants aged ≥ 18 years diagnosed with HA (without FVIII inhibitors) ≥ 12 months prior to clinical consultation were enrolled from 8 European countries



- Data on clinical outcomes and healthcare resource utilization were captured via electronic case report forms disseminated to hemophilia specialists
- PwHA completed a paper-based questionnaire utilizing 5-point Likert scales (I strongly disagree–I strongly agree) to assess the disease burden on their daily life
- Overall, 12 months' retrospective data were examined

A higher proportion of persons with moderate and severe HA had problem joints

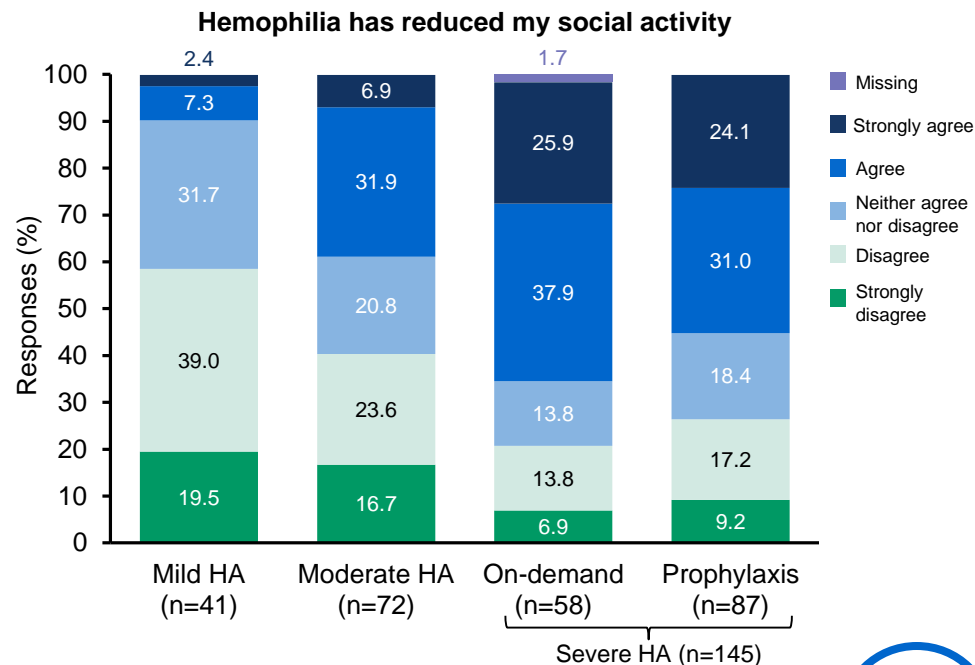
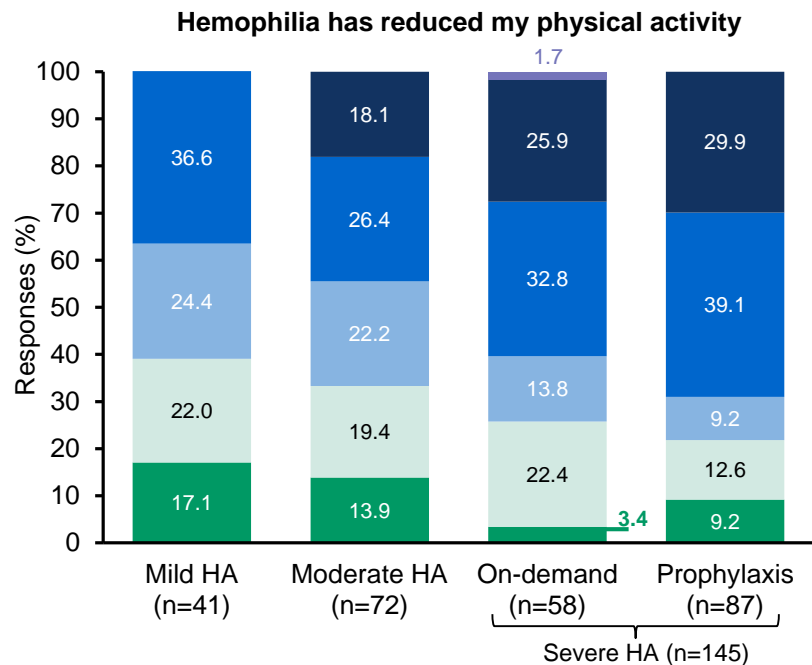
	Mild HA (n=41)	Moderate HA (n=72)	Severe HA (n=145)		All participants (N=258)
			On-demand (n=58)	Prophylaxis (n=87)	
Mean age, years (SD)	38.66 (14.86)	39.69 (15.66)	34.40 (12.56)	39.44 (15.51)	38.25 (14.90)
Median BMI (IQR)	24.7 (23.0–26.1)	24.6 (22.9–27.0)	24.5 (23.0–25.6)	24.8 (22.5–26.7)	24.7 (22.9–26.2)
Current treatment strategy, n (%)					
On-demand	14 (34.1)	22 (30.6)	58 (100)	0 (0)	94 (36.4)
Prophylaxis	2 (4.9)	5 (6.9)	0 (0)	87 (100)	94 (36.4)
No treatment	25 (61.0)	45 (62.5)	0 (0)	0 (0)	70 (27.1)
Has adapted hemophilia treatment, n (%)					
No	27 (65.9)	46 (63.9)	32 (55.2)	36 (41.4)	141 (54.7)
Yes	8 (19.5)	17 (23.6)	20 (34.5)	36 (41.4)	81 (31.4)
Don't know	5 (12.2)	8 (11.1)	3 (5.2)	12 (13.8)	28 (10.9)
Missing/incomplete*	1 (2.4)	1 (1.4)	3 (5.2)	3 (3.4)	8 (3.1)
Problem joints present, n (%)	8 (19.5)	28 (38.9)	26 (44.8)	42 (48.3)	104 (40.3)
Pain reported by physician, n (%)					
No pain	26 (63.4)	24 (33.3)	14 (24.1)	19 (21.8)	83 (32.2)
Mild pain	15 (36.6)	32 (44.4)	23 (39.7)	33 (37.9)	103 (39.9)
Moderate pain	0 (0)	15 (20.8)	16 (27.6)	28 (32.2)	59 (22.9)
Severe pain	0 (0)	1 (1.4)	5 (8.6)	7 (8.0)	13 (5.0)

- A problem joint is any joint that has been permanently damaged as a result of a bleeding disorder, with or without persistent bleeding; a problem joint can be defined as having chronic joint pain and/or limited range of movement due to compromised joint integrity (i.e. chronic synovitis and/or hemophilic arthropathy)¹



Percentages may not add up to exactly 100% due to rounding. **Missing/incomplete** includes those questions with no answers provided and those with answers that are illegible. BMI, body mass index; HA, hemophilia A; IQR, interquartile range; PwHA, persons with hemophilia A; SD, standard deviation.

A proportion of PwHA have reduced physical and social activity as a result of their disease



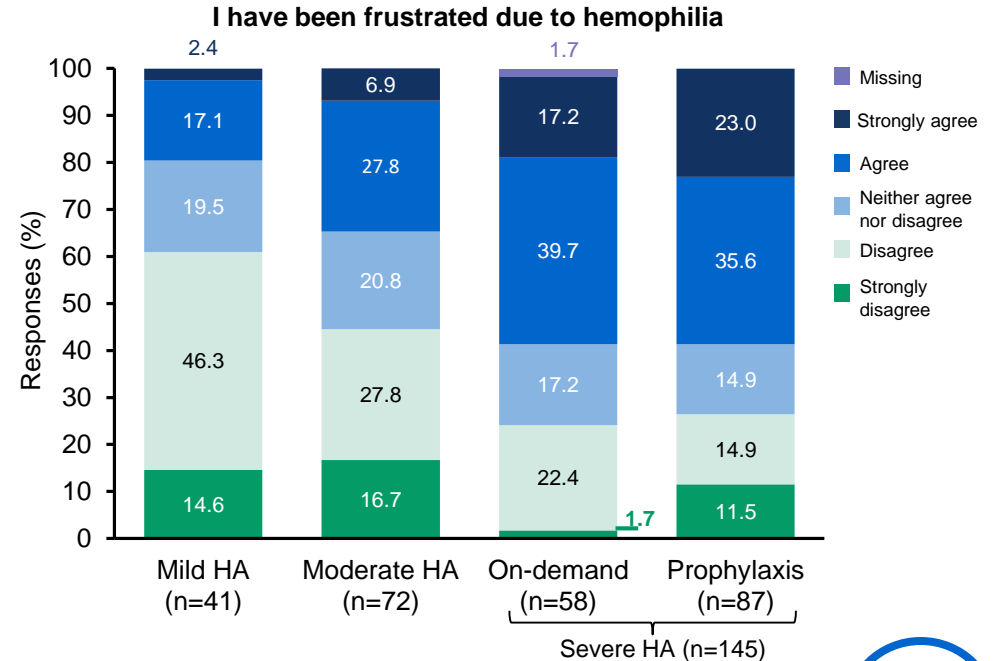
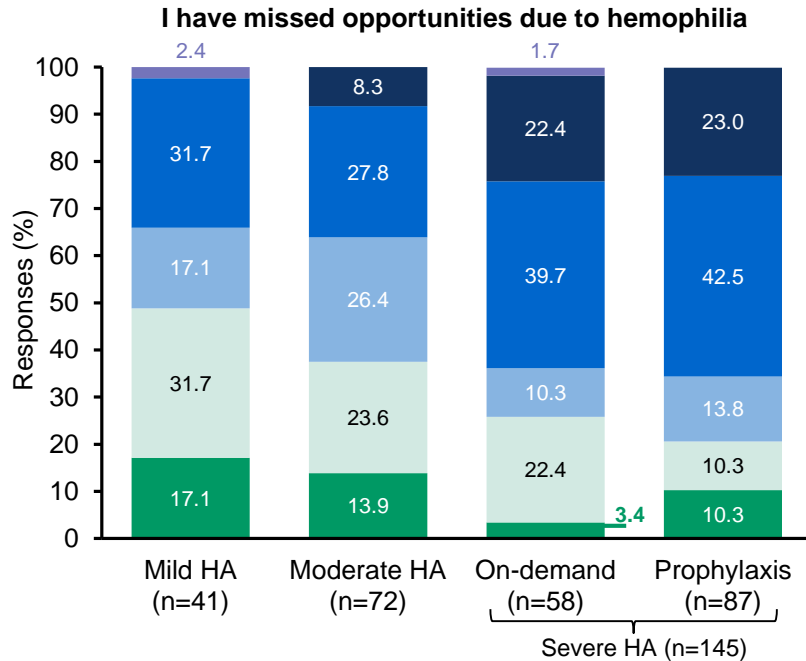
- Due to their HA, **36.6%** of persons with **mild**, **44.4%** with **moderate**, and **64.8%** with **severe HA**, stated that they had **reduced physical activity**, and **9.8%** with **mild**, **38.9%** with **moderate** and **58.6%** with **severe HA**, stated that they had **reduced social activity**

Percentages may not add up to exactly 100% due to rounding.

For persons with severe HA receiving on-demand treatment, the missing 1.7% corresponds to n=1. HA, hemophilia A; PwHA, persons with hemophilia A.



A proportion of PwHA felt that HA had caused them to miss opportunities, and felt frustration due to their HA



- Due to their HA, **31.7%** of persons with **mild**, **36.1%** with **moderate**, and **64.1%** with **severe HA**, stated that they had **missed opportunities**, and **19.5%** with **mild**, **34.7%** with **moderate** and **57.9%** with **severe HA**, stated that they had been **frustrated**

Percentages may not add up to exactly 100% due to rounding.

For persons with mild HA and persons with severe HA receiving on-demand treatment, the missing 2.4% and 1.7% each corresponds to n=1.

HA, hemophilia A; PwHA, persons with hemophilia A.



Conclusions



In all disease severity groups, there was a notable proportion of PwHA that had **problem joints**, felt **pain**, had **reduced their physical and social activity**, felt that they had **fewer opportunities** and felt **frustrated** due to their disease



The impact of HA on daily life was **most pronounced in persons with severe HA**; this included both those receiving on-demand treatment and those receiving prophylaxis



The impact on daily life **was also apparent in mild and moderate HA**, indicating that there may be an **unmet medical need** in these groups



Varying degrees of historic prophylactic access in the contributing countries are a limitation of this study; in addition, **restricted question wording** did not capture the causes of frustration and specifics of missed opportunities were not detailed

