Impact of Haemophilia Severity on Healthcare Resource Usage: Results from a Multinational Real-World Survey

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Introduction

- Haemophilia is an X-linked disorder associated with recurrent bleeding episodes due to dysfunctional clotting factor VIII (haemophilia A, HA) and IX (haemophilia B, HB).
- Severity is determined by the level of clotting factor (CF), with severity associated with spontaneous bleeds and life-threatening uncontrolled bleeds.
- Bleeds in the joints cause damage, which can impair mobility and may ultimately require surgery.
- Management of haemophilia requires extensive healthcare resource utilization (HCRU), involving a multi-disciplinary healthcare team.
- Real-world evidence is currently lacking to describe HCRU across disease severity.

Methods

- The Adelphi Haemophilia Disease Specific Programme (DSP)™ was a cross-sectional survey of male patients with haemophilia A and B, conducted in France, Germany, Italy, Spain, United Kingdom, and the United States between February 2020 – May 2021.
- The survey methodology has been published and validated [1-3].
- Physicians completed patient record forms for their next consecutively consulting patients with HA and HB, collecting demographic, clinical and treatment data and hospitalisations.
- Patients were grouped as mild (>5% of normal CF levels), moderate (1-5%), and severe (<1%) disease activity using baseline CF at the time of data collection.

Limitations

- While minimal inclusion criteria governed physician selection, participation was influenced by willingness to complete the survey.
- Physicians recruited consecutive patients, with data collected at the time of consultation, to mitigate against selection bias and recall bias. However, more frequently consulting patients and those with more severe disease activity were more likely to be captured.
- The survey was conducted during the COVID pandemic, which may have influenced recruitment.
- Despite this, the patient sample is likely to be representative of those presenting in a real-world setting.

Objective

- This study describes hospitalisations, surgeries, and consultations for patients with haemophilia A (HA) and B (HB) to better understand healthcare resource utilisation (HCRU) based on disease severity.

Conclusions

- Despite receiving treatment, patients with severe HA and HB had high hospitalisation and consultation rates, highlighting increased HCRU and potentially greater economic burden.
- There is a need for future therapies to better manage factor levels and reduce the negative clinical outcomes of haemophilia to lower HCRU and economic burden.

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• Pfizer Inc. subscribed to this survey and did not influence the original survey through either contribution to the design of questionnaires or data collection.
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Disclosures:
• ST, LW, VM, AK, JA and SP are employees of Pfizer Inc., New York, United States of America.
• EM, JGW, NB, SO and KG are employees of Adelphi Real World, Bollington, United Kingdom.

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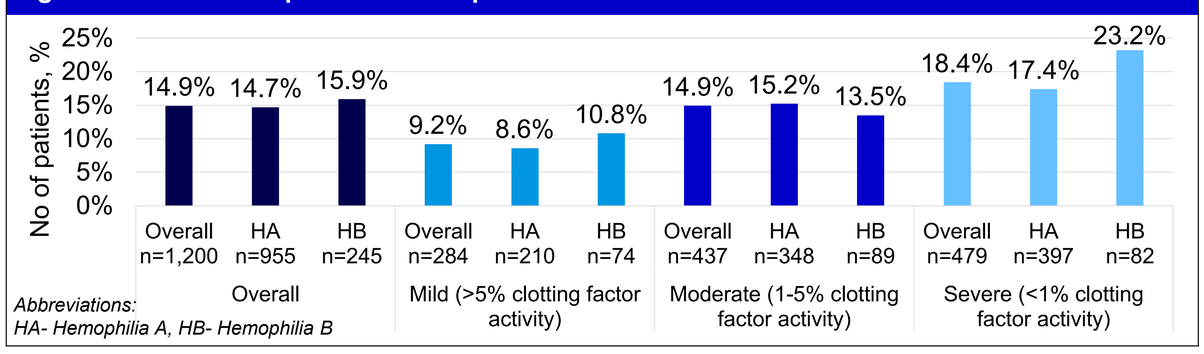
Results

Table 1: Patient demographics and clinical characteristics

	Overall			Mild (>5% clotting factor activity)			Moderate (1-5% clotting factor activity)			Severe (<1% clotting factor activity)		
	Overall n=1,392	HA n=1,116	HB n=276	Overall n=320	HA n=236	HB n=84	Overall n=528	HA n=426	HB n=102	Overall n=544	HA n=454	HB n=90
Patient age												
Mean (SD)	29.2 (16.0)	28.9 (15.7)	30.1 (17.0)	32.6 (15.7)	32.5 (15.7)	32.8 (15.9)	31.4 (15.9)	31.5 (15.8)	31.0 (16.2)	25.0 (15.3)	24.7 (14.6)	26.6 (18.4)
Ethnic Origin, n (%)												
White	1193 (86%)	966 (87%)	227 (82%)	273 (85%)	205 (87%)	68 (81%)	455 (86%)	367 (86%)	88 (86%)	465 (85%)	394 (87%)	71 (79%)
Patient BMI												
Mean (SD)	23.9 (5.0)	24.0 (5.1)	23.7 (4.2)	24.0 (3.3)	23.9 (3.2)	24.2 (3.5)	24.3 (5.9)	24.4 (6.4)	23.9 (3.6)	23.5 (4.7)	23.6 (4.6)	23.0 (5.3)
Time since hemophilia diagnosis (years)												
Mean (SD)	19.7 (14.6)	19.7 (14.5)	19.5 (14.9)	20.0 (15.6)	20.1 (15.3)	19.6 (16.6)	19.0 (14.1)	18.6 (14.1)	20.4 (13.9)	20.1 (14.4)	20.4 (14.4)	18.3 (14.5)
Inhibitor Status, n (%)												
Never had inhibitors	1035 (74%)	805 (72%)	230 (83%)	266 (83%)	192 (81%)	74 (88%)	379 (72%)	305 (72%)	74 (73%)	390 (72%)	308 (68%)	82 (91%)
Previous inhibitors	183 (13%)	152 (14%)	31 (11%)	34 (11%)	27 (11%)	7 (8%)	84 (16%)	63 (15%)	21 (21%)	65 (12%)	62 (14%)	3 (3%)
Current inhibitors	174 (12%)	159 (14%)	15 (5%)	20 (6%)	17 (7%)	3 (4%)	65 (12%)	58 (14%)	7 (7%)	89 (16%)	84 (19%)	5 (6%)
Is the patient currently receiving prescribed treatment for their haemophilia? n (%)												
Yes	885 (86%)	696 (86%)	189 (82%)	181 (68%)	130 (68%)	51 (69%)	324 (85%)	266 (87%)	58 (78%)	380 (97%)	300 (97%)	80 (98%)
No	150 (14%)	109 (14%)	41 (18%)	85 (32%)	62 (32%)	23 (31%)	55 (15%)	39 (13%)	16 (22%)	10 (3%)	8 (3%)	2 (2%)
Among those currently receiving treatment: How does the patient receive the current treatment? n (%)												
On-demand	240 (27%)	172 (25%)	68 (36%)	130 (72%)	90 (69%)	40 (78%)	88 (27%)	67 (25%)	21 (36%)	22 (6%)	15 (5%)	7 (9%)
Prophylactically	645 (73%)	524 (75%)	121 (64%)	51 (28%)	40 (31%)	11 (22%)	236 (73%)	199 (75%)	37 (64%)	358 (94%)	285 (95%)	73 (91%)

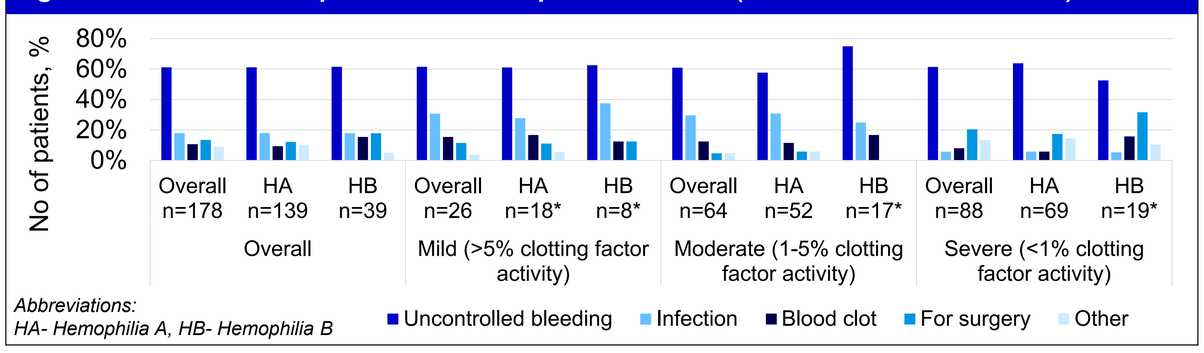
- Haematologists provided data for 1,116 patients with HA (mild: n= 236, moderate: n= 426, severe: n= 454) and 276 patients with HB (mild: n= 84, moderate: n= 102, severe: n= 90) (Table 1).
- Overall, 74% of patients had never had inhibitors and 86% were currently receiving treatment. 28% of mild, 73% of moderate and 94% of patients with severe activity were receiving treatment prophylactically.
- In the past 12 months, 17.4% of severe HA and 23.2% of severe HB patients had at least one hospitalization (mild HA: 8.6%, mild HB: 10.8%, moderate HA: 15.2%, moderate HB: 13.5%) (Figure 1).
- Overall, the most frequent reasons for hospitalization included uncontrolled bleeding (HA: 61.2%, HB: 61.5%), infection (HA: 18.0%, HB: 17.9%), surgery (HA: 12.2%, HB: 17.9%) or blood clot (HA: 9.4%, HB: 15.4%) (Figure 2).
- Overall mean consultations per HA patient with healthcare professionals were; mild HA: 6.9 (7.7), moderate HA: 9.7 (12.1), and severe HA: 15.2 (25.9) (Figure 3).
- For HB patients, mean consultations were; mild HB: 9.2 (7.8), moderate HB: 10.2 (9.6), and severe HB: 9.7 (18.1).
- A high mean number of consultations for severe patients were with haemophilia nurses (severe HA: 13.0, severe HB: 6.5) and physiotherapists (severe HA: 11.0, severe HB: 9.5)
- The proportion of patients who had ever undergone joint surgery differed with severity (mild HA: 8.1%, mild HB: 1.2%, moderate HA: 10.8%, moderate HB: 10.8%, severe HA: 18.5%, severe HB: 18.9%) (Figure 4).

Figure 1: Patients hospitalized in the past 12 months



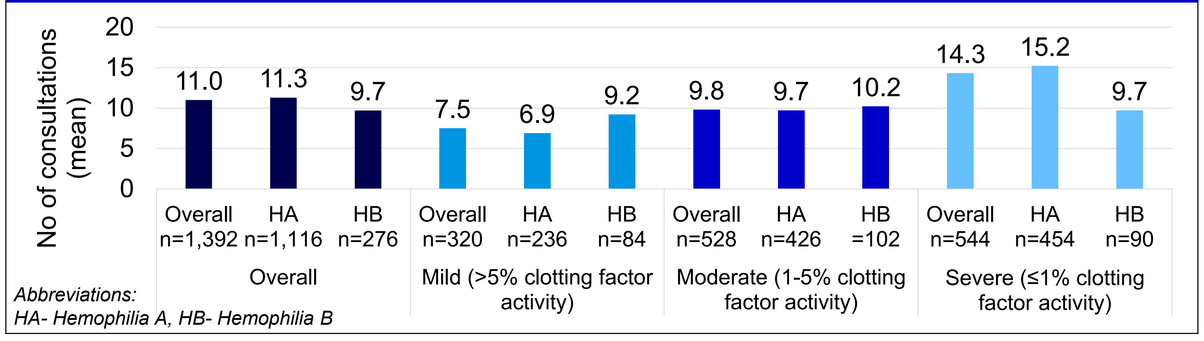
Abbreviations:
HA- Hemophilia A, HB- Hemophilia B

Figure 2: Reason for hospitalization in the past 12 months (*=low base of less than 20)



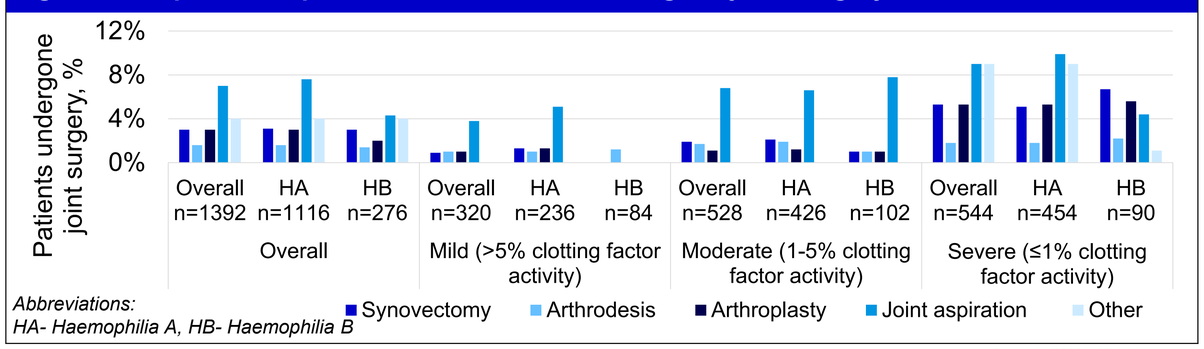
Abbreviations:
HA- Hemophilia A, HB- Hemophilia B

Figure 3: Total number of consultations in the past 12 months



Abbreviations:
HA- Hemophilia A, HB- Hemophilia B

Figure 4: Proportion of patients that have ever undergone joint surgery



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