A Real-World Survey of Health Inequalities as Drivers for Burden on Male Hemophilia Patients in the US, EU5 and Asia

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Introduction

Hemophilia is characterized by bleeds and joint problems due to dysfunctional clotting factor VIII and IX in Hemophilia A and Hemophilia B, respectively. Therefore, there is an increased risk of spontaneous bleeds occurring in joints and muscles[1].

People with hemophilia (PwH) experience substantial burden as a result of these disease outcomes. Factors such as distance to a hemophilia treatment center (HTC), disease associated costs and caregiver inconvenience can increase the likelihood of negative outcomes occurring, in turn increasing the burden of the disease[2] .

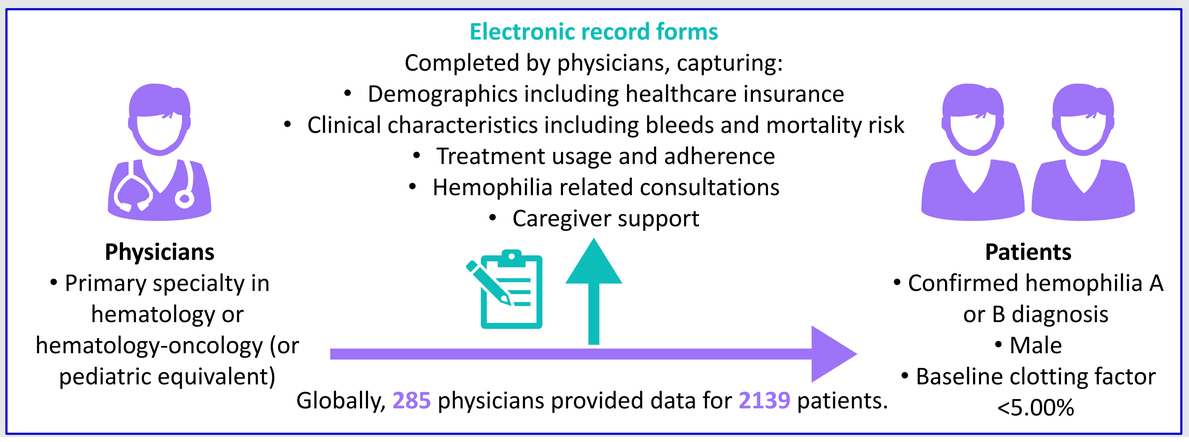
The impact of hemophilia on a patient's physical function can increase difficulty to maintain employment, further increasing the financial burden[3]. Physical limitations emphasize the need for caregiver support when managing daily activities[3].

Methods

Data were drawn from the Adelphi Real World Disease Specific Programme™, a cross-sectional survey with retrospective data collection of physicians and male PwH A and B in France, Germany, Italy, Spain, the United Kingdom (EU5), the United States (US) and Japan, Saudi Arabia and India (Asia) (July 2023-October 2024).

Physicians reported data on insurance, consultations, caregiver support, and mortality risks. Analyses were descriptive.

DSP™ are cross-sectional surveys with retrospective data collection of a geographically representative sample of physicians. The DSP methodology has been previously described, validated, and the results proven to be consistent over time [4,5,6,7].



Objective

To investigate health care resource utilization, caregiver utilization, and risk of mortality among male hemophilia patients.

Conclusions

Physicians reported approximately half of PwH in Asia had no insurance and were never seen in an HTC.

PwH in Asia experienced a high perceived risk of mortality.

Less adult PwH in the US/EU5 required a caregiver than in Asia.

This indicates that decreased insurance and HTC usage are potential drivers of burden in hemophilia, highlighting the need to address health inequalities.

Limitations

- Physicians completed surveys for their next consecutively consulting patients, meaning more frequently consulting patients and those with a more severe disease state are more likely to be captured within the DSP™.
- As data was reported retrospectively, there is a possibility of recall bias; this was reduced by collecting data at earliest time point following patient consultation.
- Without collecting all relevant data points that can impact health inequalities, i.e access limitations, the potential for bias is introduced, undermining validity and replicability.

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- Data was collected by Adelphi Real World via the Hemophilia III Disease Specific Program ™, an independent survey whereby all data are the intellectual property of Adelphi Real World. Pfizer Inc. subscribed to access this data source.
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Disclosures:

- ST is an employee of Pfizer Inc., Cambridge, United States of America.
- ST, NP is an employee of Pfizer Inc., New York, United States of America.
- CK is an employee of Pfizer Inc., Canada.
- NB, EM, SL ,CB and RS are employees of Adelphi Real World, Bollington, United Kingdom.

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Results

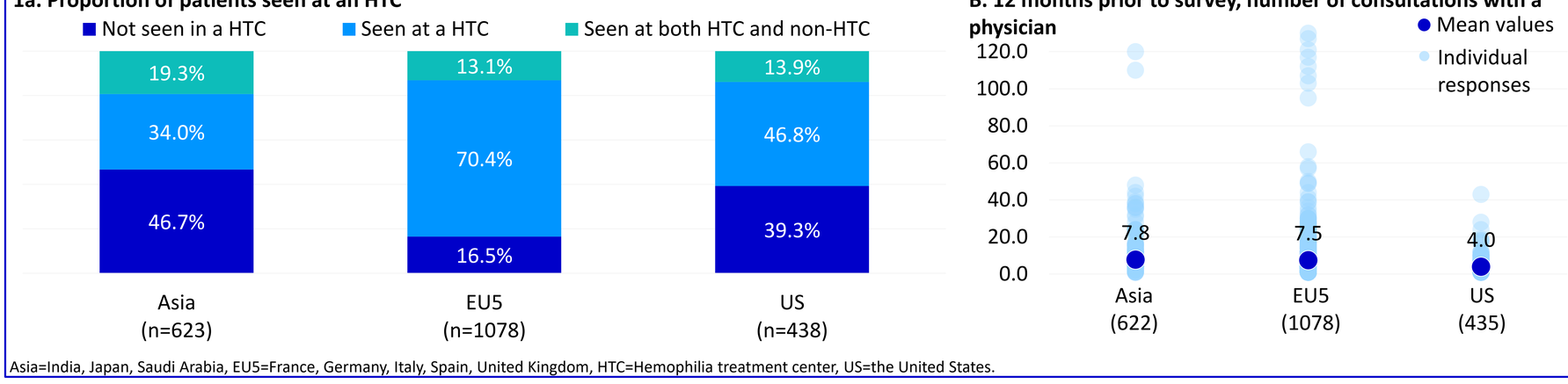
Table 1. Patient characteristics

	Overall *(n=2139)*	Asia *(n=623)*	EU5 *(n=1078)*	US *(n=438)*
Age	*n=2139*	*n=623*	*n=1078*	*n=438*
Years, mean (SD)	26.9 (15.3)	24.7 (14.9)	27.9 (15.5)	27.7 (14.9)
≥18 years old, n (%)	1633 (76.3)	406 (65.2)	877 (81.4)	350 (79.9)
Employment, n (%)	*n=1641*	*n=418*	*n=886*	*n=337*
Working full time	746 (45.5)	165 (39.5)	395 (44.6)	186 (55.2)
Not working full time	895 (54.5)	253 (60.5)	491 (55.4)	151 (44.8)
Impact of hemophilia on employment, n (%)*	*n=372*	*n=151*	*n=164*	*n=57*
Not working full time due to hemophilia	184 (49.5)	106 (70.2)	61 (37.2)	17 (29.8)
Baseline clotting factor at time of survey, n (%)	*n=2077*	*n=603*	*n=1060*	*n=414*
1.00% - 5.00% of the normal activity of clotting factor	1304 (62.8)	425 (70.5)	581 (54.8)	298 (72.0)
<1.00% of the normal activity of clotting factor	773 (37.2)	178 (29.5)	479 (45.2)	116 (28.0)
Treatment at time of survey, n (%)	*n=2126*	*n=613*	*n=1078*	*n=435*
Solely receiving on-demand	150 (7.1)	61 (10.0)	67 (6.2)	22 (5.1)
Solely receiving prophylaxis	558 (26.2)	250 (40.8)	220 (20.4)	88 (20.2)
Receiving on-demand alongside prophylaxis	1405 (66.1)	300 (48.9)	786 (72.9)	319 (73.3)
No treatment	13 (0.6)	2 (0.3)	5 (0.5)	6 (1.4)
Health insurance, n (%)	*n=2084*	*n=603*	*n=1071*	*n=410*
Has health insurance cover for hemophilia treatment	1803 (86.5)	324 (53.7)	1071 (100.0)	408 (99.5)

Asia=India, Japan, Saudi Arabia, EHL=Extended half life, EU5=France, Germany, Italy, Spain, United Kingdom, SD=Standard deviation, SHL=Standard half life, US=the United States,
*Base drop because physicians were only shown question if patient was working part-time, not working due to retirement, unemployed, or on long term sick leave.

- Physician-reported patient healthcare usage is reported in **Figure 1a and 1b.**
- Physicians reported 60.7% of US PwH, 83.5% EU5 and 53.3% Asia had been seen at least once in an HTC.

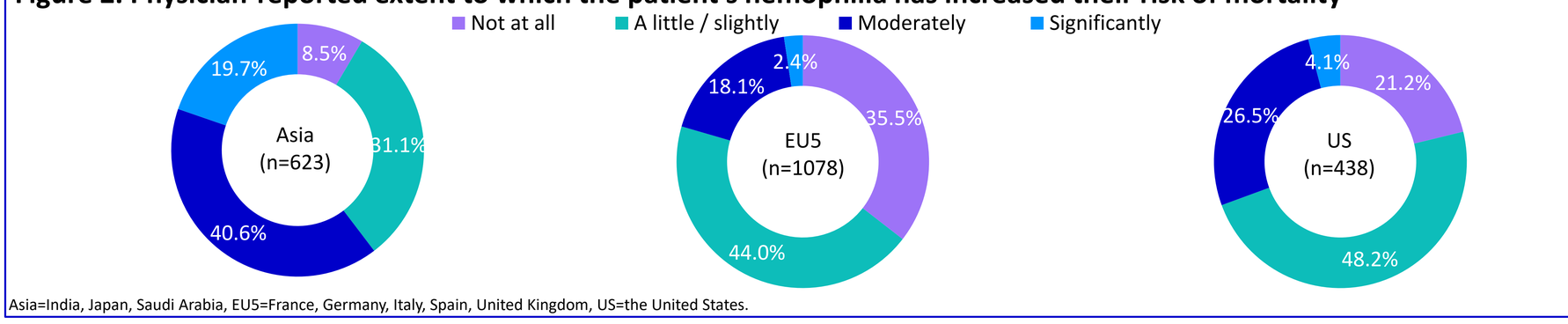
Figure 1. Physician-reported patient healthcare usage



Asia=India, Japan, Saudi Arabia, EU5=France, Germany, Italy, Spain, United Kingdom, HTC=Hemophilia treatment center, US=the United States.

- The extent to which physicians reported that hemophilia has increased the patient's risk of mortality is summarised in **Figure 2.**
- Physicians perceived the risk of mortality to "moderately" or "significantly" increase due to the patient's hemophilia for 30.6% of PwH in US, 20.5% in the EU5 and 60.4% in Asia
- Additionally, patients in Asia had a median (IQR) 2.0 (1.0-2.0) bleeds in the 12-months prior to survey, in the US and EU5 PwH had 0.0 (0.0-0.0) bleeds.

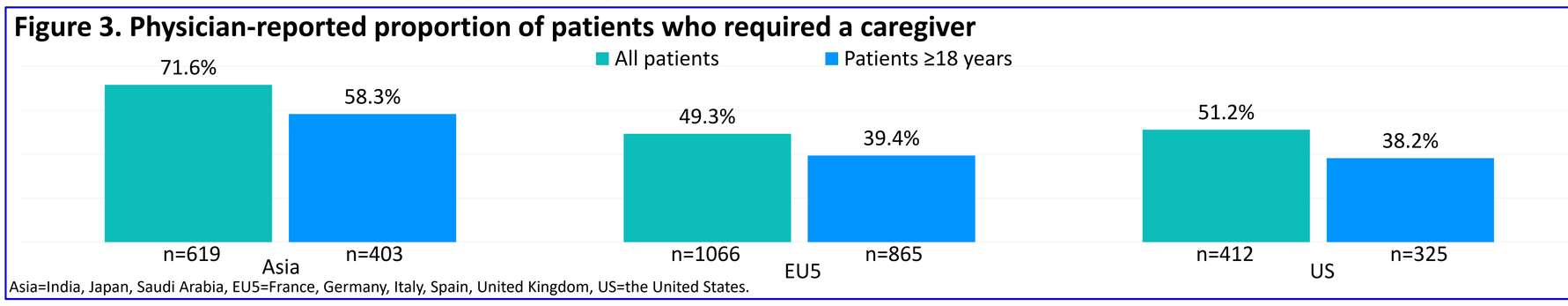
Figure 2. Physician-reported extent to which the patient's hemophilia has increased their risk of mortality



Asia=India, Japan, Saudi Arabia, EU5=France, Germany, Italy, Spain, United Kingdom, US=the United States.

- Physician-reported caregiver usage for PwH is summarized in **Figure 3.**
- Of the patients ≥18 years who required a caregiver (Asia; n=235, EU5; n=341, US; n=124), care was predominantly provided by the patients' parent or guardian (Asia; 52.3%, EU5; 51.3%, US; 37.1%) or their partner or spouse (Asia; 46.4%, EU5; 41.6%, US; 58.1%).
- Physicians reported of the 406 PwH ≥18 years in Asia, 75.4% had difficulty with physical activities, whilst PwH in EU5 (n=877) and the US (n=350), 38.9% and 36.6% had difficulty, respectively.

Figure 3. Physician-reported proportion of patients who required a caregiver



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