Identifying Evidence Gaps for Hemophilia B by Developing a Factsheet Using Targeted Review of Literature: Shaping the Future Real-World Evidence Studies

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Background & Objective

- Owing to the shrinking budget due to the global economic downturn, evidence from sources other than randomized controlled trials is being accepted, especially in the case of rare diseases
- Factsheet is one such compilation of the available literature for a disease, based on targeted literature review inclusive of grey literature
- Hemophilia is a general term for a group of rare bleeding disorders caused by congenital deficiency of certain clotting factors[1]
- Hemophilia B is a rare genetic bleeding disorder in which affected individuals have insufficient levels of clotting factor IX[1]
- Hemophilia B is less prevalent than hemophilia A which is the most common form of hemophilia[1]
- Objective: To develop a factsheet for Hemophilia B in the US population to identify evidence gaps that would help in shaping the future RWE studies

Methodology

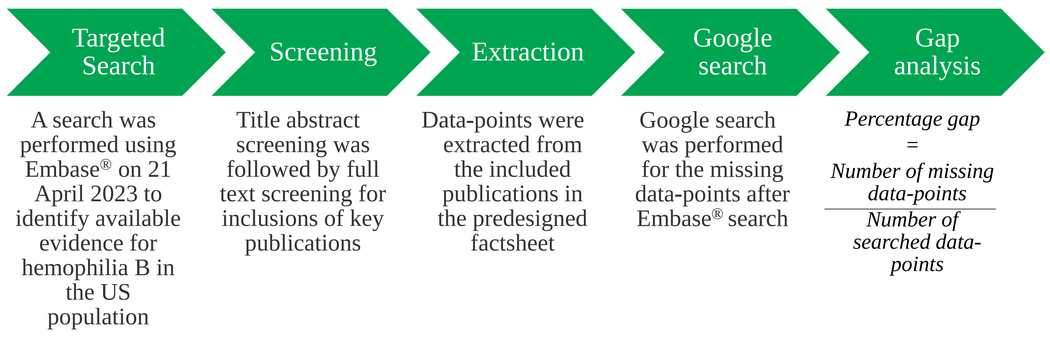


Figure 1a. Step-wise approach for the compilation of factsheet

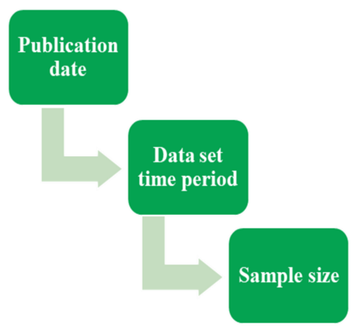


Figure 1b. Priority order for study selection

Data points extracted

- Epidemiology
- Demographics
- Disease-specific characteristics (diagnostics, severity, bleeding site, and inhibitor status)
- Comorbidities
- Mortality
- Treatment patterns
- HCRU and costs
- QoL
- Disease management (HTCs and self-care)

Results

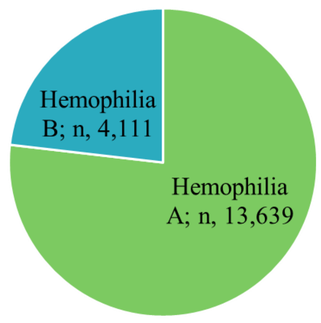


Figure 2a. Population distribution[2]

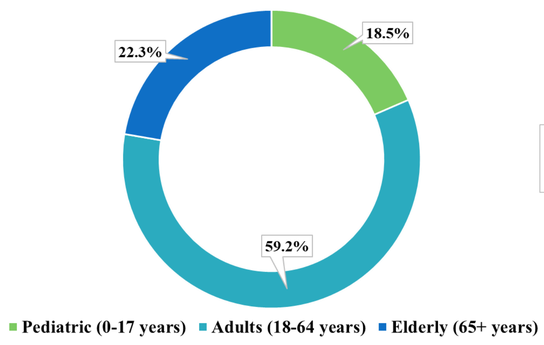


Figure 2b. Age group, % admitted with hemophilia B[3]

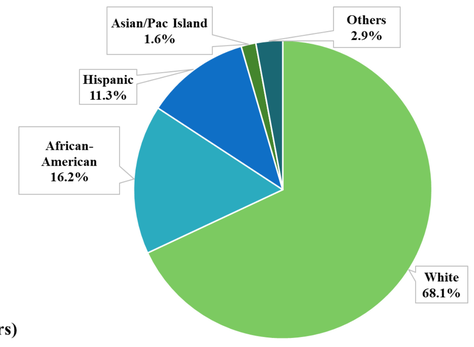
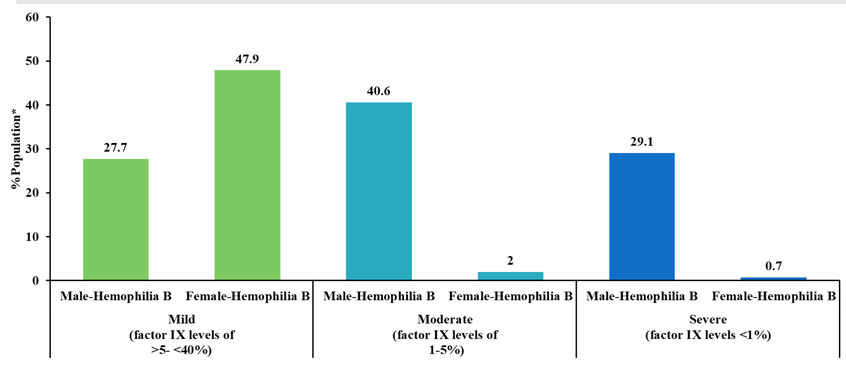


Figure 2c. Distribution by race[3]

- 151 records were retrieved from Embase®
- After title/abstract and full text screening, 32 publications were included
- Data was extracted from 12 publications/reports (Embase®/Google: 9/3) based on priority order (publication date > dataset time-period > sample size) of study selection **(Figure 1b)**
- Data-points related to epidemiology, demographics, disease severity are presented in **Figure 2 (a-e)**
- The reported incidence was 1 birth per 19,283 male births in US[5]
- The reported prevalence was 3.7 per 100,000 US male population[5]
- The reported mortality rate for hospitalized patients was 1.2%[3]
- Mean Charlson comorbidity index score was 0.9 (SD:1.7)[3]
- Recombinant factor IX was the most common treatment reported (63%)[6]
- Prophylaxis treatment was more commonly reported than episodic treatment[6,7]



*Factor IX >40%: 1.4% (Males) and 44.8% (Females)
Unknown: 1.2% (Males) and 4.6% (Females)

Figure 2d. Severity at diagnosis[4]

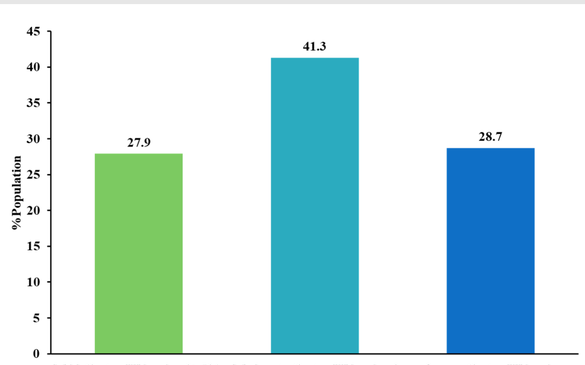


Figure 2e. Distribution by severity[5]

Cost Data Mean (SD)	Direct	Total cost[8]	$201,635 ($411 530)
		Pharmacy Cost[8]	$146,291 ($360,532)
		Inpatient cost[8]	$18,747 ($99,752)
		Outpatient cost[8]	$28,60, ($149,083)
		Hospitalization cost[3]	$209,660 ($529,885)
	Indirect	ED Visits[8]	$1,333 ($5,928)
		Absenteesim[10]	$760 ($2,286)
	Total Cost[9]		$140,240 ($170,392)
Resource Use Mean (SD)	No. of emergency department visits[8]		0.6 (1.2)
	Number of inpatient admissions[8]		0.3 (0.6)
	Number of outpatients visits[8]		17.7 (22.9)
	Hospitalization[8]		1.2 (3.7)
	Productivity loss (missed days of work due to hemophilia B)[9]		3.6 (6)

Table 1. Healthcare cost and resource use[3,8,9,10]

- **Table 1** represents all the extracted data-points related to HCRU and cost
- Mean EQ-5D score was 0.7 (SD:0.2)[3]
- All data-points related to the categories of demographics, comorbidities, and mortality were identified

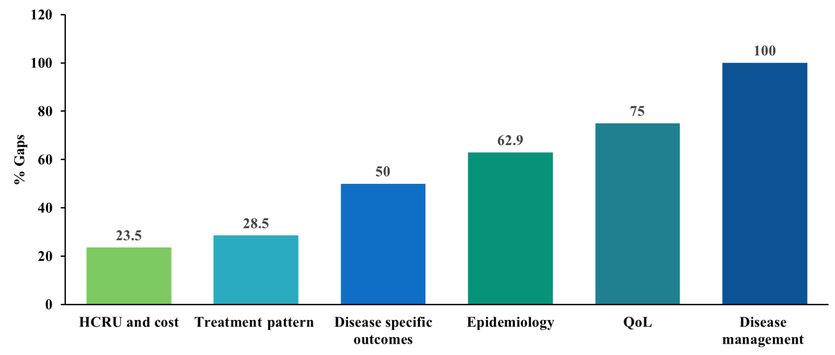


Figure 3. Gaps (%) identified for data-points

- Gaps existed in data-points (**Figure 3**) related to disease management (HTCs and self-care, 100%), QoL (75%), epidemiology (62.9%), disease-specific characteristics (50%), treatment patterns (28.5%), and HCRU and costs (23.5%)

Conclusions

- The identified evidence indicated huge gaps (≥50%) in various data points including disease-specific characteristics, disease management, QoL, and epidemiology for Hemophilia B in the US
- These gaps can help in shaping and planning future RWE studies related to Hemophilia B

Abbreviations: EQ-5D: EuroQoL five dimension questionnaire, HCRU: Health care resource utilization, HTC: Hemophilia treatment centers, QoL: Quality of life, RWE: Real-world evidence, SD: Standard deviation, TI/AB: Title/abstract, US: United States

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