

Economic Burden of Hemophilia B Patients in the United States: A Systematic Literature Review

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Introduction

Hemophilia B (HB) is a rare X-linked congenital bleeding disorder characterized by a deficiency of coagulation factor IX (FIX).

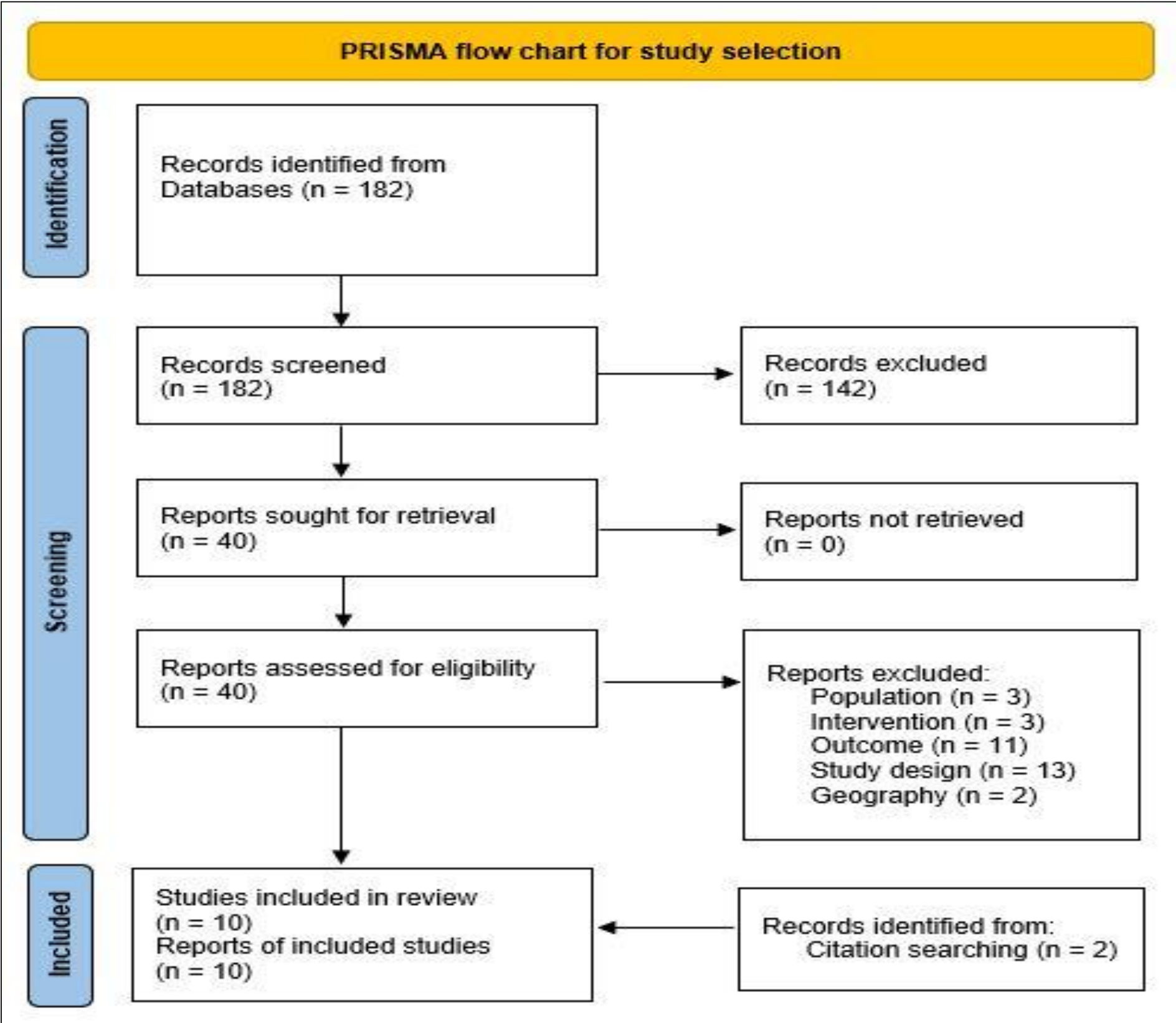
Diagnosed and undiagnosed prevalence in 2020, USA, per 100,000 males are 2.57 and 1.14 HB respectively.¹

Though HB affects only a small portion of population, it imposes high overall cost and financial burden on individuals, payers, and society in general.

Current systematic literature review (SLR) aimed to summarize the economic burden reported in patients with HB.

Methodology

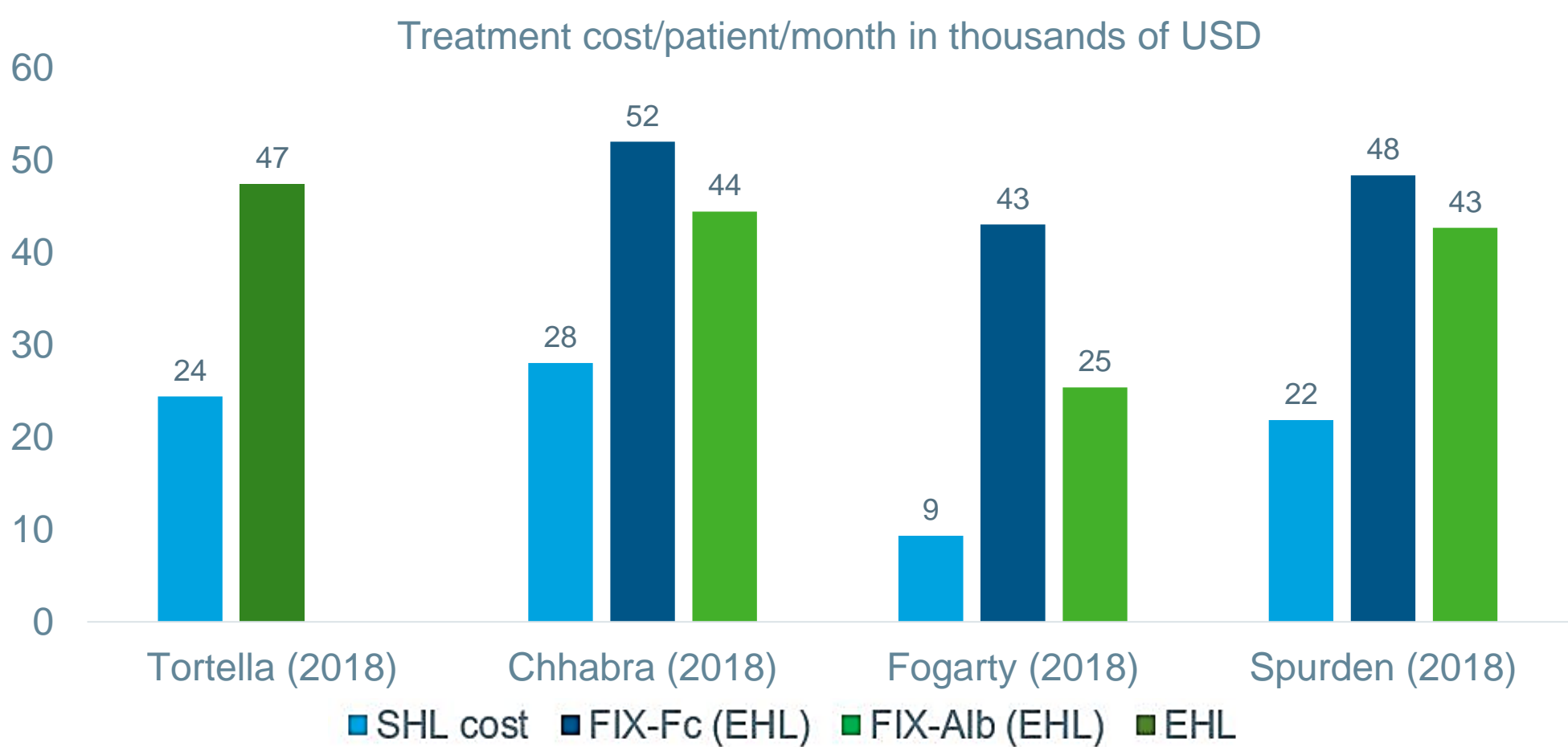
- Data sources:** An extensive literature search was conducted via Ovid on MEDLINE, EMBASE and Cochrane databases by combination of Emtree/ MeSH terms and keywords from inception to December 2021. Bibliography of included studies and supplementary searches were also conducted for additional publications.
- Eligibility Criteria:** Observational studies investigating treatment cost of HB without inhibitors with both short half life (SHL) and extended half-life (EHL) products in USA were included.
- Screening process:** Publications were screened for eligibility by two reviewers at abstract and full-text level.



Results

- Ten studies were included after screening 182 citations. Included publications captured data from claims database (n=9) and population survey (n=1).
- All the publications studied HB patients without inhibitor.
- Median FIX expenditure per-patient-per-month (PPPM) ranged from \$25,389 to \$51,986 for extended half-life (EHL) and \$9,347 to \$28,025 for short half-life (SHL) product cohort.^{2,3}
- Average inpatient and outpatient cost of EHL vs SHL was \$0 vs \$15,110 per-patient-per-year (PPPY) and \$3,529 vs \$17,709 PPPY, respectively.⁷

- CHESS US and CHESS US+ study reported mean annual direct medical cost (\$614,886), mean annual non-medical direct costs (\$2,371), and indirect costs (\$6,931).¹⁰ Another study reported mean direct medical cost per year to be \$201,635.¹¹
- Mean increase in expenditure per year after switch from SHL to EHL 131,748.⁵



Study (year)	Cost per patient (USD)	
	SHL	EHL
Tortella (2019) ⁴	\$202,476 /year	FIX-Fc: \$475,532 /year FIX-Alb: \$590,972 /year
Tortella (2018) ⁵	Median (IQR): \$24,414 (\$12,643-44,703) /month	Median (IQR): \$47,419 (29,886-76,999) /month
Chhabra (2018) ³	Median (IQR): \$28,025 (\$39,587) /month	Median (IQR): FIX-Fc: \$51,986 (\$49,520) /month FIX-Alb: \$44,425 (\$35,602) /month
Bowen (2018) ⁶	\$116,909 / six months	\$230,210 / six months
Li (2018) ⁷	Inpatient: \$15,110 /year Outpatient: \$17,709 /year	Inpatient: 0 /year Outpatient: \$3,529 /year
Fogarty (2018) ²	Median (IQR): \$9,347 (\$20,557) /month	Median (IQR): FIX-Fc: \$43,014 (\$52,517) /month FIX-Alb: \$25,389 (\$23,163) /month
Spurdén (2018) ^{a8}	\$48,259/ three months	\$165,022 / three months
Spurdén (2018) ^{b9}	Median (IQR): \$21,849 (\$36,102) /month	Median (IQR): FIX-Fc: \$48,336 (\$49,970) /month FIX-Alb: \$42,664 (\$45,135) /month
Burke (2021) ¹⁰	\$397,491 /year	\$788,491 /year

Table: Cost of Treating Hemophilia B without inhibitors in the USA
Abbreviations: EHL: extended half life; FIC-Alb: Albutrepenonacog alfa; FIX-Fc: Elfrenonacog alfa; FIX: Factor 9; IQR: Inter quartile range.

Conclusion

- This SLR provides economic burden of HB patients without inhibitor using FIX treatment in the United States.
- Cost of treatment per patient was higher in EHL group compared to SHL group.
- Within the EHL cohort, cost of treatment was found to be higher in FIX-Fc group compared to FIX-Alb group.
- Further cost analyses could provide additional insight into these findings and can be utilized as inputs for the economic modelling.

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