The Economic Burden of Severe Hemophilia A Without Inhibitors among Adults with Health Insurance Coverage in the United States: Insights from the "Cost of Hemophilia across the US: a Socioeconomic Survey" CHESS US and CHESS US+ Studies

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Background

- Severe hemophilia A (SHA) is a congenital bleeding disorder characterized by substantial clinical burden due to severe and repeated bleeding in the absence of prophylactic Factor VIII (FVIII) replacement therapy[1]
- Hemophilia has been associated with high treatment costs, with the vast majority generally associated with clotting factor[2]

Aim

- To understand disease burden for people with hemophilia and the healthcare system by quantifying societal economic impact (in terms of direct, direct non-medical and indirect costs) associated with SHA without inhibitors

Methods

- Cross-sectional data on insured adults with SHA without inhibitors from the 'Cost of Hemophilia across the US: a Socioeconomic Survey' (CHESS US)[3], a physician-reported database, and a separate patient-reported database (CHESS US+)[3] were employed to quantify:
  - Direct medical costs (DMC) (including factor treatment, hospital visits, consultant visits, tests) based on physician-reported data in CHESS US
  - Direct non-medical costs (DNMC) (including professional and/or informal caregiver costs, disability allowances, devices and home alterations, center visits transit costs, over the counter medication, alternative therapies) based on data from CHESS US+
  - Indirect costs (IC) associated with the impact of disease and current treatments on work and activities (e.g., labor force participation and underemployment, work productivity, absenteeism, early retirement) based on patient-reported data from CHESS US+
- The outcomes were summarized and compared using descriptive statistics as mean ± standard deviation (SD) or freq. (in; %) across:
  - **FVIII therapy regimen** (total cohort or prophylaxis cohort)
  - **Insurance type** (private or government insurance)
- Mean (SD) annual costs are reported in 2017 US$ (year data was collected)

Results

- Two hundred eighty-one insured non-inhibitor SHA patients were included in the analysis from the CHESS US cohort and 241 from the CHESS US+ cohort, mean age was 30.1 and 34.9 respectively **(Table 1)**
- A total of 522 patient records were included
- Societal impact costs for those on prophylaxis treatment were primarily driven by early retirement costs and caregiver costs **(Table 2)**
- **DMC** (n=281) was similar across insurance types **(Table 3)**, but were higher in the prophylaxis cohort ($656,707) compared to the overall figure ($493,152) **(Figure 1)**
  - FVIII treatment costs accounted for >95% of DMC **(Table 3)**
  - Hospitalization costs represented the largest non-drug component of DMC **(Table 3)**
- **DNMC** (n=241)
  - Were considerably higher (~$9,000) for patients covered by government insurance when compared to their privately insured peers **(Table 3)**
  - Disability entitlement and homecare were the main drivers of DNMC **(Table 3)**
  - For those receiving disability allowance (n=36), the mean annual payment was $12,832 ($8,825)
- **IC** (n=241)
  - IC were five-fold higher in government-insured patients **(Table 3)**
  - IC were predominantly driven by early retirement and underemployment **(Table 3)**
  - DNMC and IC were both higher for the prophylaxis cohort than for the total cohort **(Figure 2)**

Table 1. Cohort characteristics

	N=522
Employed, n *(%)*	355 (68%)
Receiving prophylaxis, n *(%)*	402 (77%)
Privately insured, n *(%)*	327 (63%)
Age (Mean ± SD) CHESS US cohort (n=281) CHESS US+ cohort (n=241)	 30.1 ± 25.0 34.9 ± 11.5

Abbreviations: SD, standard deviation.

Table 2. Prophylaxis societal impact costs

(Mean ± SD)	N=193
Early retirement costs	$6,509 ± 16,918.3
Underemployment costs	$2,096 ± 8,369.2
Caregiver costs	$3,277 ± 17,267.3
Disability entitlement* *Government insurance (N=76)* *Private insurance (N=117)*	$2,157 ± 6,113.2 $3,141 ± 5,745.3 $1,517 ± 6,282.3
Absenteeism costs	$923 ± 3,665.7
Other non-medical costs**	$779 ± 1,242.6

*This has been stratified by insurance type. **This includes devices and home alterations, transit costs to HTC visits, over the counter medication and alternative therapies.

Table 3. DMC, DMNC & IC by insurance type

	Insurance type		Total N=281
	Government	Private	
	N=102	N=179	
Direct Medical Cost *(Mean ± SD)*	$497,902 ± 713,399.9	$490,446 ± 757,306.9	$493,152 ± 740,396
Factor Treatment cost	$475,935 ± 718,061.9	$476,675 ± 756,290.0	$476,407 ± 741,351
Hospitalisations	$20,985 ± 48,656.3	$12,812 ± 42,217.0	$15,779 ± 44,749
	N=93	N=148	N=241
Direct Non-Medical Cost *(Mean ± SD)*	$11,181 ± 27,833.3	$2,182 ± 6,847.4	$5,655 ± 18,573.0
Homecare	$7,154 ± 25,153.7	$341 ± 3,172.3	$2,970 ± 16,116.1
Disability entitlement	$3,058 ± 5,649.2	$1,200 ± 5,615.0	$1,917 ± 5,689.2
Indirect Cost *(Mean ± SD)*	$17,610 ± 22,249.6	$3,317 ± 10,838.2	$8,832 ± 17,615.7
Early retirement	$12,428 ± 21,799.1	$2,037 ± 9,944.7	$6,047 ± 16,383.7
Underemployment	$3,727 ± 10,899.7	$715 ± 4,591.1	$1,878 ± 7,785

Abbreviations: SD, standard deviation.

Figure 1. DMC and FVIII cost

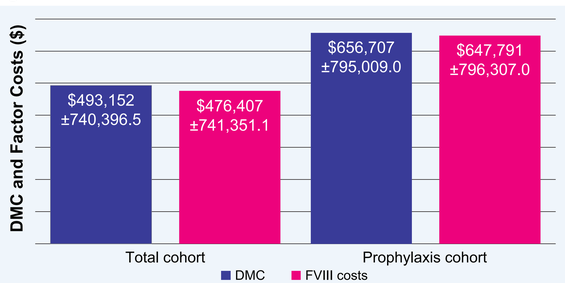
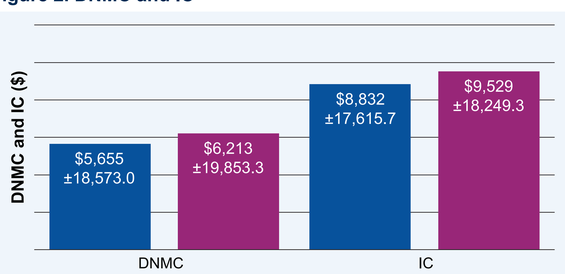


Figure 2. DNMC and IC



Limitations

- Due to the voluntary and retrospective nature of data collection in CHESS US/US+, recall bias could result in over- or under-estimation of the costs reported. A degree of selection bias cannot be excluded

Conclusions

- FVIII cost, particularly among prophylaxis patients, remains the predominant cost-driver from both healthcare system and societal perspectives
- DNMC and IC also represent important sources of societal cost, affecting patients, government programs and society, accounting for approximately $15,000 a year
- Improved personalized care, care coordination, and development of new treatment options may help to reduce healthcare system and societal cost burden
- Further research is warranted to better characterize the costs to the healthcare system and society as a whole associated with hemophilia and its care, to better inform future resource allocation decisions

References

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