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THE BURDEN OF HAEMOPHILIA IN 5 EUROPEAN COUNTRIES: METHODS AND RESULTS FROM THE COST OF HAEMOPHILIA IN EUROPE: A SOCIOECONOMIC SURVEY (CHESS) 2022

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OBJECTIVE: The primary objective was to quantify haemophilia-related costs, by disease severity, encompassing direct medical costs (e.g. treatment costs, hospital visits), direct non-medical costs (e.g. health aids/devices, travel costs) and indirect costs (e.g. work productivity impact, caregiver burden). Clinical burden was also assessed.

CONCLUSIONS: These findings demonstrate the significant clinical and financial burden of haemophilia in Europe.

BACKGROUND

- **Haemophilia** is a congenital, recessive, X-linked **bleeding disorder** caused by a deficiency or absence of coagulation factor VIII or IX, which leads to frequent, acute and prolonged spontaneous or traumatic bleeding events.[1]
- The **considerable burden** of haemophilia in Europe necessitates continued generation of up-to-date information.
- CHESS aims to assess the **socio-economic burden** of haemophilia in adult male patients of all severities in 5 European countries: Italy, France, Germany, Spain and the United Kingdom.

METHODS

- CHESS is a **12-month retrospective, repeated cross-sectional** study with questions covering physician-reported **demographic, clinical and healthcare resource use** data and patient-reported **productivity and humanistic burden**.[2]
- Clinical outcomes assessed included **annual bleed rate** (ABR) and **problem joints**.[3]
- **Cost components** were estimated based on reported quantities and unit costs sourced from literature and presented in 2022 €.
  - **Per-patient direct medical cost** (DMC) (including haemophilia-related health-resource utilisation) was based on physician-reported quantities.
  - **Direct non-medical costs** (DNMC) (including disability allowances, professional caregiver costs, devices and home alterations, centre visits transit costs and over the counter medication).
  - **Indirect costs** (IC) associated with the impact of disease and current treatments on work and activities (e.g. patient and care-giver work productivity, absenteeism, labour force participation, underemployment and early retirement).
  - **Total costs** (per patient) were quantified by summing the costs across components.
  - **Population cost** was extrapolated by multiplying the mean treatment cost & other DMC by prevalence estimates from the 2021 WFH Annual Global Survey.[3]
- Results are presented by **haemophilia severity** (based on baseline factor level): mild (>5-40%), moderate (1-5%), and severe (<1%).

RESULTS

- The final sample consisted of 754 physician-forms and 166 corresponding patient-forms.
- Half of the cohort had **severe** haemophilia, 19% **mild** and 31% **moderate** condition.
- In the **mild** cohort, mean (SD) **ABR** was reported as 1.1 (3.2), increasing to 1.5 (2.1) and 2.7 (4.6) for **moderate** and **severe** respectively (Table 1).
- **Joint damage** was also observed, with mean (SD) **problem joints** reported as 0.8 (1.4), 0.8 (1.3) and 1.0 (1.2) for the mild, moderate and severe cohorts.
- The highest per-patient cost component was **treatment cost**, averaging approximately **€47,000** for mild, **€99,000** for moderate and **€380,000** for severe condition, which represented an average of **94%** of total cost (Table 2).
- **Total costs** per-patient excluding treatment also increased with severity, at approximately €3,000, €9,000 and €12,000 respectively (Table 2).
- For the severe cohort, indirect costs represented **59%** of total non-treatment costs, whilst for the mild and moderate cohorts, DMCs represented **69%** and **42%,** respectively.
- All non-treatment **population cost categories** were highest for **severe** haemophilia, with total cost at €114.6m. M**oderate** and **mild** condition accounted for €36.8m and €42.3m, respectively (Figure 1).

Table 1. Demographics and Clinical Outcomes by Haemophilia Severity

	Haemophilia Severity		
	Mild	**Moderate**	**Severe**
Demographics	N=143	N=231	N=380
Age (yrs*), mean (SD)*	36.3 (13.6)	37.8 (15.11)	37.6 (14.7)
BMI*, mean (SD)*	25.0 (2.8)	24.4 (2.9)	24.7 (3.1)
Subtype*, N (%)*			
Haemophilia A	110 (76.9)	187 (81.0)	315 (82.9)
Haemophilia B	33 (23.1)	44 (19.1)	65 (17.1)
ABR*, mean (SD)*	1.10 (3.17)	1.45 (2.08)	2.71 (4.56)
Problem joints*, mean (SD)*	0.83 (1.44)	0.81 (1.27)	0.99 (1.17)

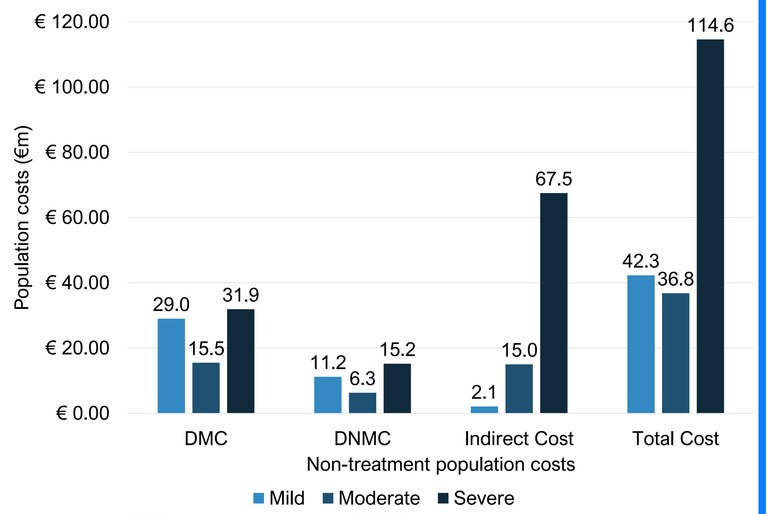
Abbreviations: ABR, annual bleeding rate; SD, standard deviation

Table 2. Costs Per Patient, by Haemophilia Severity

	Haemophilia Severity		
	Mild	**Moderate**	**Severe**
Cost component	**Per-patient cost, Mean (SD)**		
	N=143	N=231	N=380
Treatment Cost	€46,713 (137,001)	€98,731 (174,927)	€379,826 (298,632)
Non-treatment costs	N=22	N=38	N=106
DMC	€2,169 (4,151)	€3,652 (5,520)	€3,219 (4,433)
DNMC	€839 (1,426)	€1,577 (3,395)	€1,561 (2,554)
Indirect Cost	€156 (525)	€3,526 (6,707)	€6,824 (14,696)
Total Cost	€3,165 (5483)	€8,755 (12,568)	€11,603 (17,284)

Abbreviations: DMC, direct medical cost; DNMC, direct non-medical cost; SD, standard deviation.
Note: Calculation based on recorded consumption and publicly available list prices

Figure 1. Non-treatment Population Costs (€m), by Haemophilia Severity



Abbreviations: DMC, direct medical cost; DNMC, direct non-medical cost; m, million

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