The cost of severe hemophilia in five European countries: the CHESS study

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INTRODUCTION

- Haemophilia is a genetic disorder that causes a deficiency of a clotting factor in the blood. There are two main forms, (Haemophilia A and Haemophilia B), classified as mild, moderate or severe based on the extent of the factor deficiency.
- Individuals with severe haemophilia represent approximately one-third of the haemophilia population in Europe [1,2] and can experience recurrent spontaneous bleeds, often in the absence of any trauma event. In many cases, recurrent joint inflammation (arthropathy) leads to joint deformity, reduced mobility, and chronic pain.
- Initiation of prophylactic factor replacement therapy at an early age is considered critical to reducing the frequency and severity of bleed events, and subsequent arthropathy. However, uptake of prophylaxis varies substantially across European countries, and the cost-effectiveness of prophylaxis remains unclear [3–5].

OBJECTIVES

- In 2014, the 'Cost of Haemophilia across Europe – a Socioeconomic Survey' (CHESS) study was developed as the first comprehensive cost-of-illness study in severe haemophilia across five European countries (EU5).
- The study took a societal perspective and employed a 'bottom-up' methodology, with the aim of quantifying the annual direct and indirect costs of severe haemophilia A and B in adults across France, Germany, Italy, Spain, and the UK.

METHODS

- A cross-section of haemophilia specialists (surveyed between January and April 2015) provided demographic and clinical information and 12-month ambulatory and secondary care activity for patients via an online survey.
- Physicians completed a patient record form (PRF) for the next 8-10 eligible patients and invited each patient to complete a corresponding patient self-completion (PSC) questionnaire.
- Patients provided direct and indirect non-medical cost information, including work loss and out-of-pocket expenses, as well as information on quality of life and adherence.
- A cost database was developed for each country using publically-available information.
- The project was governed and approved by the University of Chester Ethics Committee.

RESULTS

- A sample of 139 physicians participated in the study –the largest ever captured within therapeutic area – profiling 1,285 patients (approximately 18% of the population of individuals with severe hemophilia across the EU5) (Table 1).
- The extrapolated annual cost of severe hemophilia was estimated at EUR 1.4 billion, or just under EUR 200,000 per patient (Figures 1 and 2).
- Drug costs, as anticipated, made up 98% of direct costs. Indirect costs consisted predominantly of out-of-pocket costs and informal care costs.

Table 1. CHESS study population characteristics (N=1,285)

Country	
France	272
Germany	194
Italy	280
Spain	218
UK	321
Haemophilia subtype (%)	
A	996 (78%)
B	289 (22%)
Age (mean (SD))	35.9 (14.7)
Treatment strategy: Prophylaxis (%)	737 (57%)
Inhibitor history (%)	
Never	1,091 (85%)
Previously	136 (11%)
Currently	58 (5%)
Patients with coinfection (%)	
HIV	38 (3%)
HCV	70 (5%)

Figure 1. Annual per-patient costs of severe hemophilia in the CHESS study population (EUR)

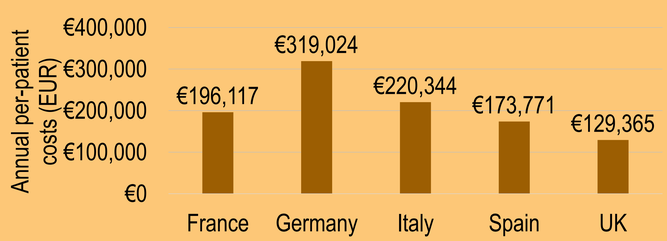
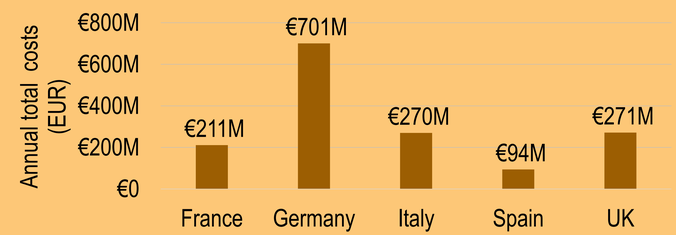


Figure 2. Extrapolated annual total costs of severe hemophilia, EU5 (EUR)



CONCLUSIONS

- The CHESS study captured the direct and indirect costs at patient level on a scale that has not been previously achieved (based on a sample of around 18% of the severe hemophilia population).
- Severe hemophilia is a costly and burdensome disease in all five CHESS study countries.
- The differential costs and outcomes for patients across the EU5 suggests more research is required on optimal models of care.
- The study has enabled the production of a granular database from which researchers can produce a comprehensive burden-of-illness study on a larger scale. This will ultimately help the wider community understand costs and societal burdens associated with severe hemophilia.

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