An Introduction to "The Cost of Haemophilia across Europe – a Socioeconomic Survey" (CHESS)

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].

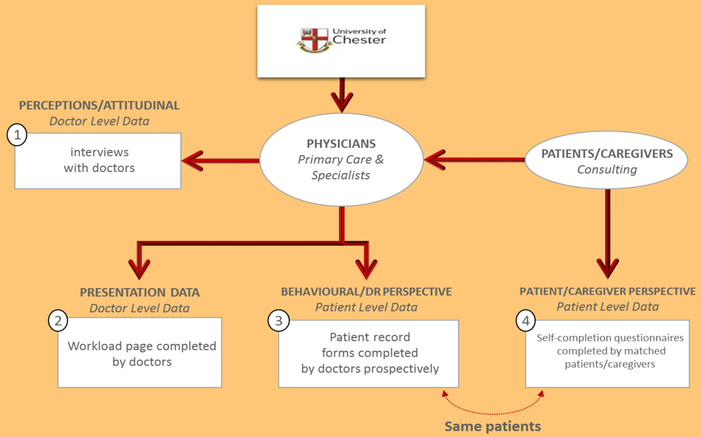
OBJECTIVES

- 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.
- All direct medical and non-medical costs were sourced from publically available data. All local currency total costs were converted to Euros using the official conversion rate as of 30th May, 2015.

Figure 1. CHESS study data capture design



- Per-patient costs were calculated by multiplying the quantities of the resource used with the national unit price of each resource. To extrapolate the sample costs to population size, per-patient costs were multiplied by country-specific estimates of the severe haemophilia population.
- The project was governed and approved by the University of Chester Ethics Committee.

Table 1. Summary of cost elements captured in the CHESS study

Cost type	Cost element
Direct medical costs	Specialist consultations (hematology, other)
	Hemophilia-related surgical procedures
	Bleed-related hospitalisations
	Diagnostic tests and examinations
	Current treatments & dosage
	FVIII/FIX consumption
Direct non-medical costs	Professional care provider
	Medical-related travel
	Requirement for aids / equipment
Indirect costs	Work productivity impact
	Informal care provision
	Self-medication

RESULTS

- A total of 139 physicians participated in the study from across the EU5, capturing information on 1,285 patients (996 hemophilia A and 289 hemophilia B – approximately 16% and 28% of the respective target populations).
- A total of 551 patients completed corresponding PSC questionnaires, with over 100 variables captured on each patient. Study demographics were largely balanced across the sample and verified by the SSC.

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).
- Across all the five EU countries reviewed, severe hemophilia is a costly and burdensome disease.
- 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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