Background and Declarations

Senior Lecturer of Health Economics (University of Chester)

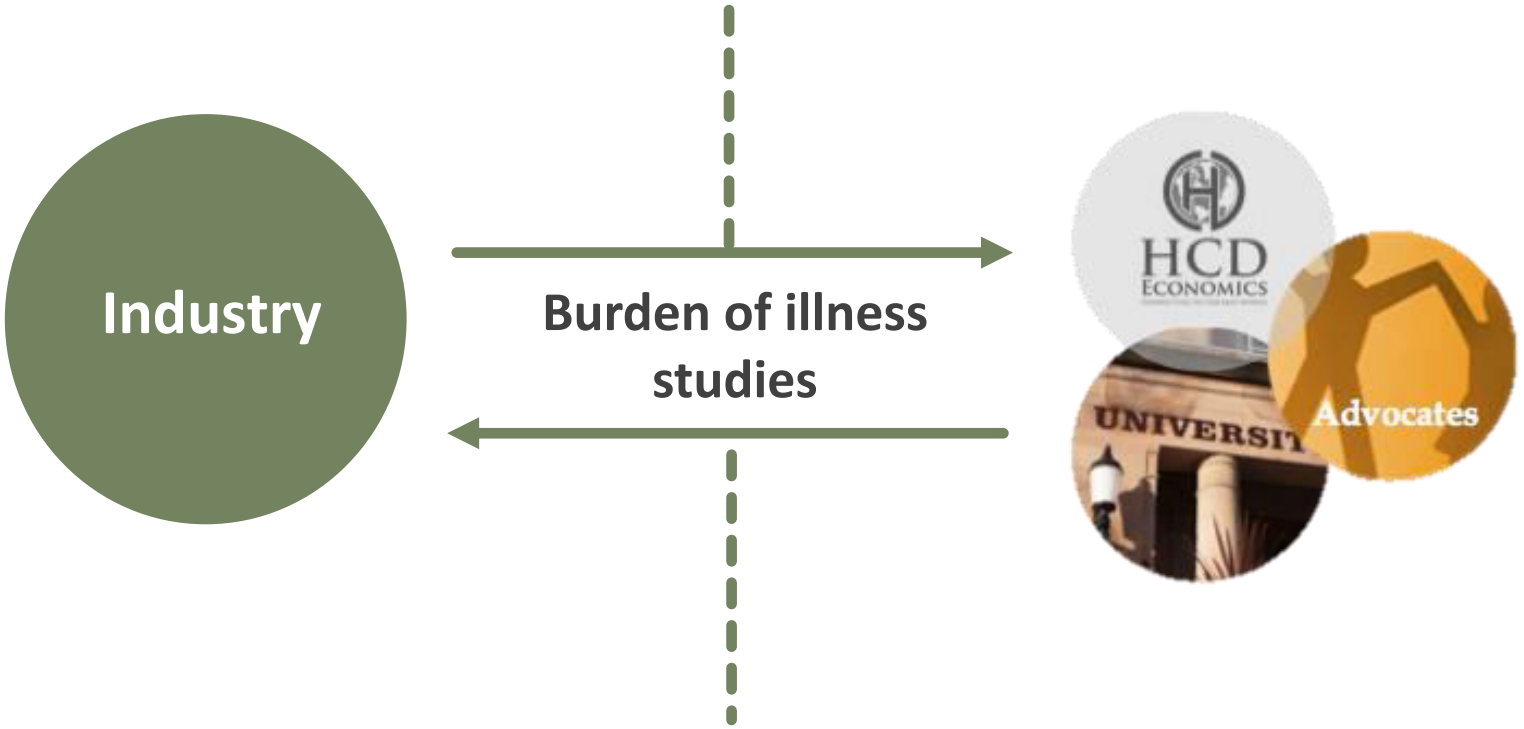
CEO HCD Economics
- Operating for 5 years with staff of 24

Community roles
- UK Haemophilia Society (Board member)
- EHC (MASAG member)
- WFH (Lead health economist on data and economics committee, WBDR SSC member)

Payer Role
- Strategic support in NHSE and member of CRG for bleeding disorders
- Research planning with rare disease function in NICE as well as data sharing arrangement

Research conducted via unrestricted research grants from primary sponsors;
- SOBI; Novo Nordisk; Shire; Roche; Bayer; Alnylam; Biomarin; Sanofi/Genzyme

Burden of Illness (BOI): Study Model



Background of CHESS 1

A summary of the original study

Background (CHESS 1)

Haemophilia is a bleeding disorder brought about by a deficiency in a clotting factor

- Severe haemophilia: factor <1%

Growing prevalence: 21.6/100,000 2011 vs 9.3/100,000 1974

Extremely high per patient costs, but costly knock-on effect of poor treatment

- Economic burden: target joints & surgeries, etc
- High societal burden in terms of work loss, carer burden

Treatment is seeing significant developments with the introduction of the long-acting treatments into the existing paradigm

- Commissioning is far from guaranteed (so we need evidence)

POLICY CURRENTLY BASED ON POOR EVIDENCE (IQWIG)

Background (1)

In 2015, the Haemophilia Society, the University of Chester and HCD Economics worked together to provide a bottom up burden of Severe Haemophilia study

Evidence and data of this granularity/quantity had not previously been collated

The original study was a huge success (138 haematologists and 1,285 patients)

- Subsequent utilisation of the study data in the form of publications and policy influencing

Study Approach

Cross-sectional & chart pull based disease audit of severe haemophilia across EU5 (France, Germany, Italy, Spain, UK)

Study ethically approved by the University of Chester's International Health Economics Research Group (IHERG)

Governed by a steering committee of key stakeholders from within the community

- Haematologists, specialist nurses, UKHS, UoC, EHC & UKHCDO etc.

Fieldworks developed by clinical and HEOR specialists

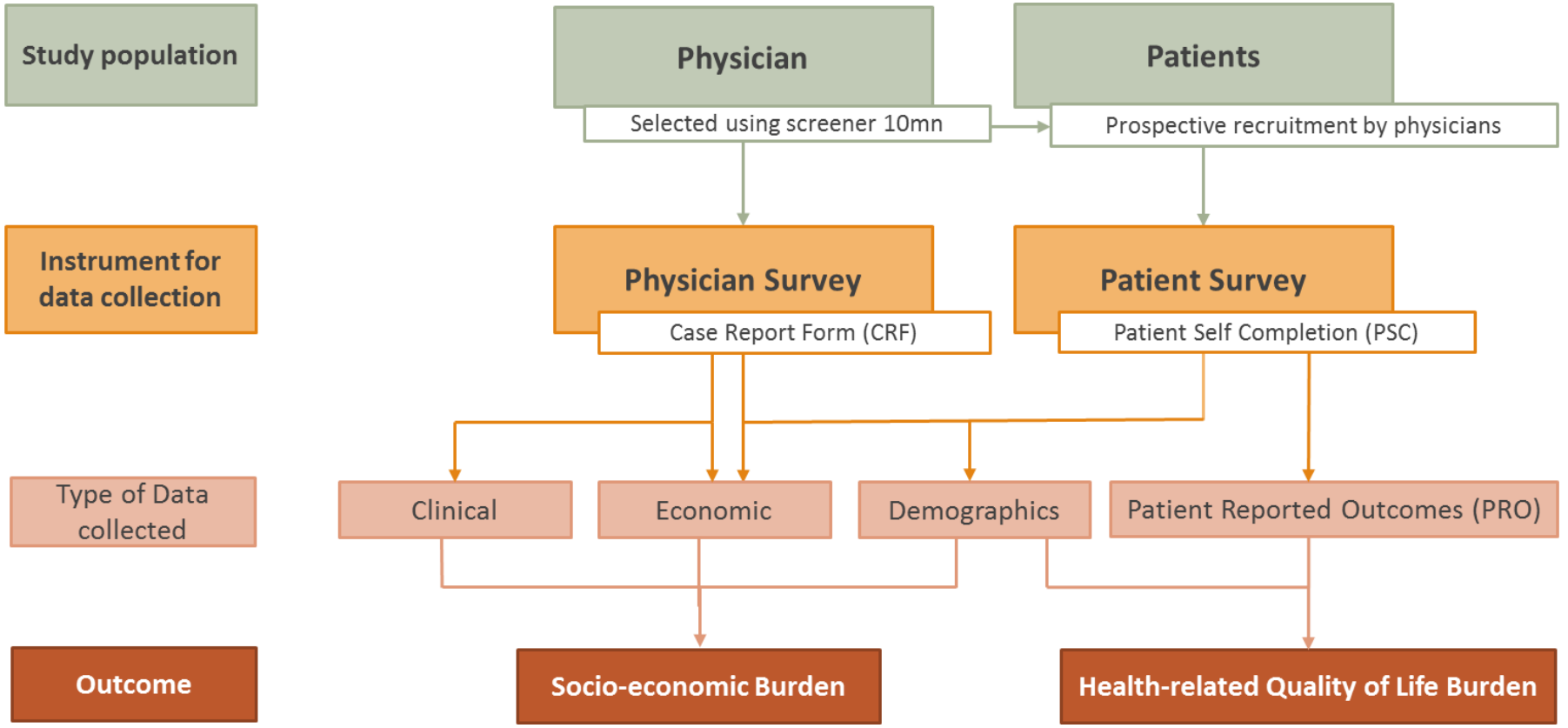
Collating both patient and doctor level information

Study Steering Committee

Study Steering Committee member list
(Project lead: Jamie O'Hara)

Member	Organisation	Role
Liz Carroll	Haemophilia Society	Chair
Mark Skinner	Independent	Deputy Chair
Gordon Greenshields	UoC	Secretary
Giuseppe Mazza	Icore Onlus (Italy) / EHC	Representative
Daniel-Anibal Garcia	Federación Española de Hemofilia	Representative
Stefanie Oestreicher	Deutsche Hämophiliegesellschaft	Representative
Thomas Sannie	Association Française des Hémophiles	Representative
Mike Makris	UKHCDO / University of Sheffield	Representative
Ann Bryan	UoC	Representative
David Hughes	UoC / HCD Economics	Representative
Paul Kingston	UoC	Representative
Declan Noone	EHC	Expert advisor

Data capture design…



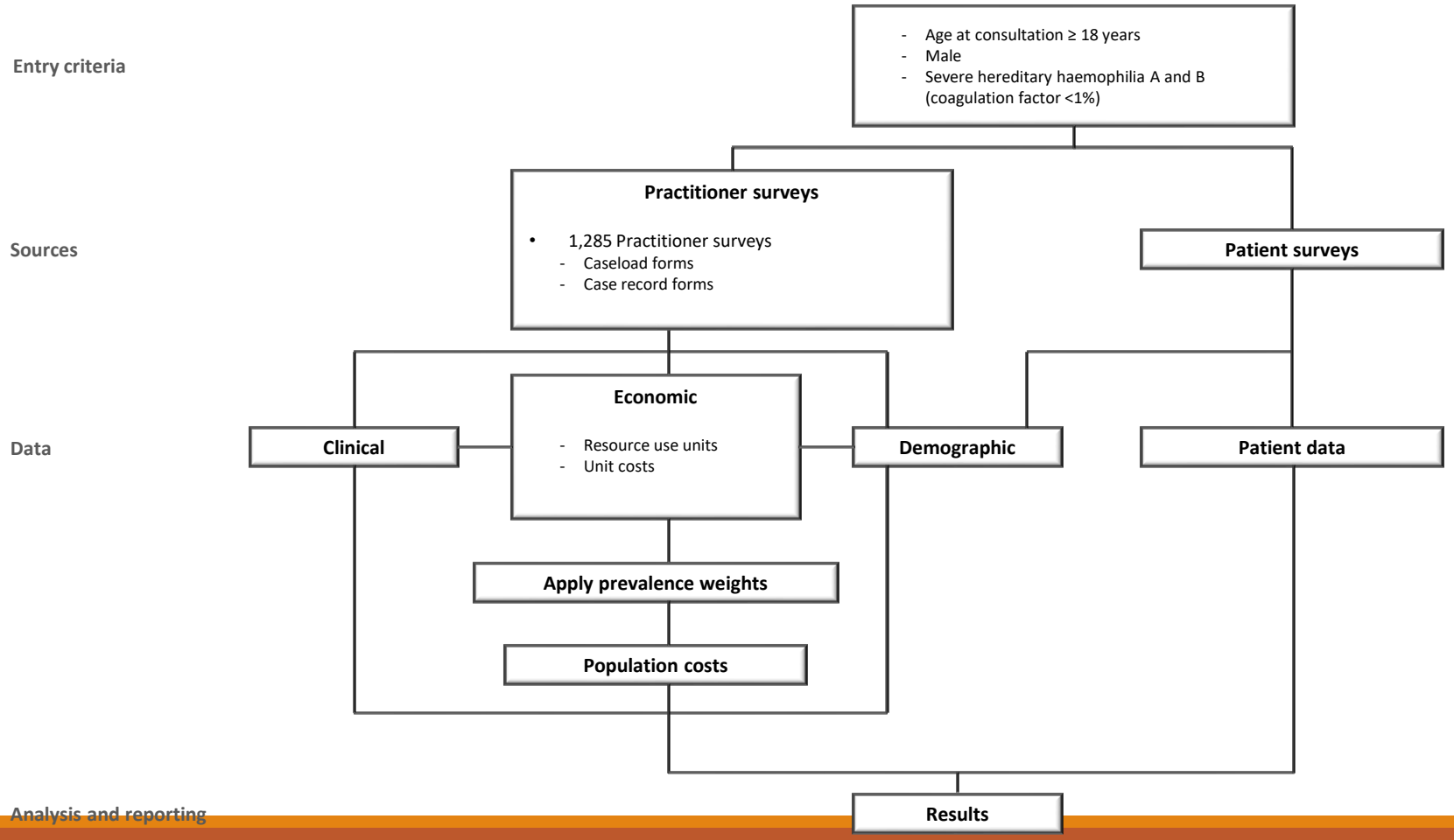
Cost sources

CHESS cost sources[a]

Country	Resources
France	Ameli, sante.gouv, ViDAL.fr, Catalogue Commun des actes médicaux
Germany	Kbv.de, meinpharmaversand.de, Einheitlicher Bewertungsmaßstab, rote-liste service
Italy	AIFA, agenziafarmaco.gov
Spain	Oblikue e-salud, Agencia espanola de medicamentos y productos sanitarios
UK	National Schedule of Reference Costs, the electronic Medicines Compendium etc.

[a] Excludes drug costs, which have been sourced via Study Steering Committee liaison and correspondence with domestic drug providers as costs are not in the public domain.

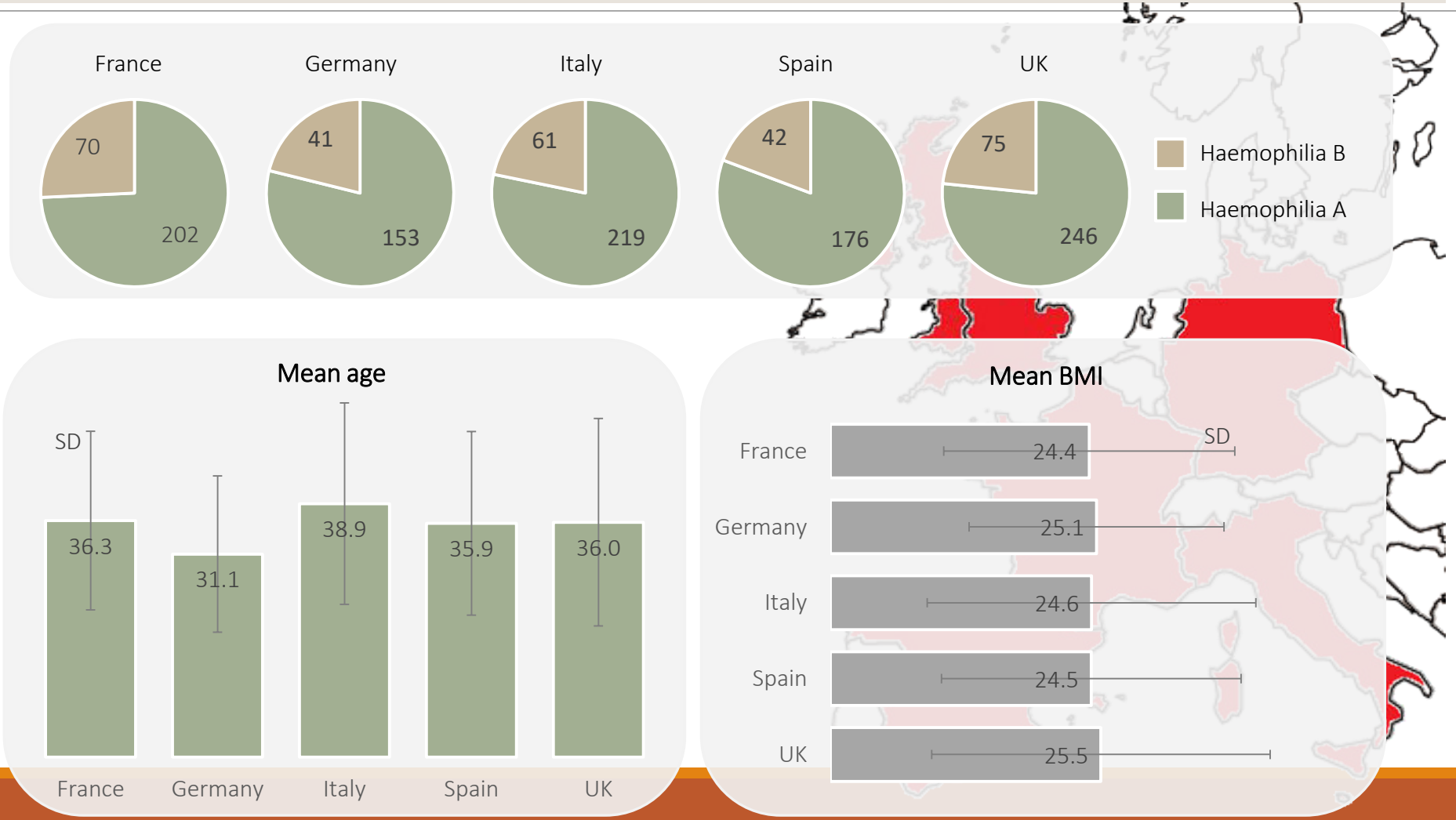
Study design: CHESS I



Results

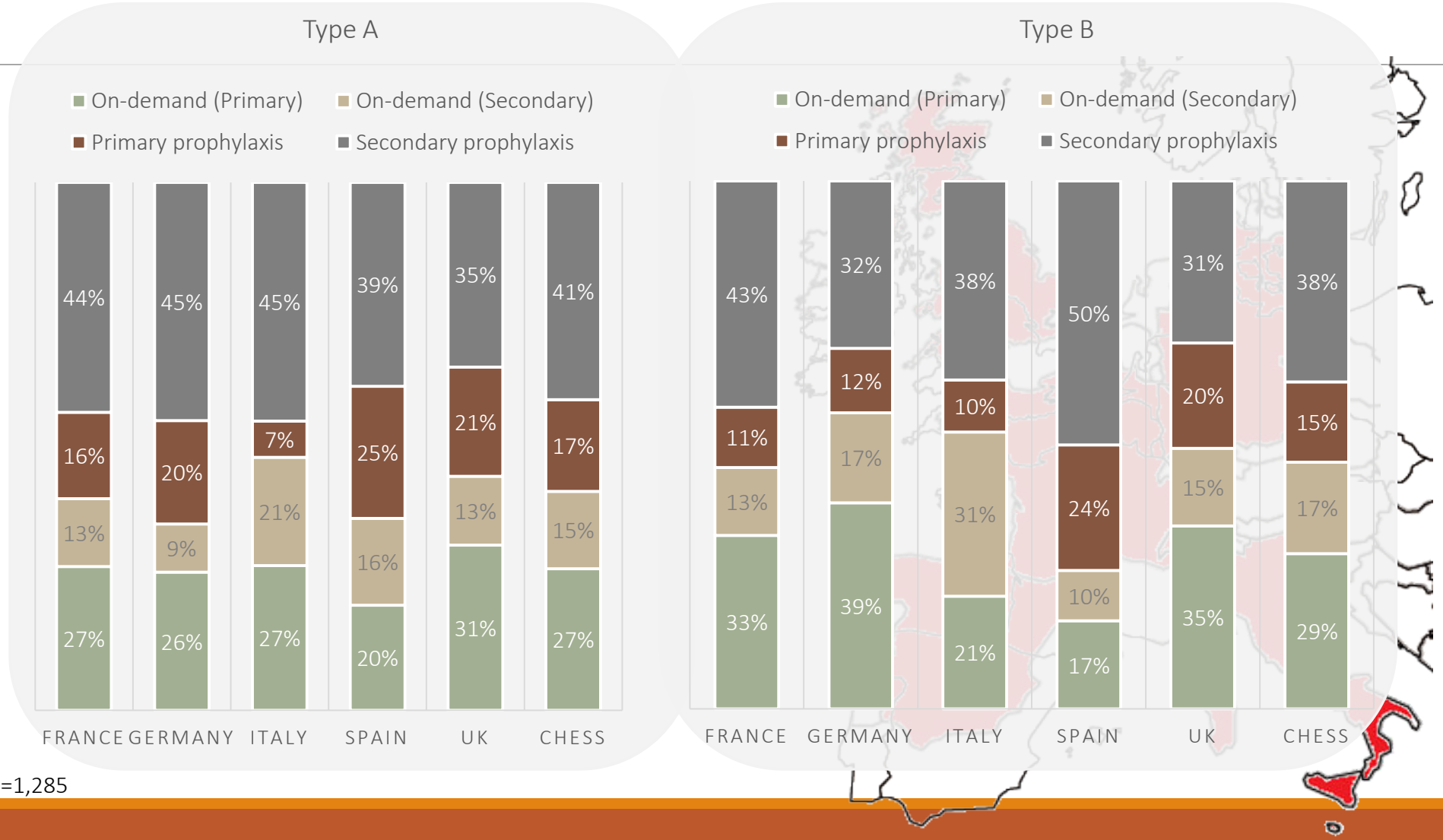
Sample; Epi; Treatment strategies; target joints

Demographics (all patients **n1,285**)



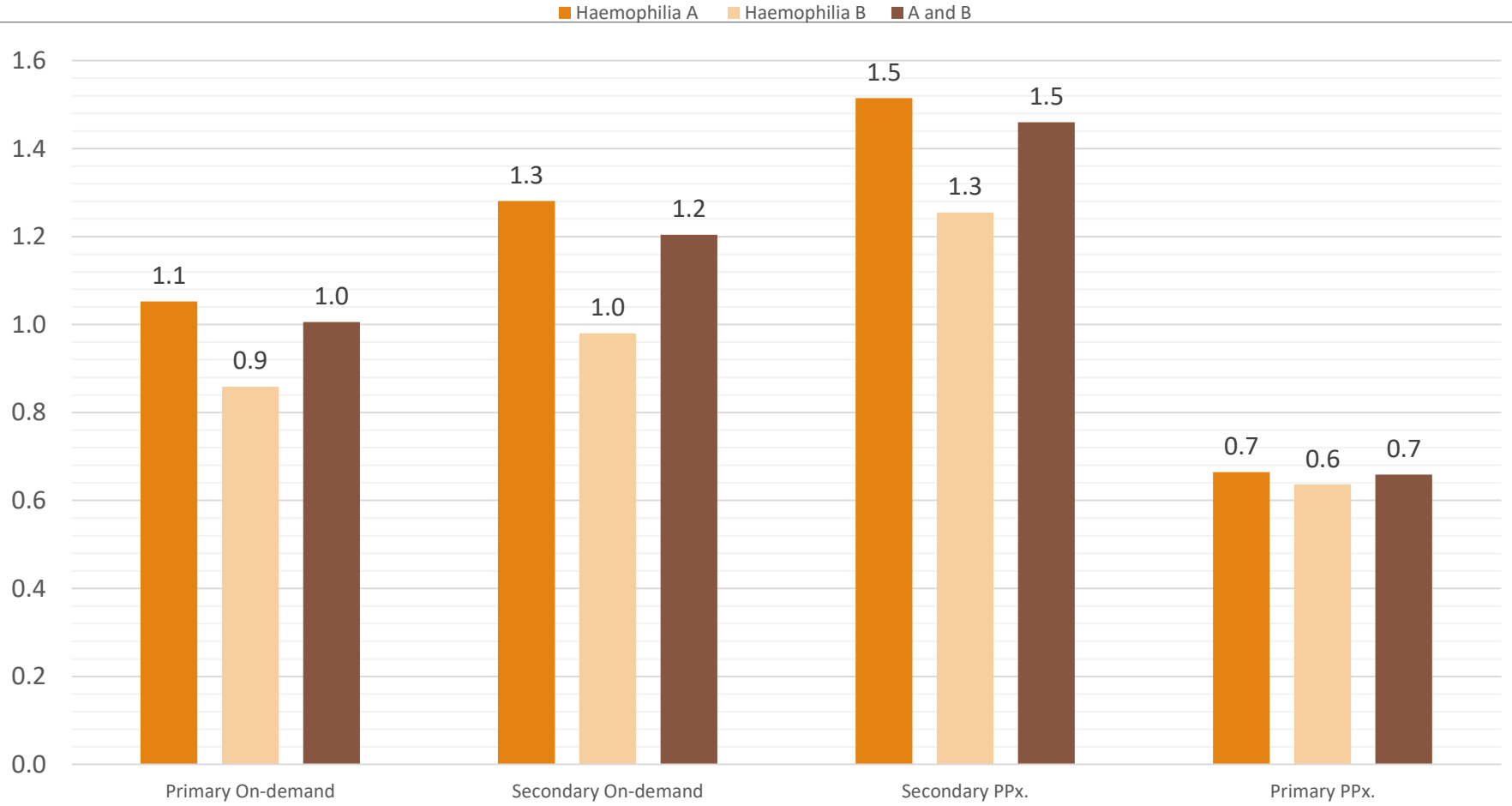
n=1,285

Treatment regimen

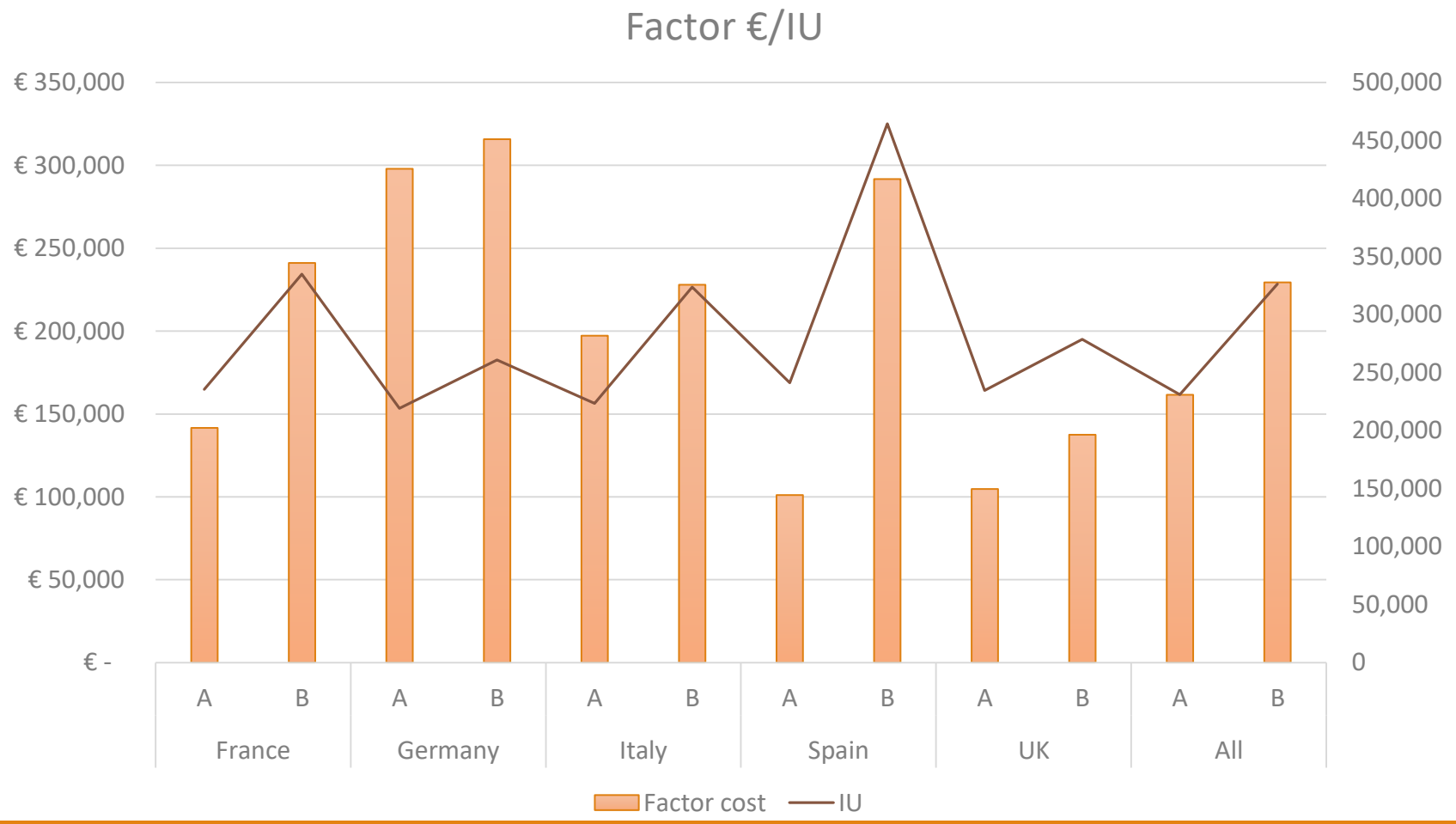


n=1,285

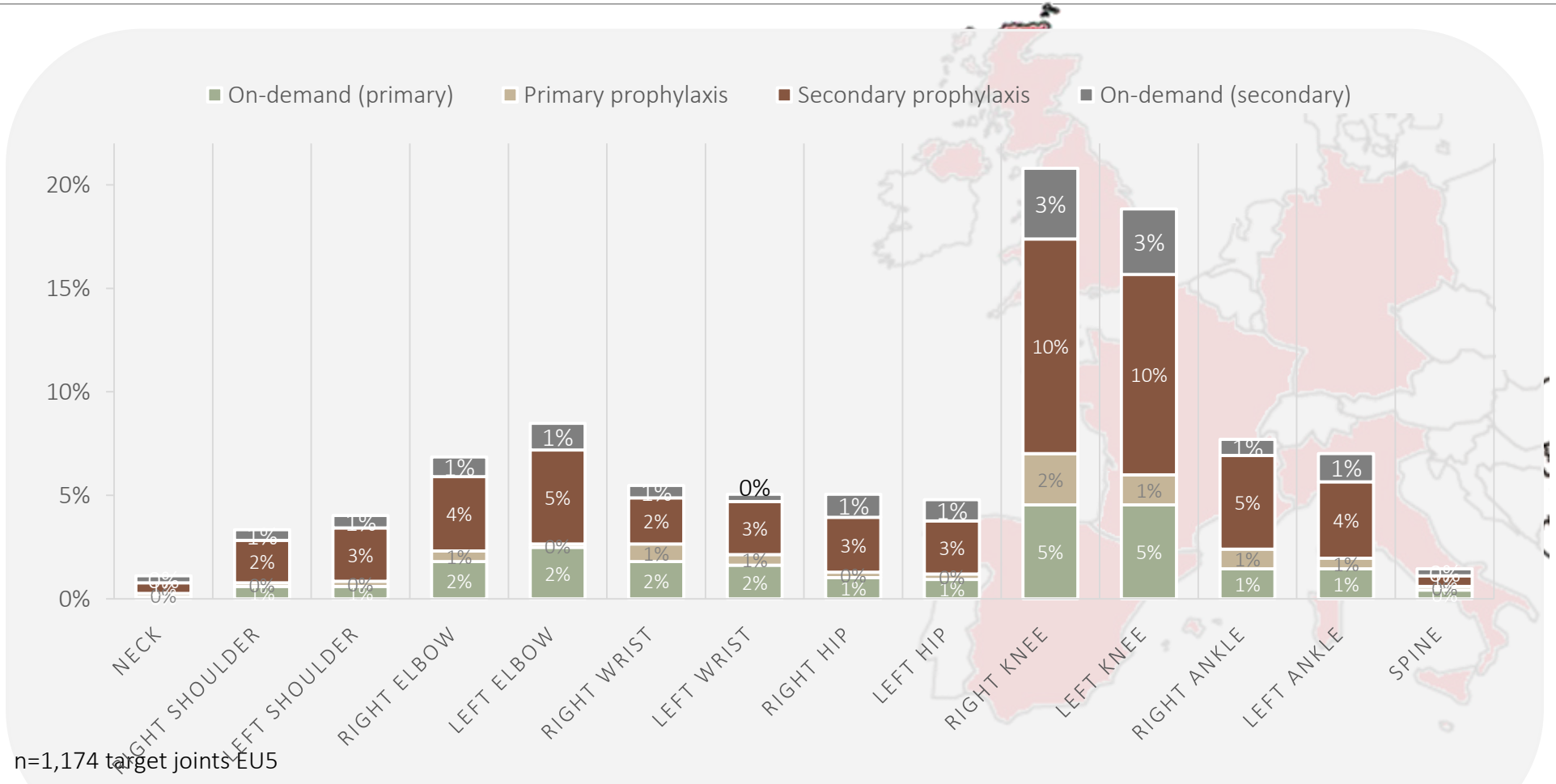
Target joint incidence by treatment type…



Annual factor costs vs IU per-patient



Target joints by treatment regimen



n=1,174 target joints EU5

Concluding comments

Drug costs account for over 95% of overall cost of severe haemophilia

Approximately 60% of all non-drug costs are indirect

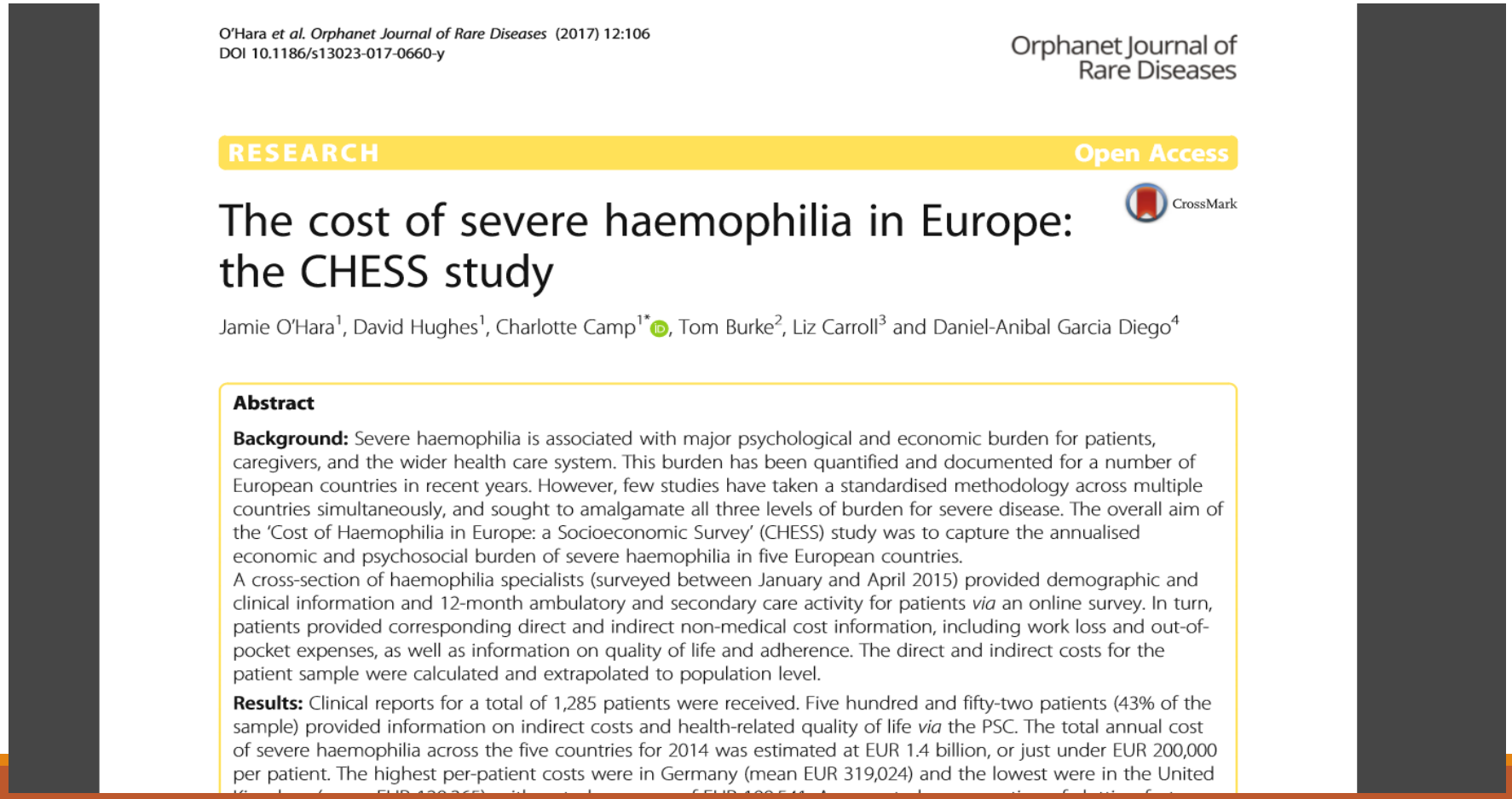
- Work lost and caregiver burden

Differential costs and outcomes for patients across the EU5, suggests more research is required on optimal models of care.

Concomitant conditions (very high anxiety levels reported in the CHESS cohort)

Good opportunity to learn more about the severe haemophilia population from the data captured

Results Manuscript…



O'Hara *et al. Orphanet Journal of Rare Diseases* (2017) 12:106
DOI 10.1186/s13023-017-0660-y

Orphanet Journal of Rare Diseases

RESEARCH Open Access

The cost of severe haemophilia in Europe: the CHESS study

Jamie O'Hara[1], David Hughes[1], Charlotte Camp[1*], Tom Burke[2], Liz Carroll[3] and Daniel-Anibal Garcia Diego[4]

Abstract

Background: Severe haemophilia is associated with major psychological and economic burden for patients, caregivers, and the wider health care system. This burden has been quantified and documented for a number of European countries in recent years. However, few studies have taken a standardised methodology across multiple countries simultaneously, and sought to amalgamate all three levels of burden for severe disease. The overall aim of the 'Cost of Haemophilia in Europe: a Socioeconomic Survey' (CHESS) study was to capture the annualised economic and psychosocial burden of severe haemophilia in five European countries.
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. In turn, patients provided corresponding 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. The direct and indirect costs for the patient sample were calculated and extrapolated to population level.

Results: Clinical reports for a total of 1,285 patients were received. Five hundred and fifty-two patients (43% of the sample) provided information on indirect costs and health-related quality of life *via* the PSC. The total annual cost of severe haemophilia across the five countries for 2014 was estimated at EUR 1.4 billion, or just under EUR 200,000 per patient. The highest per-patient costs were in Germany (mean EUR 319,024) and the lowest were in the United

Others coming/out there

RESEARCH Open Access

The relationship between target joints and direct resource use in severe haemophilia

Jamie O'Hara[1], Shaun Walsh[2], Charlotte Camp[2*], Giuseppe Mazza[3], Liz Carroll[4], Christina Hoxer[5] and Lars Wilkinson[5]

Abstract

Objectives: Target joints are a common complication of severe haemophilia. While factor replacement therapy constitutes the majority of costs in haemophilia, the relationship between target joints and non drug-related direct costs (NDDCs) has not been studied.

Methods: Data on haemophilia patients without inhibitors was drawn from the 'Cost of Haemophilia across Europe – a Socioeconomic Survey' (CHESS) study, a cost assessment in severe haemophilia A and B across five European countries (France, Germany, Italy, Spain, and the United Kingdom) in which 139 haemophilia specialists provided demographic and clinical information for 1285 adult patients. NDDCs were calculated using publicly available cost data, including 12-month ambulatory and secondary care activity: haematologist and other specialist consultant consultations, medical tests and examinations, bleed-related hospital admissions, and payments to professional care providers. A generalized linear model was developed to investigate the relationship between NDDCs and target joints (areas of chronic synovitis), adjusted for patient covariates.

Results: Five hundred and thirteen patients (42% of the sample) had no diagnosed target joints; a total of 1376 target joints (range 1–10) were recorded in the remaining 714 patients. Mean adjusted NDDCs for persons with no

Others in pipeline…

Manuscripts in development/submission

- Target joints/HRQoL (accepted; Health and Quality of Life Outcomes)
- Target joints/Cost (Published)
- Unmet need
- Aging with haemophilia (under sponsor review)
- Impact of prior inhibitor (in development)
- Burden of Inhibitors (2nd review; Haemophilia)
- More to do: primary prophylaxis in haemophilia (2nd draft)
- A vs B (accepted; Hemophilia)
- Cost of Bleeding (in development)

Policy influencing…

Commissioners using data to support development of outcomes based frameworks;

- UK (NHSE & NICE)
- AUS (NBA)
- US (ICER)

Several European universities using data to support government funded research into the condition

- Copenhagen, Sheffield, Galway and Brussels

EHC and UKHS utilising data to support advocacy initiatives

UK governmental support to develop contaminated blood policy

Advocacy Surgery at the EHC in October

EHC response issued to IQWIG at new technologies workshop and to be presented at ISPOR this year

Congress presentations…

Congresses where data has been presented

- HTAI
  - Approach and importance of patient experience
  - Depression
- ISPOR
  - Implications of TJ's on;
    - HRQoL
    - Cost
  - IQWIG methodology
- WFH
  - ABRs
  - Costs
  - etc
- EHC
  - Anxiety & depression
- EAHAD
  - Adherance
  - Aging
- ISTH
- Advocacy summit's