## The Relationship between Societal Costs Associated with Haemophilia and Disease Severity: A Regression Analysis Using CHESS II Data

<u>T. Burke</u><sup>1</sup>, A. Shaikh<sup>1</sup>, J. O'Hara<sup>1,2</sup>, N. Misciattelli<sup>3</sup>, S. Kar<sup>3</sup>, M. Blanco<sup>3</sup>, A. Nathwani<sup>3,4</sup>

<sup>1</sup>HCD Economics, Daresbury, United Kingdom, <sup>2</sup>University of Chester, Faculty of Health and Social Care, Chester, United Kingdom, <sup>3</sup>Freeline Therapeutics, London, United Kingdom, <sup>4</sup>UCL, Institute of Immunity and Transplantation, London, United Kingdom

Abstract Number: PB0848

Meeting: ISTH 2020 Congress

Theme: Hemophilia and Rare Bleeding Disorders » Hemophilia - Basic

**Background:** Haemophilia (deficiency or absence of clotting factor) is a genetic bleeding disorder characterised by prolonged trauma-related or spontaneous bleeding events and associated complications. Haemophilia has been found to pose an economic burden on patients, caregivers, and the wider health care system. There is limited research, on the wider (non-drug related) costs of haemophilia and levels of severity, specifically across different levels within the mild range.

**Aims:** To assess the relationship between societal costs and severity levels, using interim realworld data from the 'Cost of Haemophilia in Europe: a Socioeconomic Survey – II' study (CHESS II).

**Methods:** Data were extracted from the CHESS II interim dataset, a societal perspective, prevalence-based study in mild, moderate, and severe adult haemophilia A and B patients across France, Germany, Italy, Spain, UK, Romania, Netherlands, and Denmark (n=787). Disease severity was categorised according to baseline endogenous factor VIII/IX activity (IU/dl or %). 304 patients provided societal cost data. Mild haemophilia was subcategorized by endogenous factor VIII/IX expression (>5-20%, n=14; >20-40%, n=25). Societal costs consisted of healthcare system costs (excluding costs of treatment) and patient-centric costs, such as caregiver hours and time missed from work. A generalized linear model (GLM) was developed to investigate variation in societal costs across severity groups, adjusting for covariates age, BMI and country.

**Results:** The GLM model provided adequate fit, the average marginal effect at the mean was calculated from regression outputs. Analysis found that when compared to moderate and severe patients (0-5%, n=265), subgroups of mild haemophilia '>5-20%' and '>20-40%' were €7,170 (p=0.058) and €13,111 (p< 0.05) less costly, respectively. This analysis controlled for age, BMI and country effects.

**Conclusions:** This analysis suggests that a higher endogenous factor VIII/IX expression (>20-40%) may have a significant impact on reducing total per-patient cost from the societal perspective, accounting for age, BMI and country effects.

To cite this abstract in AMA style:

Burke T, Shaikh A, O'Hara J, Misciattelli N, Kar S, Blanco M, Nathwani A. The Relationship between Societal Costs Associated with Haemophilia and Disease Severity: A Regression Analysis Using CHESS II Data [abstract]. *Res Pract Thromb Haemost*. 2020; 4 (Suppl 1). https://abstracts.isth.org/abstract/the-relationship-between-societal-costs-associated-withhaemophilia-and-disease-severity-a-regression-analysis-using-chess-ii-data/. Accessed July 2, 2020.

**ISTH Congress Abstracts** - https://abstracts.isth.org/abstract/the-relationship-betweensocietal-costs-associated-with-haemophilia-and-disease-severity-a-regression-analysis-usingchess-ii-data/