

A NEW METHODOLOGY TO ASSESS UTILITY IN HAEMOPHILIA USING QUALITY OF LIFE MEASURES IN PARENTS AND CAREGIVERS: THE CAREGIVERS' BURDEN STUDY



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INTRODUCTION

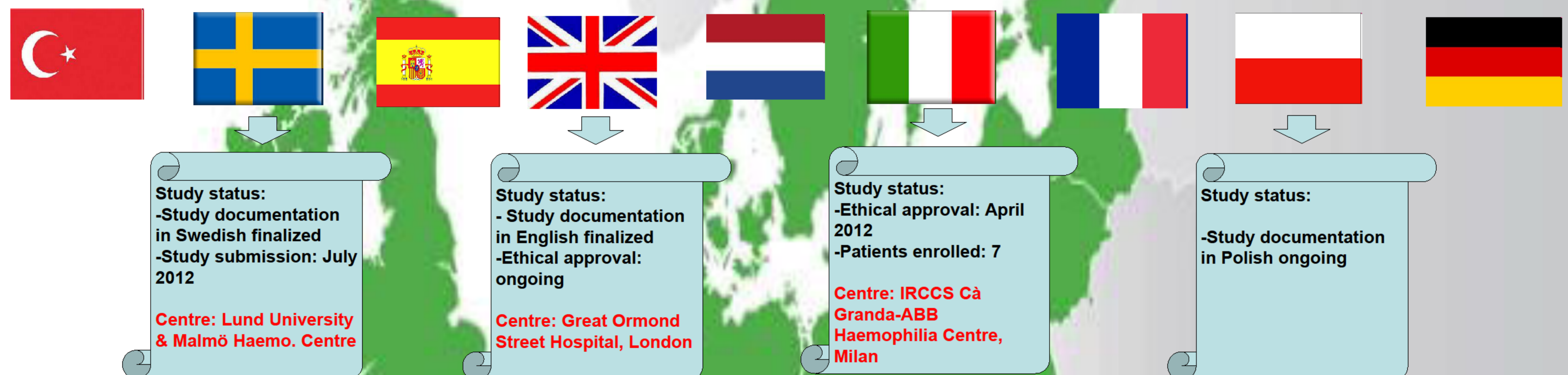
Utilities of different treatment approaches are commonly measured in patients either children or adults who undergo these treatments. Recently, HTA institutions and some reimbursement authorities take in account utilities as perceived by patients' caregivers, particularly when patients are children and/or severely handicapped. No data are available on utilities of caregivers of children with haemophilia. In order to evaluate utility as perceived by patients' caregivers and in particular if primary prophylaxis might have an impact, we designed a prospective study that compares utility in caregivers of children on prophylaxis to those on episodic treatment. The study is also assessing whether patients' quality of life can explain potential differences in caregivers' utilities.

AIM & METHODS

The aim is to evaluate and to measure caregivers' burden in such a way that it can be included in economic modelling for haemophilia showing the value of the treatment and the benefit not only to the patients but also to the caregivers of prophylaxis and on-demand treatment.

This is a pilot, non interventional, multicentre study in at least 100 European patients. Enrolment criteria include patients with severe, moderate or mild haemophilia A, with or without inhibitor, or history of inhibitor, age from 8 to 17 years old and with at least 1 caregiver. Children are given generic (EQ-5D-Y) and haemophilia-specific questionnaires (Haemo-QoL). In addition EQ-5D, SF-36 and Impact of Family Scale (IOF) will be administered to the caregivers.

COUNTRIES INVOLVED



Generic Measures

Disease-Specific Measures

EQ-5D is a generic standardised instrument for use as a measure of health outcome. Applicable to a wide range of health conditions and treatments, it provides a simple descriptive profile and a single index value for health status. EQ-5D is designed for self-completion by respondents. It is cognitively simple, designed with 5 questions. Instructions to respondents are included in the questionnaire. EQ-5D-Y is the version for children and adolescents aged from 7-12 years.

Haemo-QoL is the first haemophilia-specific HRQOL questionnaire and it is available in three age group versions (I: 4-7 years, II: 8-12 years, III: 13-16 years) as self reports for children as well as three proxy versions for parents report respectively.
 > It consists of 8 to 12 dimensions according to different age groups
 > Domains: 'physical health', 'feeling', 'view', 'family', 'friends', 'others', 'sport and school' and 'treatment'. Age groups II and III have in addition the domains 'perceived support' and 'dealing with haemophilia' and for adolescents the domains 'future' and 'relationship' are added.

SF-36 is a multi-purpose, short-form health survey with 36 questions. It yields an 8-scale profile of functional health and well-being scores as well as psychometrically-based physical and mental health summary measures and a preference-based health utility index. It is a generic measure, as opposed to one that targets a specific age, disease, or treatment group. Accordingly, the SF-36 has proven useful in surveys of general and specific populations, comparing the relative burden of diseases, and in differentiating the health benefits produced by a wide range of different treatments.

Impact on Family Scale (IOF) is a questionnaire concerning the impact of a chronic childhood disease on the family. The questionnaire consists of 27 questions (33 if the family has other children). The Impact-on-Family Scale has four dimensions of impact. Financial Burden refers to the economic consequences for the family. Familial/Social impact concerns the disruption of social interaction. Personal Strain assesses the psychological burden experienced by the primary caretaker. Mastery refers to the coping strategies employed by the family.

CONCLUSION & DISCUSSION

This study will give an important contribute to understand better the value of utility measure in haemophilia patients based on the combination between patients and caregivers outcomes permitting a better evaluation of patients' wellbeing and satisfaction and a better evaluation of prophylaxis and on-demand treatment in such a way that it can be included in economic modeling for haemophilia.

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