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Outcomes measures in patients with haemophilia: Survey of implementation in routine clinical practice and perception of relevance by European treaters of the EHTSB

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Introduction and Objectives

The development and availability of clotting factors concentrates have changed the care of persons with haemophilia (PWH), allowing replacement of the missing coagulation factors in order to treat, or ideally, prevent, the many adverse consequences of haemophilia in the short-term, or ideally, the long-term. Outcome measurements in PWH have long been limited to laboratory evaluation (quantification of circulating factor levels) and clinical outcomes (such as mortality, bleeding frequency, site severity, development of arthropathy) that do not include important aspects of patient care. Because of new standards of care, there is a clear need to consider additional outcome measures, such as the early detection and quantification of joint disease (by physical and functional criteria or imaging techniques), the health-related quality of life and economic analyses. The improvement of outcome measures is increasingly becoming crucial in defining a clear goal for therapy, determining the most appropriate treatment strategies - taking into account economical constraints - and justifying the cost related to the treatment, as well as allowing more objective comparisons of care between centres and countries[1]. These outcome measures are important on both an individual patient as well as a population basis (**Figure 1**). Several outcomes measures have been proposed over the last decades, some defining many different parameters that need to be collected. Some are either complex or time-consuming to perform, or are very expensive (imaging techniques). Not all of them have been extensively validated

Material & Methods

In order to evaluate the current implementation of outcome measures in routine clinical haemophilia practice and to appreciate the perception of their relevance by treaters, a survey was recently undertaken involving 19 of the 26 physicians who comprise the European Haemophilia Therapy Standardisation Board (EHTSB). Employing an extensive inventory of outcomes measures used in patients with haemophilia, information was collected about the subjective appreciation of the importance (from not important=1 to critical=5) and the frequency of outcome data collection during clinic review. Several selected parameters were investigated in greater detail, especially use of particular assessment tools.

Results

A literature search and the Board members' clinical experience identified 13 outcome measures that were commonly used and were worth exploring further (**Table 1**). The survey revealed that the data most commonly collected by the majority of treaters focus on parameters of haemostatic treatment: consumption of concentrates, number of major joint and muscle bleeds. These data were collected once per year or more frequently by 86%, 81% and 76% of treaters respectively. However, the data collection tended to be non-uniform and non-standardised.

By contrast, functional, physical and quality of life scorings are used less frequently and considerable heterogeneity was shown between treaters. The use of selected parameters considering musculoskeletal assessment, radiological assessment, pain, social participation, activities and quality of life are summarised in **Figure 2.**

These data show that after haemostatic treatment, musculoskeletal health (function and radiological assessment) and pain are the main focus of assessment, while less attention was paid to social participation, activities and quality of life. A lack of time was cited most often as the reason for not assessing these parameters, possibly reflecting the priority of these parameters in busy everyday clinical practice.

The way in which these measurements inform patient treatment is summarized in **Figure 3**. The survey showed that while musculoskeletal health data are collected and used to inform treatment this is not the case for parameters such as pain, activities, social functioning and quality of life that do not impact treatment strategies to the same extent. Specifically, information on pain is collected by more than 50% of treaters, but influences treatment in less than 10% of cases. Activities only sometimes inform treatment and social participation and quality of life influence practice in less than 25% of cases, even though 10-45% of treaters collect these data. Additionally, when asked to rate the relevance of these three parameters to treatment the physicians rated them as 4 on a scale of 1-5.

Table 1: Outcome parameters collected in daily practice

Outcome parameter	Data collected	
Joint bleeds	Number per year	Number of major bleeds**
Target joints*	Number of target joints	
Muscle bleeds	Number per year	Number of major bleeds**
Life-threatening bleeds	Intracranial	Other
Arthropathy	Joint score (clinical) Range of Motion Joint score (imaging)	X-ray Ultrasound scan MRI
Pain	Use of painkiller Use of anti-inflammatory agents	Presence of chronic pain*** Pain scale
Clotting factor consumption	Number of units per year (prophylaxis) Number of units per year (on demand)	Units/year/Kg body weight Level of adherence
Physical activities	Questionnaire	
Social participation	Questionnaire	
Days lost from school/work	Number	
Use of medical resources	Number of annual visits Number of days/year in hospital	Medical costs (beyond replacement)
Quality of life	Generic questionnaire	Disease-specific questionnaire
Global satisfaction	Questionnaire	Numeric/ visual analogue scale

* Target joints+ = 3 bleeds in the same joint in a 6 month period ** major bleeds = bleed requiring a visit or hospitalisation *** chronic pain= continuous or intermittent pain, related to the pathophysiology of haemophilia, requiring intervention in which the cause of pain cannot readily be removed, occurring more than one a week and lasting 3 months or more

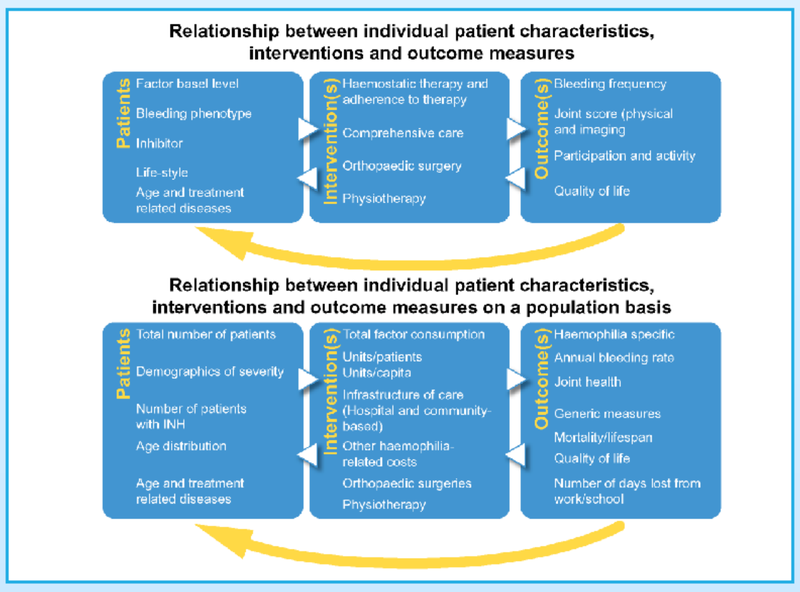

Figure 1: Relationship between patient characteristics, interventions and outcome on an individual patient and a population basis

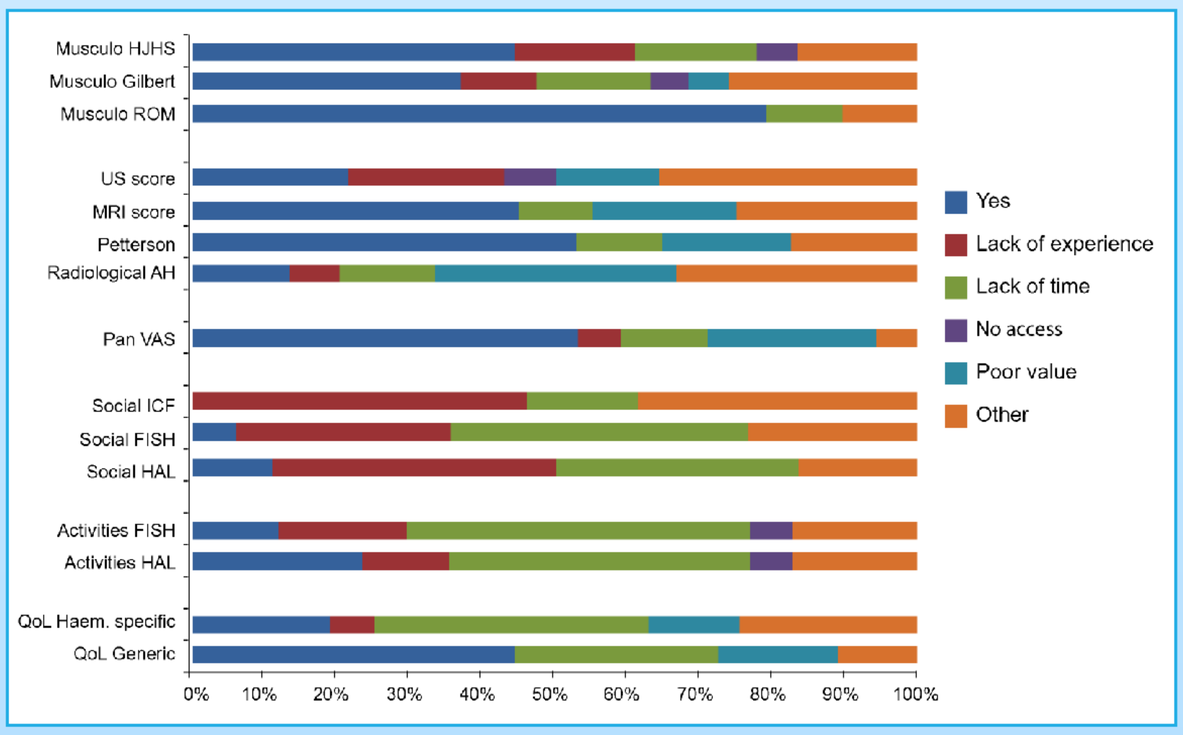

Figure 2. Summary of outcomes tools used on a regular basis by participating physicians. Tools are rated 'yes" or 'no' for the reasons given. HJHS=Haemophilia Joint Health Score; Gilbert=Gilbert Score (WFH); ROM= Range of Motion; US = Ultrasound; MRI= Magnetic Resonance Imagining; AH= Arnold-Hilgartner system; VAS= Visual Analogue Scale; ICF=International Classification of Functioning, Disability and Health; FISH= Functional Independence Score in Haemophilia; HAL = Haemophilia Activity List; QoL = Quality of Life.

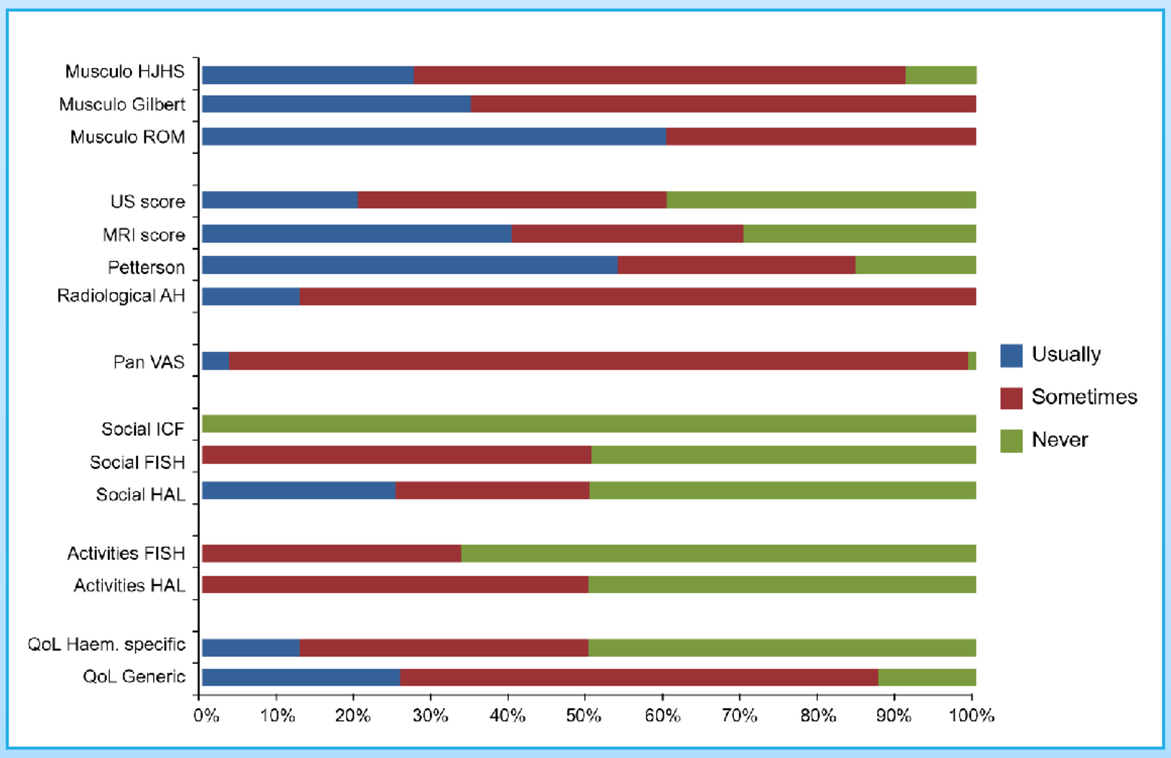

Figure 3. Summary of how outcomes inform patient management. HJHS=Haemophilia Joint Health Score; Gilbert=Gilbert Score (WFH); ROM= Range of Motion; US = Ultrasound; MRI= Magnetic Resonance Imagining; AH= Arnold-Hilgartner system; VAS= Visual Analogue Scale; ICF=International Classification of Functioning, Disability and Health; FISH= Functional Independence Score in Haemophilia; HAL = Haemophilia Activity List; QoL = Quality of Life.

Conclusion

In conclusion, this survey represents in our view the first attempt to evaluate the real-life practice of outcomes measures in haemophilia care. The survey clearly highlights the need for further validation of several outcome measures and the importance of developing standardised and user-friendly tools to improve implementation of outcomes in clinical practice.

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Reference:

1. Aledort L, Bullinger M, von Mackensen S, *et al.* Why should we care about quality of life in persons with haemophilia? *Haemophilia* (2012), 18, e154–e157. Correspondence: cedric.hermans@uclouvain.be

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