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ASSESSING QUALITY OF CARE IN HAEMOPHILIA USING A VALUE-BASED HEALTHCARE APPROACH.

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OBJECTIVES

- ✓ Advances in **haemophilia management** and treatment improved disease management and patients' quality of life with an increasing economic burden for healthcare systems.
- ✓ The **"Value-Based Healthcare in Haemophilia" (VBH2)** project aimed to define a set of **clinical and patient reported healthcare outcome indicators** to assess quality of care to inform and guide decision-making processes.

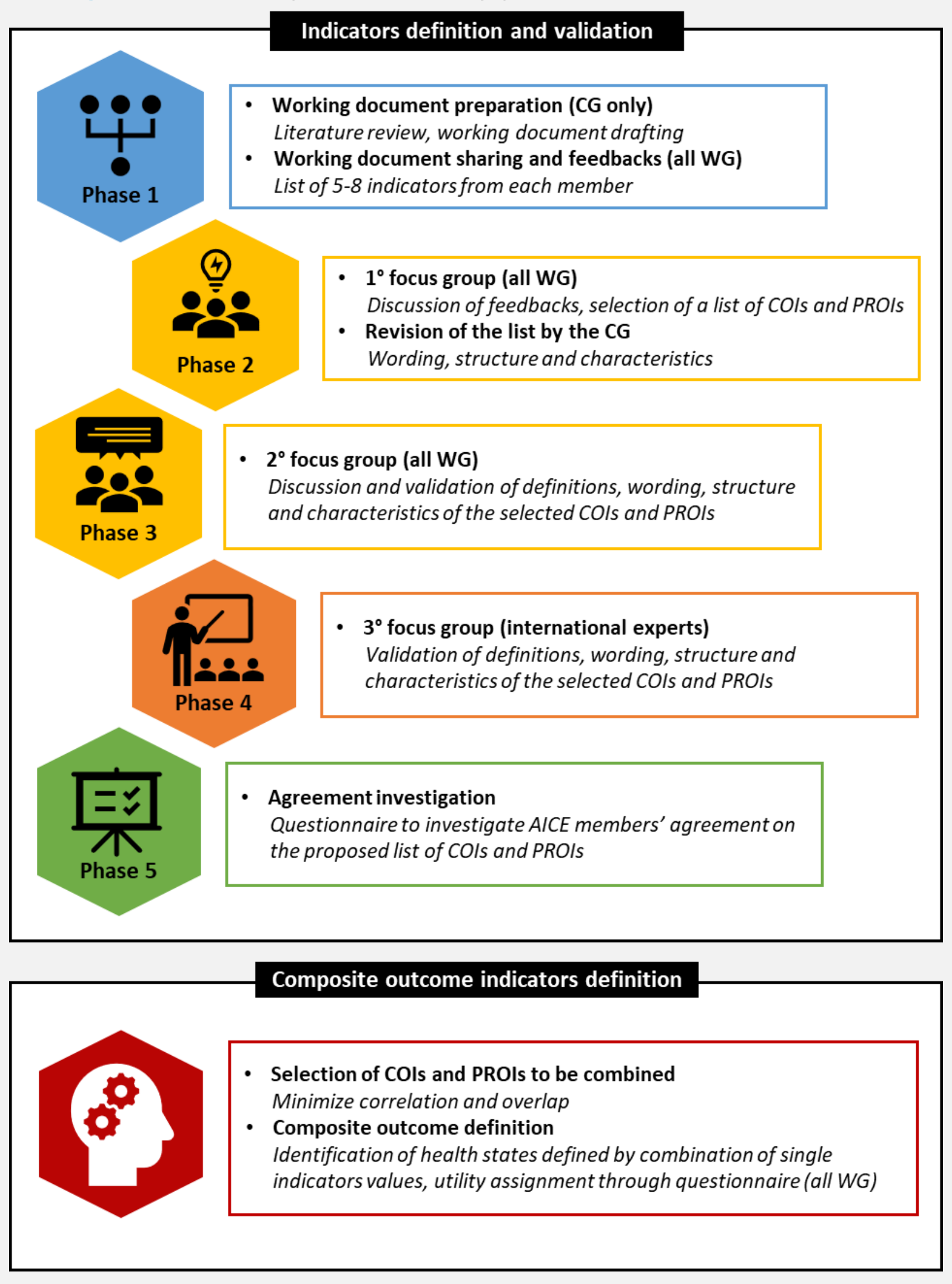
METHODS

- ✓ The study adopted a **value-based healthcare approach** to generated a set of **healthcare outcome indicators** to evaluate quality of care in haemophilia. We used a **DELPHI panel consensus method** based on available literature, current guidelines and the involvement of multidisciplinary groups of experts.

Coordinating Group (CG)	5 experts in outcome research and value-based healthcare
Working Group (WG)	17 experts in the field of haemophilia (7 hematologists, 1 rheumatologist, 1 orthopedic, 1 radiologist, 1 nurse, 1 physiotherapist, 1 psychologist, 1 biostatistician and 3 patients)

- ✓ The set of indicators included both clinical and patient-reported outcomes as separate dimensions: **COIs (Clinical Outcome Indicators) and PROIs (patient-reported outcomes Indicators)**. We also defined **two composite outcome indicators** to synthesize the clinical and the patient-reported dimensions.
- ✓ **The single COIs and PROIs identification and validation** was composed of 5 phases described in *Figure 1.* The Agreement investigation (Phase 5) on each COI and PROI used a standard RAND/UCLA 9-point agreement scale, ranging from 1 ("extremely inappropriate") to 9 ("extremely appropriate"). The level of agreement was quantified as median panel rating (MPR) and RAND "Disagreement Index" (DI) . The DI is a validated measure of variation in provider beliefs with lower values indicating increasing consensus. A COI or PROI was considered an appropriate measure of quality of care when it had 7≤MPR≤9 without any disagreement (DI <1.0)

Figure 1. Description of study phases



Indicators definition and validation

Phase 1
- **Working document preparation (CG only)**
  Literature review, working document drafting
- **Working document sharing and feedbacks (all WG)**
  List of 5-8 indicators from each member

Phase 2
- **1° focus group (all WG)**
  Discussion of feedbacks, selection of a list of COIs and PROIs
- **Revision of the list by the CG**
  Wording, structure and characteristics

Phase 3
- **2° focus group (all WG)**
  Discussion and validation of definitions, wording, structure and characteristics of the selected COIs and PROIs

Phase 4
- **3° focus group (international experts)**
  Validation of definitions, wording, structure and characteristics of the selected COIs and PROIs

Phase 5
- **Agreement investigation**
  Questionnaire to investigate AICE members' agreement on the proposed list of COIs and PROIs

Composite outcome indicators definition

- **Selection of COIs and PROIs to be combined**
  Minimize correlation and overlap
- **Composite outcome definition**
  Identification of health states defined by combination of single indicators values, utility assignment through questionnaire (all WG)

CG = Coordinating Group, WG = Working Group, COI = Clinical Outcome Indicator, PROI = Patient Reported Outcome Indicator, AICE = Associazione Italiana dei Centri di Emofilia

- ✓ **The composite outcome indicator definition** involved a restricted number of WG experts and the CG. They re-assessed characteristics of each single COI and PROI to propose a combination of them in a composite one, separately for clinical and patient reported outcomes. Each indicator was then categorized in two or three ordinal levels, that were combined to describe possible health states of haemophilia patients. A utility value was then assigned to each health state (0=worst health state, 10=best health state) based on the opinion of all the WG members. The restricted WG established that utilities should be defined by age groups: 6-13, 14-35, 36-65, >65 years. The age class 0-5 years was excluded because of issues related to outcome measurement in this age-class.

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RESULTS

SINGLE OUTCOME INDICATORS DEFINITION AND VALIDATION

Table 1. Clinical outcomes indicators (COIs) identified and approved.

Domain	Outcome	Definition	Tools	MPR	DI
Musculoskeletal (MSK) health and function	MSK health	The ability to walk and fulfil routine tasks	6MWT; HJHS v2.1	8	0.56
	Joint level: Disease activity and Osteochondral damage	The presence of chronic synovial proliferation, as an indicator of recurrent bleeding, and the presence of articular cartilage or subchondral bone abnormalities. Chronic synovial proliferation recognizes indirect signs of recurrent bleeding by detection of chronic synovial proliferation in the joint recesses. Articular cartilage or subchondral bone abnormalities identifies direct signs of joint derangement by assessing the articular cartilage and the subchondral bone abnormalities on a reference surface (one per joint).	HEAD-US score	8	0.29
Safety	Treatment Safety	The safe use of medicines to ensure that the benefit-risk ratio of each drug is favorable. Any serious reported health complication, caused by treatment: inhibitor development and treatment-related infections, thromboembolic complications, difficult venous access, thrombosis, or obstruction of central venous access devices.	Monitoring and evaluation of safety data collected with medical and nursing records. Pharmacovigilance reports.	9	0.29

6MWT: 6 Minute Walking test. HJHS: Hemophilia Joint Health Score. HEAD-US: Haemophilia Early Arthropathy Detection with Ultrasound. MPR: Median panel rating. DI: Disagreement Index.

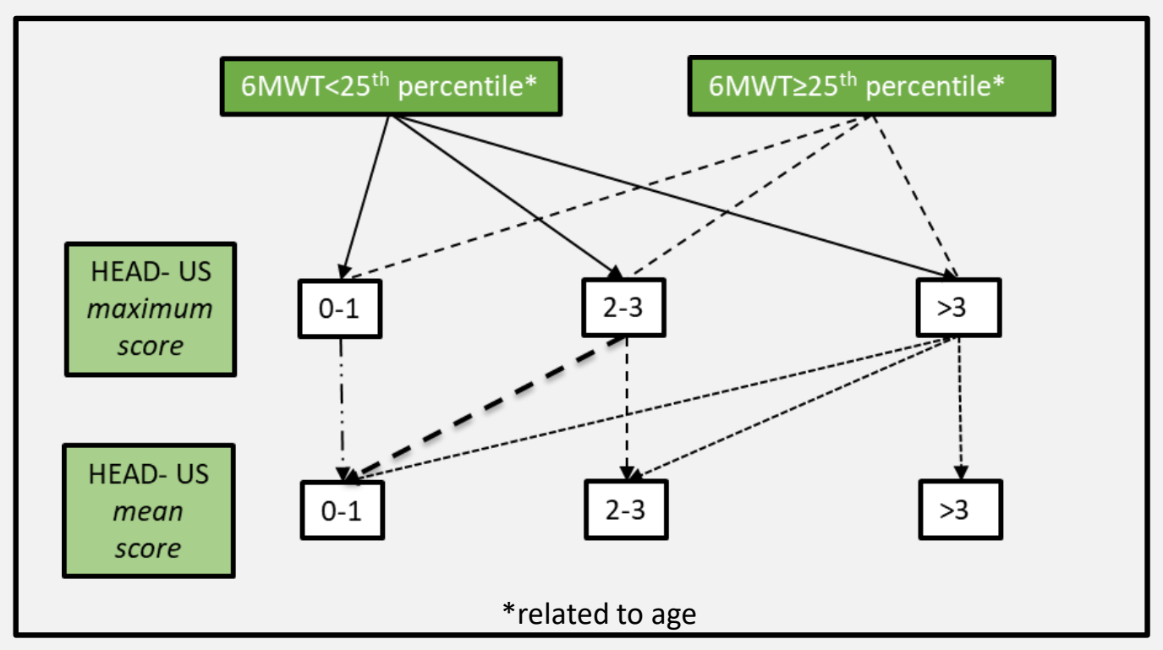
Table 2. Patient reported outcomes indicators (PROIs) identified and approved.

Domain	Outcome	Definition	Tools	MPR	DI
Bleeding frequency / phenotype	Bleeding frequency	Number of bleeding episodes as perceived by persons with haemophilia over a time period.	Patient Bleed Diaries	8	0.53
Pain	Pain severity and the associated impact on daily activities	Severity of persistent pain, number of acute painful events and the associated limits to everyday life activities.	BPI v2 Short Form	8	0.29
Mobility and Physical activities	Functional independence	Functional autonomy in everyday life and barriers imposed by the disease and its treatment.	HAL Adult; PedHAL Pediatric; EQ-5D-5L*	8	0.29
Quality of life	Health Related Quality of Life (HRQoL)	Quality of Life is individuals' perception of their position in life in the context of the culture and value systems in which they live and in relation to their goals, expectations, standards, concerns. Quality of life is an all-inclusive concept incorporating all factors that impact upon an individual's life; while HRQoL focuses on physical, psychological, functional, and social domains related to a person's perception of quality of life affected by health status.	EQ-5D-5L questionnaire	8	0.29
Satisfaction	Treatment Satisfaction	The degree to which the patients perceive how the care process and the treatment regimen fulfill their health needs, expectations, and preferences.	Hemo-Sat	8	0.56

BPI: Brief Pain Inventory. HAL: Haemophilia Activities List. PedHAL:Paediatric Haemophilia Activities List. EQ-5D-5L: EuroQol-5 Dimension-5level version. Hemo-Sat: Hemophilia Patient Satisfaction.*(mobility, usual activities, and physical activities domains)

COMPOSITE OUTCOME DEFINITION

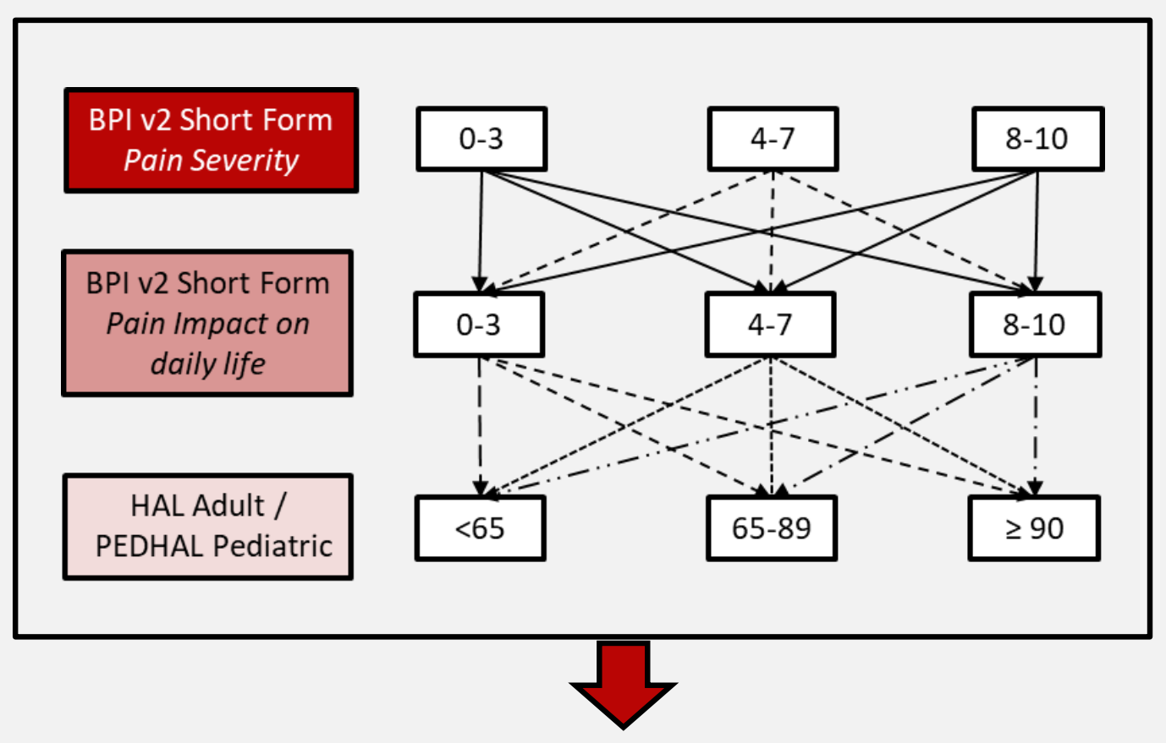
Figure 2. Composite Clinical Outcome Indicator (COI). Definition of possible health states.



Health state ID	6MWT	HEAD-US		Utilities by age groups (years)			
	Distance (meter)	max score	mean score	6-13	14-35	36-65	>65
1	≥ 25th p	0-1	0-1	10	10	10	10
2	≥ 25th p	2-3	0-1	5	6	7	8
3	≥ 25th p	2-3	2-3	4	5	6	7
4	≥ 25th p	>3	0-1	4	5	7	8
5	≥ 25th p	>3	2-3	3	4	6	7
6	≥ 25th p	>3	>3	2	3	4	6
7	< 25th p	0-1	0-1	4	4	6	7
8	< 25th p	2-3	0-1	3	4	5	6
9	< 25th p	2-3	2-3	1.5	3.5	5	6
10	< 25th p	>3	0-1	3	4	5	6
11	< 25th p	>3	2-3	1	2	4	6
12	< 25th p	>3	>3	0	0	0	0

The health state number 1 represents the best patient's conditions, while number 12 the worst ones. 6MWT: 6 Minute Walking test. HJHS: Hemophilia Joint Health Score. HEAD-US: Haemophilia Early Arthropathy Detection with Ultrasound. p=percentile

Figure 3. Composite Patient Reported Outcome Indicator (PROI). Definition of possible health states.



Health state ID	BPI v2 Short Form		HAL/PedHAL	Utilities by age groups (years)			
	pain severity[1]	pain impact[2]	normalized score	6-13	14-35	36-65	>65
1	0-3	0-3	>90	10	10	10	10
2	0-3	0-3	65-90	7	7	7	8
3	0-3	0-3	<65	5	6	6	6
4	0-3	4-7	>90	6	7	7	8
5	0-3	4-7	65-90	5	5	6	7
6	0-3	4-7	<65	5	5	5	6
7	0-3	8-10	>90	5	6	6	7
8	0-3	8-10	65-90	4	5	5	6
9	0-3	8-10	<65	4	5	5	5
10	4-7	0-3	>90	6	7	7	8
11	4-7	0-3	65-90	5	6	6	6
12	4-7	0-3	<65	5	5	5	6
13	4-7	4-7	>90	5	6	6	7
14	4-7	4-7	65-90	4	5	5.5	6
15	4-7	4-7	<65	4	5	5	5
16	4-7	8-10	>90	4	5	5	6
17	4-7	8-10	65-90	3	4	5	5
18	4-7	8-10	<65	3	3	4	5
19	8-10	0-3	>90	4	5	5	5
20	8-10	0-3	65-90	2	4	5	5
21	8-10	0-3	<65	2	3	3	5
22	8-10	4-7	>90	3	4	5	6
23	8-10	4-7	65-90	2	4	5	5
24	8-10	4-7	<65	1	2	3	4
25	8-10	8-10	>90	2	3	4	5
26	8-10	8-10	65-90	1	2	3	4
27	8-10	8-10	<65	0	0	0	0

1 Mean score of the first 4 questions. 2Means score of questions 5-11. The health state number 1 represents the best patient's conditions, while number 27 the worst ones. BPI: Brief Pain Inventory. HAL: Haemophilia Activities List. PedHAL: Paediatric Haemophilia Activities List.

CONCLUSIONS

- ✓ The presented set of health outcome indicators provides the basis for harmonized longitudinal and cross-sectional monitoring and comparison.
- ✓ The implementation of this value-based approach would enable a more robust assessment of quality of care in haemophilia,
- ✓ Proposed COIS and PROIS should be reviewed and updated routinely.

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