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REAL WORLD EVALUATION OF A NOVEL PATIENT CENTERED OUTCOME INDICATOR TO SUPPORT VALUE BASED HEALTHCARE DECISION-MAKING IN HEMOPHILIA A IN ITALY: THE HEMPROVE NON-INTERVENTIONAL STUDY

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Background and Aims. Existing outcome and care quality indicators for haemophilia A have limitations in capturing the impact of the evolving treatment landscape, highlighting the need for novel clinician- and patient-reported outcome indicators (PROIs). By reflecting health status from the patient's perspective, PROIs provide essential insights that support value-based healthcare and data-driven decision-making. Within this context, the VBH2 project introduced a set of composite indicators to assess care value in haemophilia, including a composite PROI integrating the Brief Pain Inventory (BPI) Short Form, which assesses pain severity and its impact on daily life, and the Haemophilia Activities List (HAL)/Pediatric HAL (PedHAL), which evaluate mobility and physical activity in adults and children/teenagers. The HemPROve study aims to assess the composite PROI in a real-world setting, by describing the distribution of the utility and relative scores among Italian patients with severe haemophilia A and by exploring its association with clinical characteristics.

Methods. HemPROve is a non-interventional, multicenter, cross-sectional study enrolling patients aged ≥6 years with severe haemophilia A (FVIII <1%), without inhibitors, and receiving prophylactic treatment according to routine clinical practice. Patients were consecutively enrolled across 13 Italian haemophilia centers. Data collection started in March 2025 and was completed in December 2025. Final study results are expected by July 2026. All data were collected at a single time point, including administration of the BPI and HAL/PedHAL instruments. The composite PROI will be calculated in accordance with the developers' instructions, assigning an age-specific utility score to the relevant health state identifier, derived from the combined levels of the constituent PRO domains [Table 1]. In parallel, potential modifiers will be explored among patients' collected socio-demographic and clinical characteristics, including: age at enrolment; educational level; comorbidities requiring pharmacological treatment; Haemophilia Joint Health Score (HJHS); type of prophylaxis administered at enrolment (i.e., FVIII standard half-life, FVIII prolonged half-life, non-replacement therapies), with relative treatment administration frequency and duration up to enrolment; and the number of treated bleeds in the last 24 weeks prior to enrolment.

Results. A total of 128 patients have been enrolled across the following age classes (years): 6-13, n=31; 14-35, n=42; 36-65, n=44; >65, n=11. The study is expected to provide valuable insights into patients' health-state levels and utility scores as defined by the composite PROI, to explore its applicability in haemophilia A, and to identify factors associated with variability in PROI scores.

Conclusions. Implementation of the new PROI provides a patient-centered framework that supports clinical decision-making and optimizes achievable outcomes in haemophilia A. HemPROve is funded by Roche S.p.A. Italy.

Table 1. Composite Patient-Reported Outcome Indicator (PROI): health states and utilities associated

Health state ID	BPI Short Form		HAL/PedHAL	Utilities by age groups (years)			
	Pain Severity (mean score level)	Pain impact on daily life (mean score level)	(normalized score level)	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

Source: Cortesi PA et al., Haemophilia. 2024 Mar;30(2):437-48. doi: 10.1111/hae.14943.
The Health state ID 1 represents the best patient's conditions, while ID 27 the worst ones. A higher Utility score represents a more desirable health state from a societal perspective.
BPI: Brief Pain Inventory (Copyright 1991 Charles S. Cleeland, PhD. Pain Research Group).
HAL: Haemophilia Activities List (Copyright Van Genderen et al., 2005, UMC Utrecht). PedHAL: Paediatric Haemophilia Activities List Parents' and Children's/Teenagers' versions (Copyright Van Creveldkliniek, University Medical Center Utrecht).

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