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The GOAL-Hēm journey: Shared decision making and patient-centred outcomes

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Abstract

Introduction: GOAL-Hēm is a novel, haemophilia-specific, patient-centred outcome measure (PCOM) based on goal attainment scaling, allowing people with haemophilia (PwH) to set and monitor the attainment of individualized goals for treatment.

Aim: To provide a thorough overview of the creation, validation, and development of GOAL-Hēm.

Methods: Clinician workshops were held to develop a haemophilia-specific goal menu. Qualitative data from semistructured interviews with PwH and their caregivers guided further revisions to the goal menu (i.e., goal domains and descriptors). A feasibility study was performed including a 12-week, prospective, noninterventional evaluation involving clinicians and PwH at four US haemophilia treatment centres. Finally, the Patient Voice Study gathered feedback from PwH and their caregivers via an online survey, interviews, and a focus group.

Results: The feasibility study validated GOAL-Hēm with successful outcomes in construct/content validity and responsiveness, including a large effect in patient- and clinician-rated goal attainments. The Patient Voice Study led to significant refinement of GOAL-Hēm goals and descriptors, resulting in a more straightforward and relatable menu for PwH and their caregivers. Overall, GOAL-Hēm captured qualitative data in areas important to PwH and employed quantitative methods to evaluate meaningful changes in those areas. The individualized tool was well equipped to handle the complex and chronic nature of haemophilia and was endorsed by PwH, their caregivers, and clinicians.

Conclusion: The GOAL-Hēm development journey may serve as a roadmap for other PCOMs in a variety of settings, including clinical studies, haemophilia treatment centres for care planning, and as a tool to gather real-world evidence.

KEYWORDS
goal attainment scaling, goal setting, haemophilia, individualized, patient-centred outcome measure

1 | INTRODUCTION

The fundamental goal of haemophilia treatment is to reduce bleeding and its complications; clinical research in haemophilia is based on clinically assessed outcome measures such as annualized bleed rate (ABR), joint health scores, and quality of life (QoL).[1] Even with advances in prophylactic treatment regimens leading to lower ABRs, individualized approaches are needed to provide clinically meaningful results relative to each patient's personal experience.[2] The evaluation of clinical meaningfulness requires considering whether the effects of treatment meet the needs and aspirations of people with haemophilia (PwH), their caregivers, and their physicians.[3]

Goal attainment scaling (GAS), a patient-centred outcome measure (PCOM),[4] allows both clinicians and people affected by acute or chronic medical conditions to identify and track meaningful treatment goals. GAS employs a 5-point scale from −2 to +2 ('much worse than expected' to 'much better than expected', respectively).[1,4] It was originally developed for use in community health,[5] and later more widely adopted in geriatric and rehabilitation medicine.[6] GAS is also amenable for similar use in various chronic diseases.[7] In 2015, a group of haemophilia and GAS experts convened to apply it to haemophilia via GAS-Hēm (Goal Attainment Scaling for Haemophilia).[8] Unlike ABR, the personalization of GAS in haemophilia allowed detection of small, yet clinically meaningful changes in outcomes prespecified as important to PwH.[1] GAS also offered the opportunity to assess whether and to what extent a particular goal was met.[1,6]

GAS-Hēm, renamed GOAL-Hēm, was validated in a 12-week, prospective feasibility study.[4] In the clinical setting, goals for this study were classified into three domains: *managing haemophilia*, *haemophilia complications*, and *impact on life*. PwH addressed a prespecified goal or selected a goal of their own during collaboration with their healthcare professional(s), in addition to creating a 5-point scale to measure change. The Patient Voice Study further refined the GOAL-Hēm menu to be more related to the broader haemophilia community.[9] These developments gave rise to a PCOM capturing both qualitative data (i.e., preferences of PwH and their goals) and quantitative data (i.e., impact of treatment on the goals) which is well equipped to handle the complex and chronic nature of haemophilia.

We report the 4-year journey to reach the current iteration of the GOAL-Hēm tool. The main steps included: initial clinician workshops, qualitative assessments from PwH and their caregivers, feasibility study, and final refinement with the Patient Voice Study. This process may represent a paradigm for the development of other PCOMs in haemophilia and other bleeding disorders or chronic medical conditions.

2 | STUDY DESIGN

2.1 | Four-year development timeline

Figure 1 describes the GOAL-Hēm data collection and development timeline. Key milestones included: initial development workshops, prefeasibility study, feasibility study, and Patient Voice Study.

2.2 | Initial development: Workshops 1 and 2

The initial development of GOAL-Hēm comprised two workshops with multidisciplinary groups of haemophilia experts to create a preliminary list of goal areas and attainment levels.[1,8,10] Experts in the development of GAS tools collaborated in preparing and facilitating the workshops.

Workshop 1 consisted of six multidisciplinary clinicians who generated the first menu of goals and descriptors. Goals were developed by identifying meaningful outcomes related to haemophilia. Clinicians were instructed to develop goals highly relevant to PwH; having a reasonable chance of being influenced by treatment (in the broadest sense—including, but not limited to, factor replacement therapy); and specific enough to be clearly applicable to PwH.[8] Workshop participants then developed specific descriptions for each goal area; these descriptors were used to define attainment levels for each goal. Clinicians were instructed to develop descriptors specifically identifying an outcome, identifying change in only one variable at a time, and using lay terms. Additionally, clinicians were advised each complete set of descriptors for each goal area should not overlap with each other and should completely define each potential attainment level (i.e., each of the five points in the GAS).[8]

Workshop 2 comprised eight multidisciplinary healthcare practitioners (HCPs) who had not participated in Workshop 1.[8] The first task was to evaluate the goals and descriptors identified in Workshop 1 and consider: Did the goal titles appropriately describe the content within each goal? Did the goals capture the most common goals related to haemophilia? Were the goal areas relevant and/or meaningful to PwH? Was the content within each goal area clearly explained? The second task was to consider whether other relevant goal areas that should be included existed.

2.3 | Pre-feasibility study: Semi-structured qualitative methods

A sample of PwH and their caregivers was recruited for individual interviews. The interviews were semistructured and conducted to evaluate and revise the menu of goals and descriptors developed from the initial workshops by refining the language, validating the goals, and identifying any gaps.[1,10] An expert team was involved in the development of GAS tools and responsible for the design, conduct, recording, and analysis of the qualitative interviews.

The interviews were conducted in two parts. The first part included open-ended questions with little-to-no prompting from study staff and focused on the GOAL-Hēm menu. Questions focused on the following: PwH's experience of and challenges with managing haemophilia, opportunities for improving QoL, and potential improvements for haemophilia management. The second part included a structured

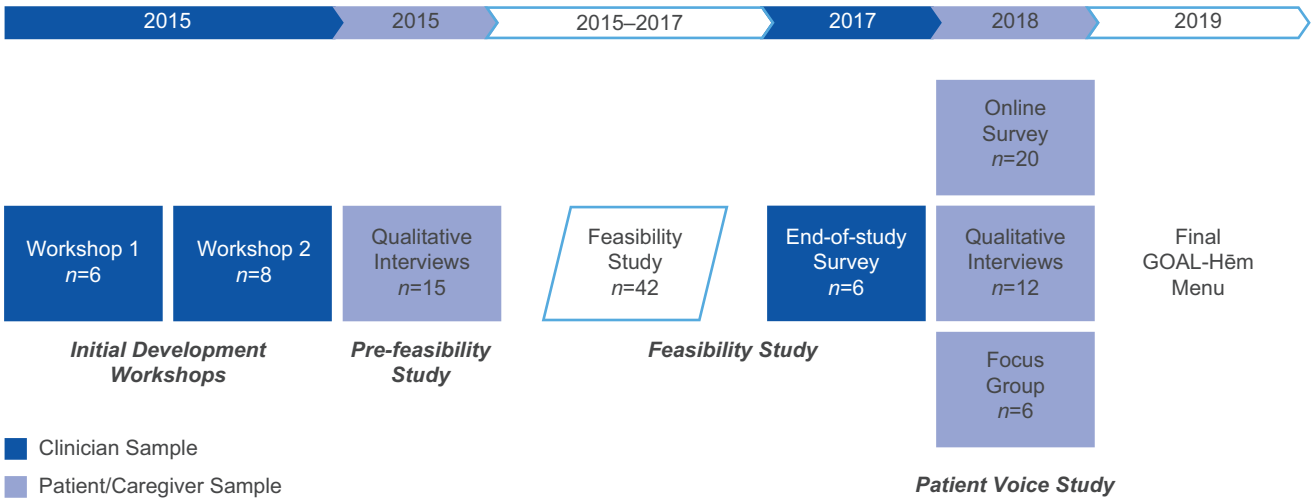

FIGURE 1 The GOAL-Hēm data collection and development timeline. Key milestones included: initial development workshops, prefeasibility study, feasibility study, and Patient Voice Study. Thirty-eight participants completed an online survey ($n = 20$), were interviewed individually ($n = 12$), or participated in a focus group ($n = 6$)

review of goal areas and descriptors, with each participant giving feedback on 8–12 goals. At least two participants gave feedback on all goals and each corresponding descriptor. Participants' opinions provided insights on the suitability, comprehensibility, and comprehensiveness of GOAL-Hēm (previously unpublished details).

2.4 | Feasibility study: Quantitative interviews at haemophilia treatment centres

The feasibility of GOAL-Hēm was assessed via a 12-week, prospective, noninterventional study at four haemophilia treatment centres (HTCs) in the United States and Canada. Participants were limited to people with moderate and severe haemophilia A (< 5%) receiving continuous prophylaxis with FVIII. GOAL-Hēm was available to investigators as an online platform and completed by trained interviewers who were HCPs in social work, nursing, medicine, or physical therapy. Baseline visits were conducted in-person, while follow-up visits at Weeks 6 and 12 were performed either in-person or via telephone interview.[4] An end-of-study survey was also completed.[11]

The quantitative measures of GOAL-Hēm feasibility and acceptability were: successful goal setting (≥90% success rate), interview completeness, goal completeness, and time to complete interviews. Content validity, construct validity, and responsiveness were also assessed. Content validity was determined based on the qualitative evaluation of the goals set. Key elements of this evaluation included degree of modification of goals and descriptors by participants, and how easily the created goals could be assigned to the menu goal area. Construct validity was evaluated via correlation at baseline and 12 weeks (end of study) using the Short Form Survey in adults (physical health component score [SF-36 PCS] and mental health component score [SF-36 MCS]) and Pediatric Quality of Life Inventory (PedsQL) in children and adolescents. Responsiveness was assessed using the standardized response mean (SRM), which was determined by dividing the mean change by the standard deviation (effect size: small > .2, moderate > .5, large > .8).[4]

2.5 | Patient Voice Study: Three distinct qualitative methods

Patient Voice was a qualitative US study that recruited PwH (any level of severity) and the caregivers of children with haemophilia. Participants reviewed GOAL-Hēm content and implementation methods via three separate methods: online survey, individual interviews, or focus group. While the feasibility of the GOAL-Hēm menu was previously demonstrated, the Patient Voice Study aimed to evaluate how comprehensible the language used to describe the goals and their descriptors was to PwH and their caregivers, and the relevance and usefulness of each goal; it also obtained direct feedback on how to revise the GOAL-Hēm menu.[9]

The online survey reviewed titles of goal areas and descriptors with regards to language and practical relevance, and the number of participants rating each item as 'clear' and 'relevant' was captured. The individual interviews lasted 60–90 min and included a structured review of 5–8 goal areas and descriptors; participants were asked to rate the language for clarity and applicability to PwH. An open-ended discussion preceded the interview, focusing on the challenges of living with haemophilia. The focus group was 3 h long and involved PwH from the Bleeding and Clotting Disorders Institute, all of whom were involved in the feasibility study. The format of the focus group was semistructured and included three topics: (1) current tools for PwH and their caregivers in everyday life (i.e., haemophilia care and management); (2) the best presentation of GOAL-Hēm to PwH and their caregivers, including most important features; and (3) the best use of the tool in routine clinic visits.[9]

TABLE 1 Overview of the GOAL-Hēm development journey

	Sample	Objective(s)	Results
Initial development workshops[1,8,10]	Workshop 1: 6 clinicians Workshop 2: 8 clinicians who did not participate in Workshop 1	Workshop 1: • Generate goal areas relevant to PwH • Develop a set of attainment levels for each goal area that could be used to complete the 5-point goal attainment scale Workshop 2: • Validate goal areas and descriptors from Workshop 1 and identify any additional concepts not already covered	• No additional goal areas identified • Goal areas recategorized into three domains: ○ Ability to manage haemophilia ○ Ability to recognize and treat complications ○ Impact of haemophilia on life • Participants built personalized 5-point scale based on three identified parameters of skill level, desire for change and severity
Pre-feasibility study[1,10]	Sections 1and 2: 15 PwH or their caregivers	Elicit PwH and caregiver input to revise and enhance the goal menu	• Goals were well received • Participants suggested reducing the list of goal areas to a more manageable range and to reduce redundancy; as a result, menu was reduced to 28 goal areas • Previously developed goals were reframed to emphasize independence • Participants endorsed individualization, noting that the ability to further customize goal areas and descriptors would be helpful, particularly with baseline status versus goal
Feasibility study[4]	Primary study: 42 PwH, including clinician facilitators • In-person baseline visits • Follow-up visits at Weeks 6 and 12 (in-person or phone) End-of-study survey: Six clinician facilitators[9]	• Investigate the feasibility of GOAL-Hēm to facilitate goal selection and tracking with GAS in people with haemophilia	• Completion rate of GOAL-Hēm exceeded feasibility criteria (≥90% success rate) • GAS interviews had acceptable time to completion with GOAL-Hēm (median baseline and follow-up visit times of 30 and 20 min, respectively) • The end-of-study survey showed the following: ○ All facilitators rated GOAL-Hēm as at least 'very useful' or 'somewhat useful' for care planning, case management, and measuring patient outcomes[11] ○ 5/6 reported difficulty using the menu because of issues of content and wording of many goals[9]
The Patient Voice Study[9]	PwH and their caregivers: • Online survey ($n = 20$) • Qualitative interviews ($n = 12$) • Focus group ($n = 6$)	• Revise the menu to be more straightforward and relatable to PwH and their caregivers	• Direct feedback on the menu resulted in many revisions, including refinement in the number of goals and descriptors (initial vs. final): ○ Adults: 29 goals and 407 descriptors versus 22 goals and 218 descriptors ○ Children: 19 goals and 228 descriptors versus 16 goals and 150 descriptors

3 | RESULTS

GOAL-Hēm is a tool to facilitate GAS that contains a haemophilia-specific goal menu developed with input from clinicians, PwH on prophylaxis, and their caregivers.[1,4,9] Table 1 summarizes the overall development of GOAL-Hēm. It specifies the participants in, purpose of, and outcomes of each step of the process.

3.1 | Workshops: The first stage of the journey

A unique set of multidisciplinary HCPs were included in each workshop (Workshop 1, $n = 6$; Workshop 2, $n = 8$).[8] Participants reviewed the four broad goal categories shown in Table 2: managing haemophilia ($n = 9$), haemophilia complications ($n = 5$), impact on daily life activities ($n = 10$), and impact on emotions and relationships ($n = 11$ goals).[10]

TABLE 2 GOAL-Hēm goal categories: Initial development workshops

Goal area category	Example goal areas
Managing haemophilia	• Being able to administer • Medication adherence • Procedure planning
Haemophilia complications	• Bleeds • Pain • Joint problems
Impact on daily activities	• Work adherence • Engaging in sports • Daily personal care
Impact on emotions and relationships	• Self-esteem • Relationships with family • Depression

Although most goals were not rated as 'common', clinicians established that the less common goal areas could be just as important to the PwH experiencing them. No additional goal areas were identified; however, the goals were recategorised into three domains: ability to manage haemophilia, ability to recognize and treat complications, and impact of haemophilia on life.[8]

Workshop 1 resulted in 35 haemophilia-specific goal areas. Participants in Workshop 2 concluded 25 (71%) of the goals were relevant and 12 (34%) were both relevant and common (previously unpublished details). For each goal area, specific descriptors were developed incorporating key parameters, such as skill level, desire for change, and utilization of available resources.[8] Additionally, each participant selected a subset of descriptors to build a personalized 5-point scale (examples in Table 3).

3.2 | Pre-feasibility study: Menu refinement

The prefeasibility study elicited input from PwH and their caregivers ($N = 15$) to revise and enhance the goal menu (previously unpublished details). The sample included four caregivers of children with haemophilia (< 12 years old), four caregivers of adolescents with haemophilia (13–17 years), four young adults with haemophilia (18–24 years), and three older adults with haemophilia (40–64 years). Haemophilia-related challenges and experiences varied greatly within the sample of PwH and their caregivers. Older adults often described pain, disabilities from joint problems, and aging-related issues. Much of the haemophilia care and management for children was done by the caregiver; most children did not have joint or mobility problems.

A common theme was the benefit of prophylactic therapy. Older adults contrasted the difference of being on prophylactic therapy compared with previous regimens. Adolescents primarily understood it was important, yet reported missing doses for no specific reason. Younger participants expressed the desire to be independent from their parents/caregivers when preparing and administering infusions. Considering participant feedback, the menu was reduced to 28 goal areas (with the option to create unique personal goals).

3.3 | Feasibility study: Validity of GOAL-Hēm

3.3.1 | Baseline data

The primary feasibility study investigated the validity of GOAL-Hēm to facilitate goal selection and tracking in 42 PwH (44 PwH signed an informed consent form, 42 PwH enrolled, of whom one terminated participation early). In-person baseline visits to set goals were either at a clinic ($n = 38$) or in the participant's home ($n = 4$). For follow-up

TABLE 3 Example goals developed using GOAL-Hēm[1]

Goal attainment level	Being able to administer factor	Bleeds
+2	I can safely administer factor by myself.	I always notice active bleeds and I treat them (e.g., add factor, adjust dose, rest joint). I always ask for help when needed and stick to a rehabilitation schedule (if established).
+1	I can administer factor by myself. I'll accept help when needed. I'm interested in improving my ability to do this.	I usually notice active bleeds and treat them (e.g., add factor, adjust dose, rest joint). Generally, I ask for help when needed and stick to a rehabilitation schedule (if established).
0	I can sometimes administer factor (1–2 of 3 per week). I'll accept help when needed. I'm interested in improving this.	I usually notice active bleeds and treat them (e.g., add factor, adjust dose, rest joint). However, I fail to ask for help when needed and do not stick to a rehabilitation schedule (if established).
−1	I can rarely administer factor (0–1 of 3 per week). I might resist help when needed. I am not very interested in improving this.	I sometimes notice active bleeds and treat them (e.g., add factor, adjust dose, rest joint). Sometimes I fail to ask for help when needed and do not always stick to a rehabilitation schedule (if established).
−2	I am unable to self-infuse. I may resist help with this. I am not interested in improving this.	I never notice active bleeds and do not treat them (e.g., add factor, adjust dose, rest joint). I do not ask for help and I do not want help.

TABLE 4 Distribution of goals set in feasibility study (Figure from Roberts et al. 2018[4])

	Goals (number)	Total goals (number)	Participants, % (*n*/*N*)[a]
Selected from the menu	Weight, exercise, and nutrition (6) Joint problems (3) Being able to administer factor (4) Medication adherence (3) Pain (2) Engaging in sports (2) Leisure activities (2) Following treatment plan (2) Career planning (1) Use of assistive devices (1)	26	57 (24/42)
Defined individualized goal[b]	Weight, exercise and nutrition (11) Leisure activities (6) Joint problems (4) Pain (3) Daily personal care (2) Being able to administer factor (2) Narcotic misuse (1) Medication adherence (1)	30	55 (23/42)
Created unique goal not on the menu[c]	Other (7)		

[a]Forty-two participants set 63 goals at baseline.
[b]'Individualized goals' are participant-defined goals that covered content included in the GOAL-Hēm menu.
[c]'Unique goals not on the menu' had content not related to the original menu shown as 'Other' ($n = 7$).

visits at Weeks 6 and 12, 10 each were conducted at the clinic and 30 (two missing) and 31 by phone (one missing), respectively (previously unpublished details). Baseline characteristics have been reported and included a total of 42 participants (median age, years [range]): nine children (8 [5–12]), nine adolescents (15 [13–18]), and 24 adults (29 [19–64]).[4]

3.3.2 | Validity and responsiveness results

Table 4 shows the distribution of goals set (i.e., content validity). Of the 63 goals set at baseline, 26 (41%) came directly from the GOAL-Hēm menu; of the 37 participant-defined goals, 30 (81%) covered content included in the GOAL-Hēm menu (i.e., 'individualized'), leaving 11% (7/63) 'unique goals' with content not in the GOAL-Hēm menu. Half of participants set one goal and the other half set two goals ($n = 21$ each); adults were more likely than children or adolescents to set two goals.[4]

Of the 26 goals participants selected from the menu, most attainment levels were customized by participants: examples of goal and attainment level customization are illustrated in Figure 2.[12] As shown, participants chose a domain such as 'Managing haemophilia' and then selected a goal (e.g., being able to administer factor). Participants then customized the goal's attainment level such as changing '*…administer factor and take other medications…*' to '*……I maintain my prophy [prophylaxis] schedule…*'

Participants and clinicians reported overall improvements in GOAL-Hēm scores (i.e., improvements in attainment levels) from baseline to Week 12. Figure 3 shows the participant- (i.e., child, adolescent, adult) and clinician-scored groups at baseline, Week 6, and Week 12; mean scores increased significantly in both groups at Week 12 ($P < .01$). An example of attainment level is shown in Figure 4. 'Independent self-care management' had a baseline value of −1 out of the 5-point scale (range, −2 to +2) corresponding to 'somewhat worse than expected'; the goal was set 1 point higher at '0', representing the 'expected outcome[12]

There was a large effect (SRM > .8) in responsiveness for all participants, as evidenced by participant-scored SRMs of 1.16 in children and adolescents and 1.25 in adults, from baseline to Week 12. The SRM of scores by clinicians were even higher, with adolescents the most responsive age group. There was good responsiveness when using the PedsQL QoL measure in children and adolescents (SRMs of .78 and .74, respectively), whereas responsiveness measured using the SF-36 was poor for adults (PCS SRM of .16; MCS SRM of .24). ABR did not change significantly in any age group during the study. Overall, GOAL-Hēm responsiveness was greater than any QoL measure or ABR.[4]

3.3.3 | Interview length

The time to complete baseline goal-setting interviews was similar between goals set using traditional GAS without a menu and those set using the GOAL-Hēm menu ($P = .35$).[12] Mean time to completion was significantly influenced by study site (median times: USA, 23.0 min vs. Canada, 61.8 min; $P < .001$) and number of goals set (mean one goal, 25.7 min vs. two goals, 57.1 min; $P = .025$).[12] Overall, the median interview length for all participants was 30 min at baseline (range, 10–120 min) compared with 20 min at Weeks 6 and 12 (range for each, 10–60 min).[4]

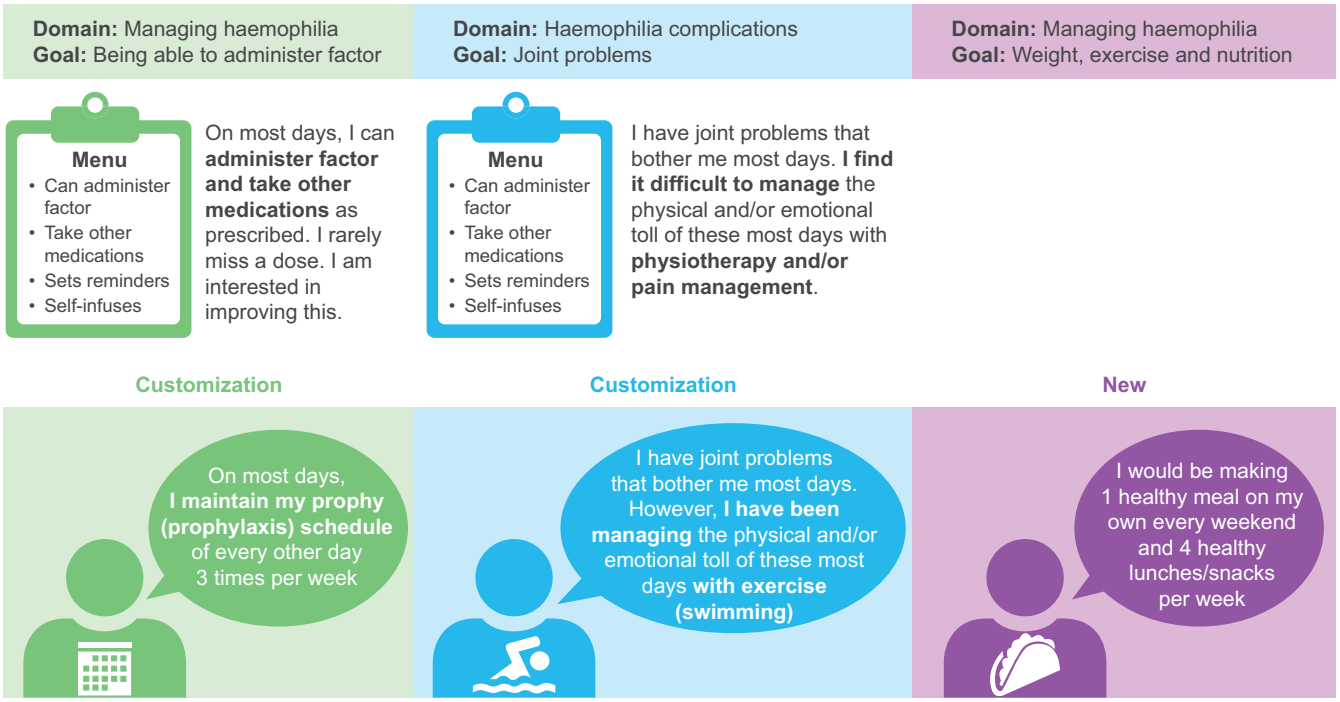

FIGURE 2 GOAL-Hēm customization of attainment levels. Example domains and goals are shown (e.g., domain: 'managing haemophilia'; goal: 'being able to administer factor'.) Participants chose a domain, selected a goal, and then customized the goal's attainment level

3.3.4 | End-of-study survey

Five of the six surveyed clinician facilitators found issues with the content and wording of many goals, which resulted in difficulties using the GOAL-Hēm menu. Nonetheless, all six clinicians agreed that GOAL-Hēm was at least 'somewhat' or 'very' useful. One clinician called the tool '*a great conversation opener, motivator, [and] guide for concerns and goals important to participants*[11]

3.3.5 | Secondary analysis

A secondary analysis of the feasibility study compared one-goal and two-goal responsiveness at each follow-up visit using SRM. The study revealed that patient-written, one-goal scales showed similar responsiveness at the Week 6 and 12 follow-ups compared with setting two goals. Both patient- and clinician-rated SRMs at Week 12 showed a large effect (SRM > .8). These results suggest that PwH may select fewer goals than recommended using standard GAS guidelines for GOAL-Hēm, which might make goal setting easier in haemophilia.[13]

3.3.6 | Other considerations in the use of GOAL-Hēm

Issues identified with the use of GOAL-Hēm included the language used in the menu and the time it took to complete the process. It can be time-consuming for clinics to schedule meetings with PwH and consistent with time commitments reported for other GAS tools,[6] the process can take even longer when PwH choose to individualize their goals. Additionally, the final follow-up at 12 weeks was difficult to schedule in some cases. Regarding the operational challenges of staff training, clinicians found there was an initial learning curve to appropriately integrate GOAL-Hēm into their daily clinical management. This was accomplished readily at follow-up visits, which showed a decrease in interview length over time.

3.4 | Patient Voice Study: Relatability of the menu content

As shown in Figure 1, 38 participants completed an online survey ($n = 20$), were interviewed individually ($n = 12$), or participated in a focus group ($n = 6$). The sample consisted of 19 caregivers and 19 adults with haemophilia, some of whom were also caregivers to a child with haemophilia (24 children total; mean age, 13 [range, 2–19] years).[9]

Feedback via online surveys, interviews, and focus groups led to considerable refinement of most goals and descriptors of GOAL-Hēm. An example of a 5-point goal attainment scale and example goals developed using GOAL-Hēm can be found in Figure 4 and Table 3. At the start of the Patient Voice Study, the GOAL-Hēm menu for adults comprised 29 goals with a total of 407 descriptors; the final menu included 22 goals and 218 descriptors. The menu for children changed from 19 goals and 228 descriptors to 16 and 150, respectively.[9] Examples of the refinements included consolidating prestudy goals of 'Work attendance' and 'Career planning' to merely 'Work'; and condensing 'Depression', 'Feelings of anger', and 'Self-esteem' to 'Emotional well-being'. Themes from the interview and focus group transcripts, as well

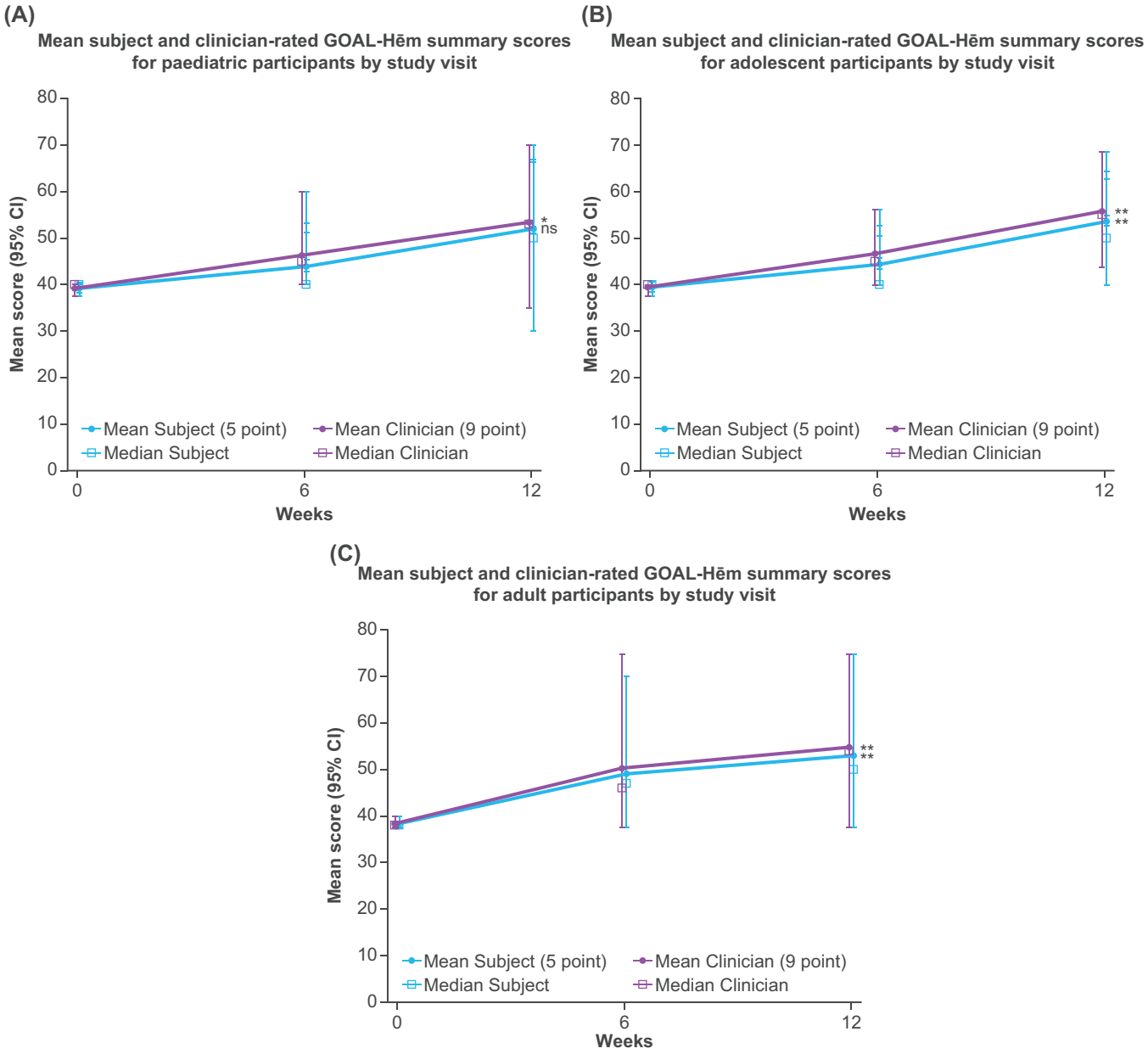

FIGURE 3 Mean (± SD) Goal Attainment Scale score by visit in the feasibility study. (A). Mean subject and clinician-rated GAS-Hēm summary scores for paediatric participants by study visit. Mean clinician-rated GAS-Hēm summary scores increased significantly for pediatric participants at Week 12. (B). Mean subject and clinician-rated GAS-Hēm summary scores for adolescent participants by study visit. Both subject and clinician-rated mean GAS-Hēm summary scores increased significantly for adolescent participants at Week 12. (C). Mean subject and clinician-rated GAS-Hēm summary scores for adult participants by study visit. Both subject and clinician-rated mean GAS-Hēm summary scores increased significantly for adult participants at Week 12

as the list of goals within each of the three domains, are shown in Figures 5 and 6, respectively.

4 | LESSONS LEARNED

Overall, the experience was positive for PwH and caretakers, as evidenced by goal attainment rates for all participants.[4] The following are additional insights collected during the development of GOAL-Hēm.

4.1 | Study outcomes

- GOAL-Hēm is sensitive to small but important changes,[4] and requires significant input from PwH and healthcare providers, in both development and implementation.
- Methodological considerations from the Patient Voice Study revealed that individual interviews and focus groups provided more robust feedback (e.g., quantity, personal comments, details) than online surveys.[9]

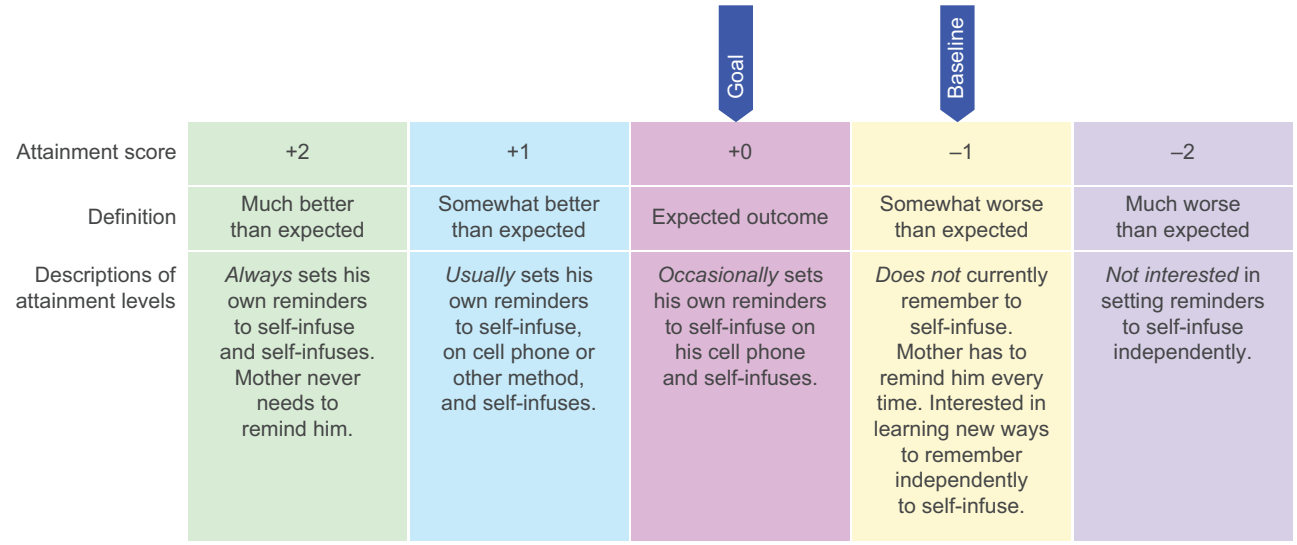

FIGURE 4 Example of a 5-point Goal Attainment Scale: independent self-care management. 'Independent self-care management' had a baseline value of −1 out of the 5-point scale (range, −2 to +2) corresponding to 'somewhat worse than expected'; the goal was set 1 point higher at '0' as the expected outcome

- The experiences of PwH during the research and development of GOAL-Hēm generated enthusiasm for the tool; they reported having more control over disease management by focusing on what is most important to them. In this way, GOAL-Hēm operates both as a measurement and management tool.
- The large effect size of GAS-Hēm for participant- and clinician-scored SRMs (baseline vs. Week 12; range, 1.16–1.36 [4]) indicated that the measure is responsive and clinically meaningful; large or medium effects of GAS-Hēm were also seen when other measures were compared (e.g., one- vs. two-goal SRMs from baseline to Week 6 [range, .7–1.26] and Week 12 [range, 1.14–1.71] [13]).

When reflecting on the large effect of GAS-Hēm (i.e., SRM > .8 [14]), it is important to consider its magnitude compared with similar assessments in other disease states. For instance, when comparing one-goal GAS in an exploratory analysis from two randomized controlled trials in Alzheimer's disease and geriatric medicine, the patient- and clinician-rated SRMs were small (.2–.5).[14,15] A systematic review of GAS in studies of psychogeriatric patients with cognitive disorders reported SRMs in the range of .2–1.7 (n = 4, three of whom were considered responsive from multiple criteria).[16] With GOAL-Hēm, the attainments based on the specific treatment goals of PwH (i.e., one- or two-goal selections) achieved the best quantitative response overall. This responsiveness appears clinically meaningful both from quantitative (large treatment effect) and qualitative perspectives (met individualized treatment goals).[17]

Correlations between GOAL-Hēm and other outcomes, such as QoL measures and ABR, were generally weak. This may suggest that GOAL-Hēm is sensitive to assessing constructs not captured by those measures. Additionally, the minimal change in QoL may have resulted from the short study duration. Therefore, construct validity could not be assessed using correlations with other outcomes. This was, however, a feasibility study, so the utility of GOAL-Hēm in detecting the relative effects of interventions remains unclear.[4]

4.2 | General takeaways

- GOAL-Hēm has the potential to provide a 'common language' for communication between PwH, their caregivers, and clinicians within HTCs and may facilitate the process of PwH moving between treatment centres.
- GOAL-Hēm represents an integrative communication tool for all multidisciplinary HTC staff, requiring only a short time to gain proficiency in its implementation (e.g., 20-min interviews at follow-up visits [4]).
- Further improvements to GOAL-Hēm could include a greater understanding of factors that influence feasibility, time to completion, and preferences of PwH.

Though an initial learning curve existed for centre staff, GOAL-Hēm was seen as a positive and useful change for both PwH, providers, and HTC staff.[1,4] Goals set using GOAL-Hēm are mutually beneficial, giving PwH more of a voice in the management and tracking of their progress toward their objectives, which are clinically meaningful to them, their caregivers, and the multidisciplinary HTC staff.

5 | DISCUSSION

The 4-year development of GOAL-Hēm included feedback from clinicians, PwH of all ages, and their caregivers. The research resulted in the development of a tool that proved to be feasible to use in the clinic and was supported by most PwH and clinicians. Participants appreci-

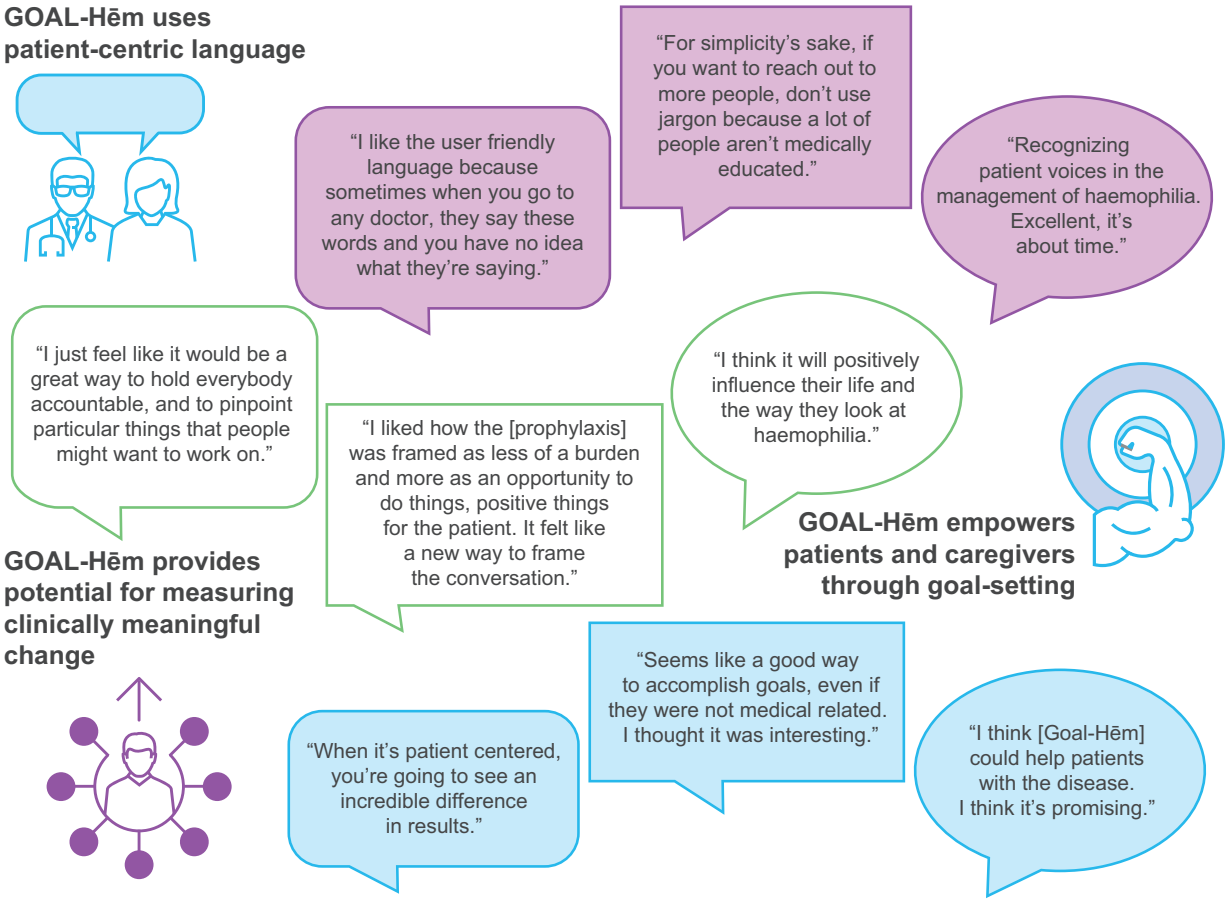

FIGURE 5 Themes from interview and focus group transcripts

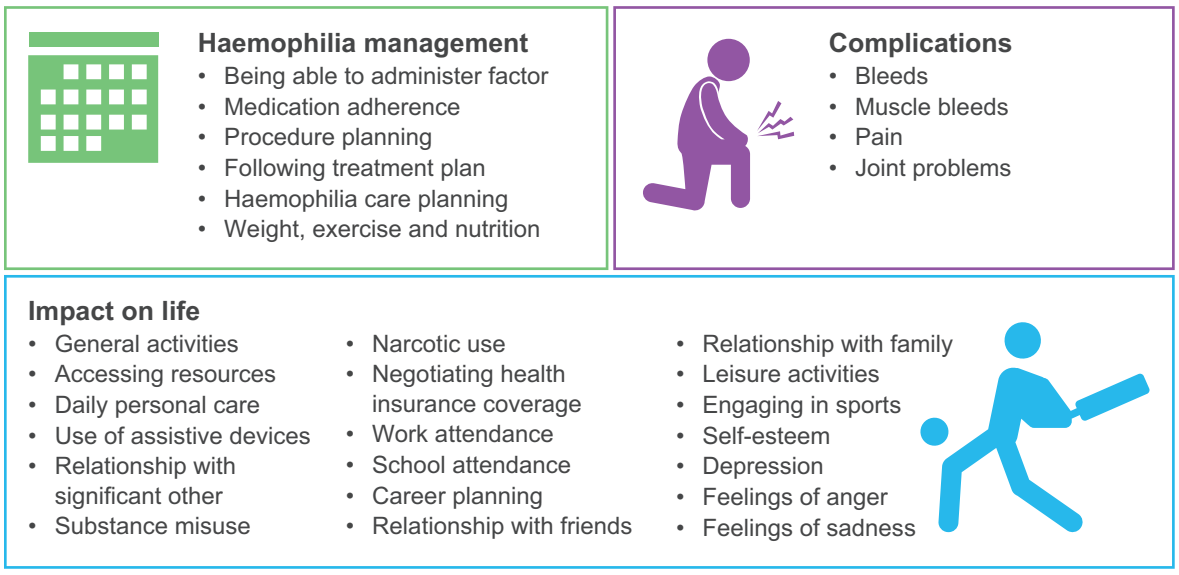

FIGURE 6 GOAL-Hēm Menu goal areas by domain

ated using the GOAL-Hēm tool as it allowed them to set their own goals and personalize their outcome measures.

Initially, working with a patient to determine and set their goals can be time consuming. However, incorporating GOAL-Hēm into clinical practice can be achieved by training a multidisciplinary team, such that the time required is shared rather than solely dependent on the clinician. Moreover, understanding the valuable nature of clinical time, ATHN has incorporated GOAL-Hēm into their new patient-facing app 'Robust Health' (www.robusthealth.com). Robust Health streamlines the process of recording and following goals over time. In addition, if a patient chooses, GOAL-Hēm goals and accomplishments can be shared directly with ATHN clinical management systems to allow recording and analysis of results longitudinally.

GOAL-Hēm has the potential to be used in additional settings, such as HTCs, and as a tool to gather real-world evidence, such as through the ATHN. ATHN has sponsored a longitudinal, observational study to assess the safety, effectiveness, and practice of treatments for people with haematologic disorders, such as haemophilia (ClinicalTrials.gov identifier NCT04398628).[18] The study, 'ATHN Transcends: A Natural History Study of Non-Neoplastic Hematologic Disorders', will follow participants for a minimum of 15 years and is expected to eventually enrol up to 3000 participants from approximately 150 ATHN-affiliated sites. The primary outcome is the safety of therapies used in the treatment of participants evaluated through assessment of adverse events included in the European Haemophilia Safety Surveillance. The study will also include the GOAL-Hēm outcome measure to describe real-world effectiveness by evaluating goal attainment. It will also explore the viability of allowing PwH to track their goal attainment progress using 'Robust Health'.

GOAL-Hēm has the potential to provide clinically meaningful results by allowing PwH to identify and track goals and health outcomes that are important to them. The final goal menu, which includes these patient-centred outcomes, is customized by age and has patient-centred language derived from qualitative feedback from key stakeholders in several settings. The final goals are in three domains: haemophilia management, haemophilia complications, and impact on life.[9] PwH endorsed GOAL-Hēm for the following reasons: level of individualization, patient-centred language, and empowerment through the goal-setting process.[4,9] Furthermore, tracking the individualized goals of PwH incrementally over time provides a novel way for HTCs to support PwH in their goal attainment journey and improves the shared decision-making process.

6 | CONCLUSION

The current review summarizes the successful development of GOAL-Hēm for use in haemophilia treatment and research. As a shared decision-making tool, GOAL-Hēm can enhance engagement of PwH in treatment and potentially improve adherence. This PCOM could be used in comparative effectiveness research to distinguish between treatments that are equivalent with respect to traditional clinical outcome measures. As both a patient engagement tool and outcome measure, GOAL-Hēm can add value in clinical and research settings.

Although initially time-intensive, this novel PCOM has demonstrated utility in detecting clinically meaningful goal attainment with the added benefit of engaging clinicians and PwH. Moreover, the development journey of GOAL-Hēm may serve as a roadmap for other PCOMs—in any therapeutic area—that aim to have clinician- and patient-endorsed content, utility, and usability.

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AUTHOR CONTRIBUTIONS

Sharon Richardson was involved in the concept and design of the study. Kenneth Rockwood was involved in the design, analysis and report writing of each study. Jonathan C. Roberts coordinated/facilitated the in-person patient focus group through BCDI. Justin Stanley and Kenneth Rockwood led data collection, initial data analysis, and development/refinement of GOAL-Hēm directly based upon data analysis. Jonathan C. Roberts, Justin Stanley, Chere Chapman, Michael Denne, Jorge Caicedo, Kenneth Rockwood, and Michael Recht were all involved in analysing data, writing/reviewing the manuscript, and approving the final version. Moses Miles was involved in writing/reviewing the manuscript and approving the final version.

CONFLICT OF INTERESTS

Jonathan C. Roberts receives research support from Takeda. He has participated in advisory boards for Genentech, Sanofi Genzyme, Takeda, Octapharma, uniQure, Novo Nordisk, Pfizer, Spark and CSL Behring. He is on speaker bureaus for Sanofi Genzyme, Takeda, Octapharma and Novo Nordisk. Sharon Richardson declares no potential conflicts of interest. Moses Miles is an employee of the American Thrombosis and Hemostasis Network, which receives grants and/or contracts from Bayer, BioMarin, CSL Behring, Genentech, Grifols, HEMA Biologics, LFB, Novo Nordisk, Octapharma, Pfizer, Sanofi, Spark and Takeda. Justin Stanley is a salaried employee of Ardea Outcomes, a consultancy that has received funding from Takeda to perform this research; Chere Chapman works for and owns equity in Ardea Outcomes. Michael Denne and Jorge Caicedo are Takeda employees at

the time of this publication. Kenneth Rockwood has asserted copyright of the Clinical Frailty Scale through Dalhousie University's Industry, Liaison, and Innovation Office. Use is free for education, research and not-for-profit health care. Users agree not to change, charge for or commercialize the scale. Kenneth Rockwood is co-founder of Ardea Outcomes, which (as DGI Clinical) in the last 3 years has contracts with pharma and device manufacturers (Hollister, INmune, Novartis, Nutricia, Roche, Takeda) on individualized outcome measurement. Michael Recht's employers have received research funding from Bayer, BioMarin, CSL Behring, Genentech, Grifols, Hema Biologics, LFB, Novo Nordisk, Octapharma, Pfizer, Sanofi, Spark, Takeda and uniQure. He has served as a paid consultant to Catalyst Biosciences, CSL Behring, Genentech, Hema Biologics, Kedrion, Novo Nordisk, Pfizer, Sanofi, Takeda and uniQure. He is on the board of directors of Foundation for Woman and Girls with Blood Disorders and Partners in Bleeding Disorders.

DATA AVAILABILITY STATEMENT

Data available on request. Some data are available in Roberts JC et al. Incorporating the patient voice and patient engagement in GOAL-Hēm: Advancing patient-centric hemophilia care. Res Pract Thromb Haemost. 2022 Feb 7;6(1):e12655. doi: 10.1002/rth2.12655.

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