




ORIGINAL ARTICLE

Incorporating the patient voice and patient engagement in GOAL-Hēm: Advancing patient-centric hemophilia care

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Abstract

Background: Goal Attainment Scaling for Hemophilia (GOAL-Hēm) is a novel, hemophilia-specific, validated patient engagement tool and patient-reported outcome instrument.

Objective: We evaluated the degree to which the language of GOAL-Hēm was patient-centric and the content valuable and relevant for people with hemophilia (PWH) and/or their caregivers.

Patients/Methods: Patients and caregivers participated in one of three investigations: an online survey, one-on-one patient interviews, or a focus group. The survey and interviews assessed the clarity and relevance of the GOAL-Hēm menu items. Interviews were semistructured, audio recorded, and transcribed verbatim. Feedback from interviews was coded as “clear,” “unclear,” “remove,” or “add.” The focus group explored participants’ experience of GOAL-Hēm and elicited recommendations for implementation. Quotations from focus group and interview transcripts were indexed and charted to emergent themes for analysis.

Results: Participants comprised 19 adults with hemophilia and 19 caregivers of children with hemophilia (survey, $n = 20$; interview, $n = 12$; focus group, $n = 6$). After their feedback, 32% (15/48) of goals were retained unchanged. Further feedback resulted in the removal of 45% (286/635) of the goal descriptors, and 30% (193/635) of the retained descriptors were modified. Three new (total = 38) goals and 42 descriptors (total = 368) were added to the menu. Thematic analysis indicated that participants were enthusiastic about patient-centric language, empowered through the goal-setting process, and recognized GOAL-Hēm could measure clinically meaningful change.

Conclusion: By listening closely to patients and caregivers, we refined GOAL-Hēm to better capture the experiences of PWH, enhance content validity, and augment implementation strategies. Incorporating the patient voice is integral to developing patient-centered outcome measures.

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KEYWORDS

Caregiver, focus group, goal, hemophilia, patient engagement, patient reported outcome measure

Essentials

- Patient/caregiver input is crucial in developing and improving patient-centric outcome measures.
- Participant feedback was obtained via online survey, one-on-one interview, and focus group.
- This study led to major changes in the GOAL-Hēm menu of goals and goal descriptors.
- Participants felt empowered by the goal-setting process and having input to refining the tool.

1 | BACKGROUND

Recognition of the importance of the patient voice in medicine has been steadily increasing.^{1,2} This is especially true for inherited bleeding disorders, where the patient voice has a growing contribution to hemophilia clinical research, hemophilia treatment center practice, and value creation.³ The evolving landscape of treatment options creates a pressing need for better ways to individualize treatment planning to optimize those outcomes most meaningful to patients.^{4,5} Perhaps the most important arena in which to incorporate the patient voice is in the development of patient-reported outcome measures.^{5,6}

GOAL-Hēm (Goal Attainment Scaling for Hemophilia) is a novel, validated patient engagement tool and patient-reported outcome instrument.^{7,8} GOAL-Hēm assesses both clinically and personally meaningful changes by allowing patients to choose and track disease-related treatment goals, as well as more global personal goals.⁹ Development of the GOAL-Hēm menu was informed by input obtained from patients and caregivers in 15 individual, semistructured interviews and as part of a comprehensive feasibility study.^{9,10} This goal-setting approach has been a staple in many therapeutic areas, including chronic medical conditions, and is recognized by regulatory bodies as an important measure in clinical trials.^{8,11} GOAL-Hēm addresses challenges frequently experienced by people with hemophilia (PWH) A on prophylaxis in three domains: managing hemophilia, hemophilia complications, and impact on life⁸ (Figure 1^{8,12}). In the clinical setting, patients can address a prespecified goal or choose their own during collaboration with their health care professional(s), in addition to creating a 5-point scale to measure progress (Table 1).⁸

The validity and feasibility of GOAL-Hēm was demonstrated in a 12-week, prospective, noninterventional, multicenter study across the United States and Canada of 42 PWH A (factor activity level <5%) on continuous prophylaxis therapy.⁸ Clinicians who facilitated interviews using GOAL-Hēm in the feasibility study completed an end-of-study survey.⁸ Among these, five of six rated GOAL-Hēm “useful” or “somewhat useful.” However, five of six also reported difficulty using the GOAL-Hēm menu because of issues around the content and wording of many of the goals (unpublished data provided by authors).

Based on feedback from the feasibility study, we recognized a need to further revise the GOAL-Hēm menu to make it more

straightforward and relatable to PWH. We therefore initiated the Patient Voice Study to enhance the patient-centricity of the instrument and ultimately to facilitate its implementation in the hemophilia community.

2 | OBJECTIVES

The aims of the Patient Voice Study were threefold: (1) to evaluate the language of goals and goal descriptors used in GOAL-Hēm with respect to comprehensibility and ease of use for patients and caregivers; (2) to assess the usefulness and relevance of each goal and its descriptors for PWH and/or their caregivers; and (3) to obtain direct input on how to revise the GOAL-Hēm menu.

3 | METHODS**3.1 | Recruitment and participants**

A total of 38 participants were recruited for the study. PWH or caregivers of PWH were contacted through the US recruiting company M3 to participate in the online survey or interviews. PWH or caregivers of children with hemophilia who participated in the feasibility study at the Bleeding and Clotting Disorders Institute were contacted and asked to participate in the focus group. Only English-speaking adults with hemophilia or adult caregivers of children with hemophilia from the United States were included. Informed consent was provided via opt-in online surveys, verbal recordings (i.e., one-on-one interviews), and written consent for the focus group. Institutional review board approval was granted by the University of Illinois College of Medicine at Peoria. Participants were not involved in the analysis or interpretation of the findings.

3.2 | Study designs

The Patient Voice Study comprised three parts: an online survey, individual interviews, and a focus group. Kenneth Rockwood, a physician with 25 years of experience in qualitative research, was involved with the study design and supervised the qualitative analyses. Kari

FIGURE 1 GOAL-Hêm Menu goal areas by domain^{8,12}

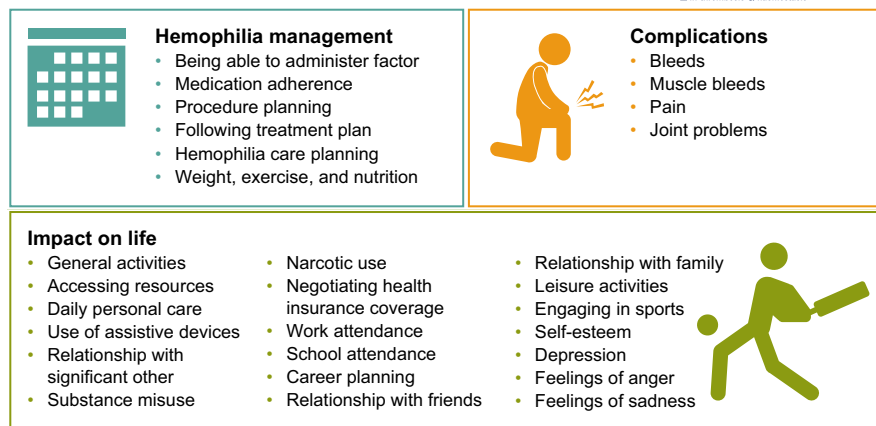


TABLE 1 Example of goal with descriptors of attainment levels

Goal: Independent self-care management	
Goal attainment level	Descriptor
Much better than expected	<i>Always</i> set their own reminders to self-infuse and self-infuse. Mother never needs to remind them
Somewhat better than expected	<i>Usually</i> set their own reminders to self-infuse, on cell phone or other method, and self-infuse
Expected outcome	<i>Occasionally</i> set their own reminders to self-infuse on cell phone and self-infuse
Somewhat worse than expected	<i>Do not</i> currently remember to self-infuse. Mother has to remind them every time. Interested in learning new ways to remember independently to self-infuse
Much worse than expected	<i>Not interested</i> in setting reminders to self-infuse independently

Knox, a registered nurse with 2 years of experience in hematology and trained in motivational interviewing, was the lead facilitator for the focus group and conducted the one-on-one interviews. No qualitative data analysis software was used. The interview guides for the one-on-one interviews and focus group are in Appendix S1.

3.2.1 | Online surveys

Respondents completed the survey in April and May 2018. The survey comprised basic demographics, a review of goal area titles, and descriptors for language and practical relevance. Respondents rated each item using the options “clear” or “unclear,” and “relevant” or “not relevant” to PWH. Respondents could also provide specific feedback for any rating of “unclear” or “not relevant.” Quantitative assessment was based on the proportion of respondents who rated each item as both “clear” and “relevant.” Unstructured feedback was evaluated by two researchers who assigned a code to each response.

3.2.2 | Interviews

The interviews were 60–90 min in length and conducted in April and May 2018. Each interview was audio recorded and transcribed verbatim. The interviewer followed a general interview guide that included formal review of the consent form followed by verbal consent from participants, introduction of the interviewer, purpose of

the study, an informal presentation on goal attainment scaling and the development of GOAL-Hêm, and then a structured review of five to eight GOAL-Hêm goal areas and descriptors. For each item reviewed, participants were asked to rate whether the language was clear and applicable to PWH. This was followed by an open-ended discussion regarding the challenges faced by PWH. The interviewer took field notes during the interview. Feedback was coded by three researchers as “clear,” “unclear,” or “remove.”

Participants were invited to suggest changes to items that were unclear. If participants suggested new content, this was coded as “add.” Quantitative assessment was based on the proportion of respondents who rated each item as “clear.” Qualitative assessment of the open-ended portion of the interview was performed by two researchers using a thematic framework analysis. First, the audio recordings were played, and the transcripts reviewed; participants did not receive the transcripts. Next, quotes from each transcript were independently analyzed, indexed, and coded. Finally, codes were discussed until a consensus was reached to identify a thematic framework, and all quotes were categorized by theme.

3.2.3 | Focus group

The 3-h focus group meeting, which was also audio recorded and transcribed verbatim, was held in June 2018 with patients recruited from the Bleeding & Clotting Disorders Institute in Peoria, Illinois. All focus group participants also took part in the feasibility

study. Moderated by two researchers, the meeting began with a presentation outlining goal attainment scaling, which included the goal-setting process, results of the feasibility study in which they participated, and our plan to revise the GOAL-Hēm menu.

The focus group followed a semistructured format divided into discrete, topic-based discussions. Topic 1 covered current tools patients and caregivers use in their everyday life, and specifically those used for hemophilia care and management. The reported tools included applications such as Advoy and Microhealth, as well as other tools such as Fitbit, websites/internet, Apple watches (to track steps, heart rate, calories), calendars, handwritten journals, books, Weight Watchers, and outreach programs. Participants were asked to describe what they liked and disliked about these tools. The second topic covered how best to present GOAL-Hēm to patients and caregivers and which features were most important to them. The final topics reviewed the norms of clinic visits and how best to facilitate use of GOAL-Hēm in routine clinic visits. The final topics included a conversation about the preferred mode of interaction with GOAL-Hēm (e.g., written versus digital, such as an app or a website) and how best to implement this in practice.

A thematic framework analysis was used following the same methods described for the interviews.

3.3 | Revising the language of GOAL-Hēm based on participant feedback

GOAL-Hēm consisted of 635 descriptors: 19 goals with 228 descriptors for children and 29 goals with 407 descriptors for adults. Each goal area title and associated descriptors (see example shown in Table 1) was evaluated by two to five participants via online surveys and individual interviews. Items were reworded for clarity if one or more participants rated them as “unclear” and items were removed if $\geq 50\%$ of participants disliked them or advised “remove.”

4 | RESULTS

4.1 | Participant characteristics

Table 2¹³ shows the characteristics of study participants based on the method of data collection. A total of 38 adults or caregivers (19 each) participated in the qualitative study; 24 children with hemophilia were being cared for by the 19 caregivers. The age range of participants extended from a 2 year old, represented by a caregiver, in the online survey to a man 60 years of age who took part in the interviews. Most of the participants were male (68% of adults, 83% of children), with all male participants in the one-on-one interviews. Regarding the online survey, 50% (5/10) of participants were females with hemophilia and 50% (5/10) were males with hemophilia. The focus group comprised three males with hemophilia (one who was also a caregiver), one female with hemophilia (who was also a

caregiver), and two female caregivers. There are no data on anyone refusing to participate or dropping out. The characteristics of each member (i.e., clinician, manager, patient) and their attendance of meetings can be found in Appendix S1.

4.2 | Online survey

All goals were reviewed by at least one respondent (median, 1.5; range, 1–4). For the adult GOAL-Hēm menu, 28/29 (97%) goal areas and 384/407 (94%) goal descriptors were rated as clear and relevant. On the child GOAL-Hēm menu, 14/19 (74%) goal areas and 170/228 (75%) goal descriptors were rated as clear and relevant.

Altogether, 42/48 (88%) goals and 554/635 (87%) descriptors were endorsed (i.e., rated as both clear and relevant). Goals not endorsed were often described as being too impersonal. For example, regarding the “Procedure Planning” goal, one respondent commented that “it’s too medical and doesn’t feel useful as a title.” Of the 81 descriptors that were not endorsed, 74/81 (91%) were reported as “not relevant” by at least one participant, whereas one or more participants rated the remaining 7/81 (9%) descriptors as “relevant” yet unclear. Forty-nine of 81 (60%) descriptors were considered “not clear” by at least one participant and 32/81 (40%) were rated both “not relevant” and “not clear” by at least one participant.

Recommendations were given in 9% of descriptors (7/81), which were to provide definitions ($n = 5$) or rephrase ($n = 2$). Examples of five recommendations, along with the researcher’s coding and comments, are shown in Table 3.¹⁴ There are three examples of participants requesting definitions within the goals of “bleeds” and “infusing,” which resulted in a new definition for the acronym RICE (Rest, Ice, Compression, Elevation). There is also one example shown for the child goals of self-esteem and relationship with family; however, no changes were needed in this instance.

4.3 | Patient interviews

In contrast to the online survey, in the interviews most goal area titles and descriptors were not fully endorsed; approximately 70% were rated as either “unclear” or “remove.” Additionally, interview participants identified new concepts not included in the menu. A new goal area, “Independence,” was recommended for both adults and children, and “Screen Time” was suggested for the child menu; overall, 42 additional descriptors were added.

The open-ended portion of the interviews was characterized by mostly positive feedback, but critical comments were also made. The three main themes to emerge from the thematic analysis were patient-centered language, empowerment through goal setting, and potential to measure clinically meaningful change (Figure 2).

Most interview participants (8/12) endorsed having a patient app for GOAL-Hēm. Some patients felt it would be useful to have an interactive smartphone app facilitating communication with their

TABLE 2 Characteristics of study participants by data collection method and age group¹³

Characteristic	Age group	Online survey	One-on-one interviews	Focus group	Total
Participants, n	Caregiver	10	6	3	19
	Adult ^a	10	6	3	19
	Child ^b	10	9	5	24
Age, mean (range), y	Adult ^a	35 (25–51)	41 (21–60)	28 (19–43)	35 (19–60)
	Child ^b	13 (5–17)	13 (6–17)	14 (2–19)	13 (2–19)
Male, n (%)	Adult ^a	5 (50)	6 (100)	2 (67)	13 (68)
	Child ^b	7 (70)	9 (100)	4 (80)	20 (83)

^aSome adults with hemophilia also cared for a child with hemophilia.

^bSome participants cared for more than one child with hemophilia. Although children did not participate directly in the study, their demographics are shown to document inclusion of representative population.

TABLE 3 Examples of descriptors rated as “not clear” or “not relevant”

Goal	Descriptor	Participant comments	Researcher's coding and comments
Personal status examples			
Self-esteem (child)	I feel sad	These feelings have nothing to do with my son's hemophilia	No change. Caregiver responded with respect to themselves, not with respect to a child with hemophilia who struggles with self-esteem issues
Relationship with family (child)	I'd like to improve communication with my family	We already have good communication	No change. Caregiver only considered their personal experience, not necessarily all PWH
Other recommendations			
Bleeds (adult)	I could do better at applying RICE after a bleed	What is RICE?	Reword. Define RICE acronym (Rest, Ice, Compression, Elevation)
Bleeds (adult)	I'd like to establish a physiotherapy routine after I have a bleed	What is physiotherapy?	No change. Although one participant did not understand the term “physiotherapy,” physiotherapists are often involved in the assessment and treatment of people with bleeding disorders ¹⁴
Infusing (child)	I want to learn how to give myself factor	Define “factor”	No change. Although one caregiver did not understand the term “factor” in this context, factor concentrates are the main treatment method for hemophilia (FVIII for hemophilia A and FIX for hemophilia B)

Abbreviations: F, factor; PWH, people with hemophilia.

hemophilia treatment center to set goals before appointments and help track progress toward these goals.

4.4 | Focus group

Patient Voice transcripts from focus groups allowed for thematic analysis, as shown in Figure 2, and were not returned to participants. Transcripts reinforced findings from the patient interviews. Feedback on implementation of GOAL-Hêm revealed enthusiasm for a tool that was easy to understand, with relatable content. Participants agreed that goal setting could encourage PWH to be more proactive regarding their health and foster independence.

Participants agreed on the potential to partner with their care team to set individualized goals.

Participants from the focus group suggested that GOAL-Hêm should be used as a “brain starter”—that is, a tool to help patients think more clearly about their needs and aspirations. All participants in the focus group ($N = 6$) felt that the menu should be in digital format and available at least 1 week before clinic visits. Additional digital information sent before the clinic visit would also be beneficial (e.g., smartphone app, website, email), as well as online access between clinic visits. Participants suggested incorporating a range of capabilities within a patient app or website that would allow them to track factor levels and bleeds, engage with the hemophilia community, and receive feedback to enhance their motivation to continue

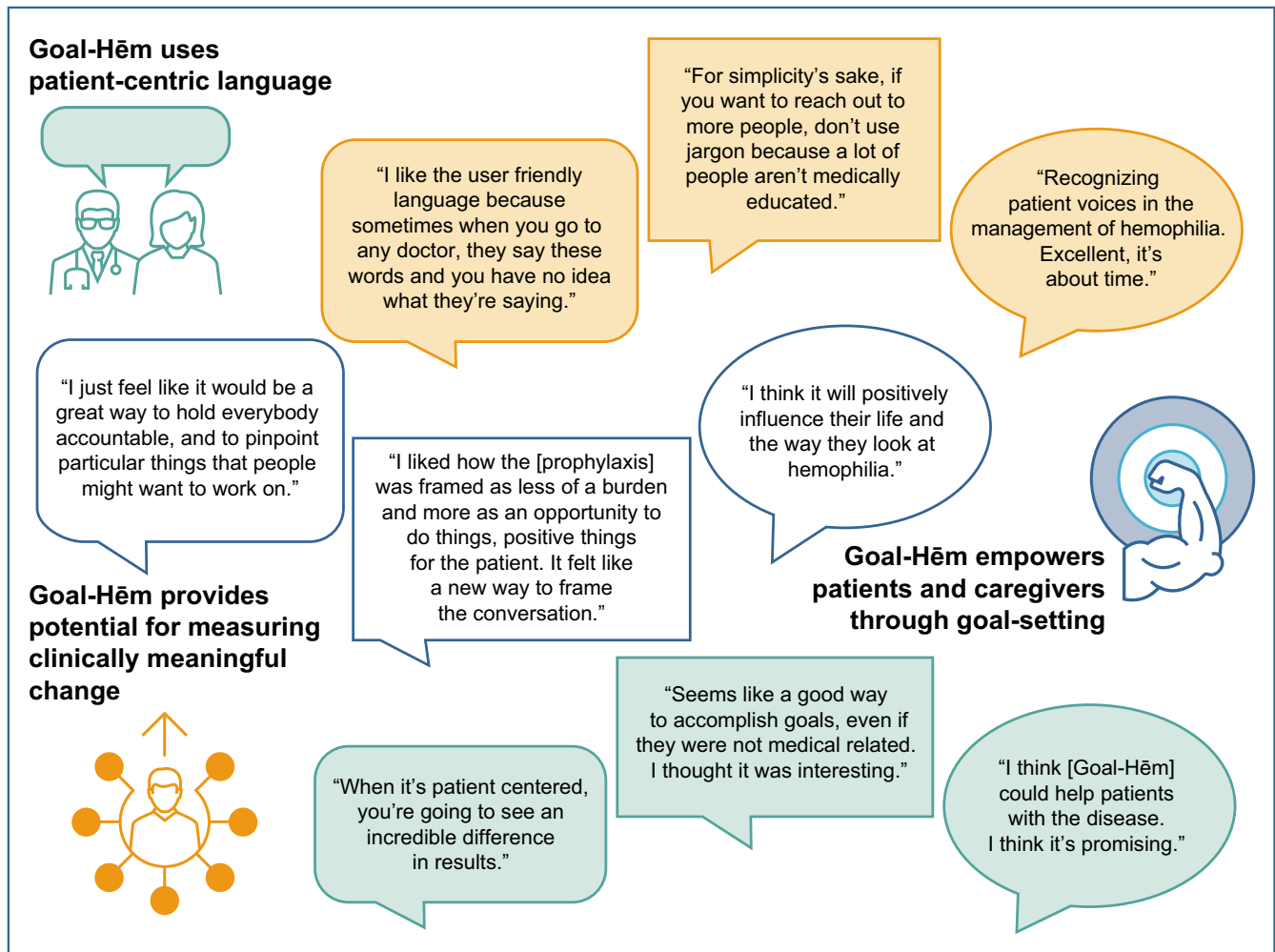


FIGURE 2 Themes from interview and focus group transcripts¹³

to strive toward their goals. At the same time, many participants felt that for GOAL-Hēm to be successful, it must be easy to use and not overly time consuming. As one caregiver stated: “We’ve tried to minimize how much time hemophilia takes out of our lives.”

4.5 | Revisions to GOAL-Hēm based on study results

Based on the collective participant feedback, more than one-half of the goals were modified (58%). Thirty-two percent of goals were unchanged (Table 4). In total, five goals (10%) were removed entirely (“Narcotic Use” and “Substance Misuse” from both the adult and child menus, and “Use of Assistive Devices” from the adult menu), and two new goals added (“Independence” to both menus and “Screen Time” to the child menu). Modification of goals consisted primarily of combining goals with similar content or separating goals with multiple components (Table 5). The final number of goals were 16 and 22 in the child and adult GOAL-Hēm menus, respectively.

In contrast, more descriptors were removed (45%) than modified (30%), reducing the total number of descriptors in the adult GOAL-Hēm menu by 189 (46%) and the child menu by 78 (34%). However, 42 further descriptors were added to the menu based on feedback from participants (Table 6). Modifications of descriptors, in addition to combination or separation, sometimes involved changing the language to make it more comprehensible and/or patient-centric. For example, a caregiver commented that a child might not understand the descriptor “I am unsure how to mix my factor independently.” This concern resulted in revision of the language of this descriptor to, “I don’t know how to prepare factor for my infusions by myself.”

5 | DISCUSSION

Advancing patient-centric care of PWH underscores the need for a tool that can measure progress toward personalized treatment goals. For research, as well as clinical purposes, it is critical that truly individualized goals be constructed and properly scaled. Further, for a tool to be embraced by patients and caregivers, it must be

TABLE 4 Refinements to number of goals before and after study

	Adult menu	Child menu	Total
Prestudy baseline menu	29	19	48
No change	11 (38%)	4 (21%)	15 (32%)
Modified ^a	15 (52%)	13 (68%)	28 (58%)
Removed	3 (10%)	2 (11%)	5 (10%)
Added	1	2	3
Poststudy final menu	22	16	38

^aModifications included combining goals with similar content, which accounts for the smaller number of goals in the final menu beyond subtracting those removed.

TABLE 5 Examples of GOAL-Hēm goal refinement

Prestudy GOAL-Hēm goals	Poststudy GOAL-Hēm goals
Muscle bleeds Bleeds	Bleeds
Work attendance Career planning	Work
Weight, exercise, nutrition	Weight Exercise Nutrition
Depression Feelings of anger Self-esteem	Emotional well-being

TABLE 6 Refinements to number of descriptors before and after study

	Adult menu	Child menu	Total
Prestudy baseline menu	407	228	635
No change	89 (22%)	68 (30%)	157 (25%)
Modified ^a	128 (31%)	65 (29%)	193 (30%)
Removed	190 (47%)	95 (42%)	286 (45%)
Added	20	22	42
Poststudy final menu	218	150	368

^aModifications included combining goals and their descriptors with similar content, which accounts for the smaller number of goal descriptors in the final menu beyond subtracting those removed.

developed with their active input and participation. Development of GOAL-Hēm has adhered to this principle since its inception, culminating in the Patient Voice Study described here. Perhaps the most striking aspect of the feasibility study, which immediately preceded the Patient Voice Study, was that most participants created their own goals—demonstrating the need for further refinement of GOAL-Hēm to work as a standardized tool and improve patient-reported outcome measures. By engaging patients and caregivers in different settings with various communication modalities, we were able to gather the necessary feedback on the content and implementation

of GOAL-Hēm in sufficient depth to advance the development of this novel patient engagement and outcome measurement tool.

The main accomplishment of the Patient Voice Study is the quantity and quality of input obtained. Participants provided extensive feedback on the clarity and the relevance of the menu items. Via the online survey, individual interviews, and through involvement in the focus group process, participants provided critical feedback that led directly to refinement of most of the goals and descriptors that comprise the GOAL-Hēm menu. Overall, the number of both goals and descriptors was reduced significantly, and the wording of many goals and descriptors was revised. The revisions described here enhanced the quality and relevance of the goal areas and descriptors of the GOAL-Hēm tool, such that it better captures the lived experiences of PWH. This in turn can inform clinical researchers on how to use language in a way that works best for patients and their caregivers.

Having refined GOAL-Hēm with this extensive patient and caregiver input, GOAL-Hēm can now help to educate clinicians on how to communicate with patients in ways that are most meaningful to them and that facilitate patient-centered clinical practice.¹⁵ Incorporating the patient voice in GOAL-Hēm in this way has the potential to enhance patient engagement with their treatment and treaters, as has been shown before when training physicians on communication skills.¹⁶ Moreover, because GOAL-Hēm can be implemented by a multidisciplinary team, patient engagement is not limited to nor dependent on the clinician but is possible with the entire clinical staff. GOAL-Hēm can also be used to help broaden the concept and measurement of treatment value, which must expand beyond cost-effectiveness to include goals that are most important to patients, families, and caregivers.⁵

In addition to the content of the instrument, the study elicited critical feedback regarding how to best implement its use in the clinic. Not surprisingly, most participants recommended that GOAL-Hēm be introduced at least 1 week before clinic visits, including more general information regarding the goal-setting process. Digital formats were preferred to paper because they were perceived as more versatile and efficient. A variety of formats, including a patient app and website, were strongly endorsed in both the patient interviews and the focus group. The possibility of digital communication with clinic staff was considered valuable and would help to optimize preparation for clinic visits. In keeping with this feedback, Goal-Hēm is now supported within Robust Health, the new patient-facing app from the American Thrombosis and Hemostasis Network (ATHN). Robust Health was built to allow patients and caregivers to have more insight into the care of individual patients, as well as to build closer relationships between patients and the treatment team. The app was designed with four goals in mind: (1) bleed and treatment logging; (2) questionnaire and survey capture; (3) outcome reporting; and (4) individualized goal tracking. If an individual opts to share his or her data, goal check-ins can be completed within the app and shared with treatment center staff.

Perhaps of equal importance, patients and caregivers appreciated the opportunity to provide their input and showed enthusiasm for further engagement. This is critical because the further development of GOAL-Hēm, as with any interactive tool that aims to be patient-centric, will benefit from ongoing consultation with patients and caregivers and can never truly be seen as a static document or process. Regulatory bodies such as the US Food and Drug Administration recognize that the goal of patient-centric material is not to mimic psychometric approaches, but to be open to what patients tell us that might not have been anticipated by a fixed, traditional approach. Listening to and defining the patient voice is an iterative process, reflected in the continuous ongoing development of instruments such as GOAL-Hēm.

From a methodological perspective, the interviews and focus groups elicited more comments than the online survey, and the feedback was generally more personal and more detailed. For example, although more than 80% of goals and descriptors were endorsed by participants in the online survey, recommendations to change up to 70% were made by those interviewed. Although the thematic analysis revealed similar content from both the interviews and the focus group, the extent of input on implementation was much greater in the focus group. Thus, the varying methods of obtaining patient and caregiver input complemented each other and can be considered for use independently based on the type of patient input being sought.

Considering data saturation, the GOAL-Hēm menu is a list of items that patients could track; however, they need not use directly what is on the menu. It is likely that people from different regions, cultures, socioeconomic status, and so on, have different goals that they would like to track, which will also change as years pass. It is possible that saturation would not be reached, and GOAL-Hēm is, by design, fully individualizable to address this. With that said, there was a sufficiently large sample size using three separate methods of data collection to best reach data saturation in this study.

Most participants in this study were males with severe hemophilia A. The smaller representation of women with hemophilia, lack of data for people with hemophilia B, and PWH on non-factor replacement therapy may raise a potential study limitation. Further research and validation in these and other patient populations, including collection of socioeconomic data, would be beneficial to improve GOAL-Hēm and ensure its utility for a wider audience. Additionally, this study was performed before the approval of emicizumab; therefore, patients on non-factor products were not included. New studies could potentially be conducted in patients receiving emicizumab where GOAL-Hēm use is optional.

A major step in the implementation of GOAL-Hēm within the hemophilia community is occurring because it is now embedded in ATHN Transcends, an ongoing cohort study involving hemophilia treatment centers nationwide that will transform how real-world data are gathered and used.¹⁷ Many opportunities arise in the context of GOAL-Hēm's implementation within ATHN. We suggest using this tool can help refine and further shape hemophilia treatment center culture around the importance of eliciting and more

clearly defining patients' goals in developing and executing treatment plans. We also believe that GOAL-Hēm will have an important role to play as new therapies for hemophilia A come to play a greater role, including extended half-life factor therapy (including potential future options allowing once weekly or fewer infusions), nonintra-venous delivery systems for factor therapy, non-factor therapy, and gene therapy. In this context, GOAL-Hēm could prove useful in clinical research to better assess the true value of each new therapeutic approach; because overall bleeding rates have decreased with widespread prophylaxis, it is increasingly difficult to compare novel therapies using only the annualized bleed rate.¹⁸ Finally, GOAL-Hēm could offer an essential tool to help guide patients, caregivers, and providers as they navigate the increasing complexity of available treatment options.

6 | CONCLUSIONS

GOAL-Hēm has the potential to improve patient outcomes through enhanced patient engagement with treatment and treaters, improved detection of clinically meaningful change for both clinicians and PWH, and development of patient goal-setting and goal-attainment data for use in patient-reported outcome research. By providing a way to measure personalized goal attainment, GOAL-Hēm can bridge an important gap in our ability to measure and thereby improve patient-centric hemophilia care.

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RELATIONSHIP DISCLOSURE

J.C.R. receives grants and/or contracts from Takeda and consulting fees from Sanofi Genzyme, Takeda, Octapharma, uniQure, Novo Nordisk, Pfizer, Spark, and CSL Behring. Additionally, J.C.R. is involved with speaker bureaus for Sanofi Genzyme, Takeda, Octapharma, and Novo Nordisk. J.C.R. also receives support from Sanofi Genzyme, Takeda, Octapharma, uniQure, Novo Nordisk, Pfizer, Spark, and CSL Behring for meeting and advisory board attendance and travel. M.R. receives grants and/or contracts from Bayer, Biomarin, CSL Behring, Genentech, Grifols, HEMA Biologics, LFB, Novo Nordisk, Octapharma, Pfizer, Sanofi, Spark, and Takeda. Additionally, M.R. receives payment or honoraria for consulting and advisory board participation from Catalyst Biosciences, CSL Behring, Genentech, HEMA Biologics, Kedrion, Novo Nordisk, Pfizer, Sanofi, Takeda, and uniQure. M.R. is on the Board of Directors for the Foundation for Women and Girls with Blood Disorders and Partners in Bleeding Disorders. S.E.G. has provided funding to the Bleeding

and Clotting Disorders Institute (BCDI) in support of the patient voice focus group study and is an employee of BCDI as Grants Manager/Research Coordinator. J.S. is a salaried employee of Ardea Outcomes. K.R. is Co-Founder, President, and shareholder of Ardea Outcomes. M.D. and J.C. are Takeda employees at the time of this publication.

AUTHOR CONTRIBUTIONS

Jonathan C. Roberts, Michael Recht, Sarah E. Gonzales, Justin Stanley, Michael Denne, Jorge Caicedo, and Kenneth Rockwood were all involved in analyzing data, writing/reviewing the manuscript and approving the final version. Jonathan C. Roberts and Sarah E. Gonzales 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. Sarah E. Gonzales recruited and consented patients for the focus group.

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SUPPORTING INFORMATION

Additional supporting information may be found in the online version of the article at the publisher's website.

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