

# Goal Attainment Scaling for haemophilia (GAS-Hēm): testing the feasibility of a new patient-centric outcome measure in people with haemophilia

J. C. Roberts<sup>1</sup>  | S. Lattimore<sup>2</sup> | M. Recht<sup>2</sup> | S. Jackson<sup>3</sup> | D. Gue<sup>3</sup> | S. Squire<sup>3</sup> | K. S. Robinson<sup>4</sup> | V. Price<sup>5</sup> | M. Denne<sup>6</sup> | S. Richardson<sup>6\*</sup> | K. Rockwood<sup>4,7</sup>

<sup>1</sup>Bleeding & Clotting Disorders Institute, Peoria, IL, USA

<sup>2</sup>The Hemophilia Center at Oregon Health & Science University, Portland, OR, USA

<sup>3</sup>Division of Hematology, University of British Columbia, Vancouver, BC, Canada

<sup>4</sup>Dalhousie University, Halifax, NS, Canada

<sup>5</sup>IWK Health Center, Halifax, NS, Canada

<sup>6</sup>Shire, US Medical Affairs, Chicago, IL, USA

<sup>7</sup>DGI Clinical Inc., Halifax, NS, Canada

## Correspondence

Jonathan C. Roberts, Bleeding & Clotting Disorders Institute, Peoria, IL, USA.  
Email: jroberts@ilbcdi.org

## Funding information

Shire

**Introduction:** To address the need for a patient-reported outcome that can measure clinically and personally meaningful change in people with haemophilia (PwH) on prophylaxis, an approach based on Goal Attainment Scaling (GAS) was developed: the GAS-Hēm.

**Aim:** To establish real-world feasibility of GAS-Hēm in PwH.

**Methods:** Patients aged 5-65 years were enrolled from four North American centres for a 12-week study. The primary outcome was the proportion of participants who completed GAS-Hēm interviews at baseline, 6 and 12 weeks. GAS-Hēm scores were obtained by subject- and clinician-rated goal attainment at Weeks 6 and 12, and compared with quality of life (QoL) measures and annualized bleed rate (ABR) for construct validity. Goals were evaluated qualitatively for content validity. Responsiveness was calculated using standardized response means (SRM).

**Results:** Forty-two participants set 63 goals. Participants preferred to define (37/63) their own goals or further individualize (23/63) from the GAS-Hēm menu. Thirty of the 37 self-defined goals were matched to goals on the GAS-Hēm menu. The most common goal areas were: weight, exercise and nutrition (n = 17); leisure activities (n = 8); and joint problems (n = 7). Both participant- and clinician-rated GAS-Hēm scores at 6 weeks (n = 40) and 12 weeks (n = 41) demonstrated satisfactory goal attainment (SRM [subject-rated] at 12 weeks for adult and paediatric groups was 1.25 and 1.16, respectively). Correlations of GAS-Hēm scores with QoL measures and ABR were uniformly small.

**Conclusion:** GAS-Hēm was feasible and tapped constructs not captured by ABR or QoL measures.

## KEYWORDS

goal attainment scaling, haemophilia A, outcome measure, patient-centred, patient engagement, prophylaxis

\*S Richardson is no longer employed by Shire

## 1 | INTRODUCTION

People with haemophilia (PwH) in developed nations have benefited from improvements in bleeding control and joint health status as prophylaxis became standard of care.<sup>1-3</sup> Consequently, traditional clinical outcome measures, such as annualized bleeding rate (ABR), and quality of life (QoL) measures (eg the Short Form (36) health survey [SF-36]), are less able to discriminate clinically and personally meaningful change in PwH.<sup>4,5</sup> There is growing need for a personalized patient-reported outcome (PRO) measuring such change in clinical settings.<sup>6</sup> Therefore, an approach based on Goal Attainment Scaling (GAS)<sup>7,8</sup> was implemented to develop a goals-based, personalized outcome measure for haemophilia: the GAS-Hēm.<sup>8</sup> The key to this method is setting measurable, meaningful goals relating directly or indirectly to the challenges of having haemophilia and assessing goal attainment over specified intervals.<sup>6,9</sup>

GAS is used in various therapeutic areas in medicine, especially in the area of rehabilitation.<sup>10-12</sup> In Canada, it has served as a primary outcome measure in regulatory approved clinical trials.<sup>13,14</sup> Adaptation of the GAS method to haemophilia may provide an effective means of demonstrating and documenting the impact of comprehensive care on outcomes and complements ongoing advances in personalized medicine.<sup>15</sup>

Introducing GAS in a new therapeutic area can be difficult because of its novel approach to outcome measurement, which requires formal recording of goals and systematic measurement of their attainment. For healthcare practitioners, a recognized challenge of implementing GAS is becoming skilled at setting patient-voiced, attainable goals and accurately describing baseline status and a full range of possible outcomes for each goal.<sup>16</sup> One helpful approach has been the use of a standardized menu of goal area attainment levels, as previously performed in geriatric rehabilitation<sup>17</sup> and dementia.<sup>12</sup>

The GAS-Hēm, created with input from a multidisciplinary group of haemophilia professionals and PwH, comprises 29 prespecified goals in three domains (Table 1). During the initial interview, participants select a goal or goals (or define their own) and collaborate with the clinician to create a 5-point goal attainment scale to measure progress (Table 2). To facilitate this process, GAS-Hēm includes descriptors of attainment levels for each goal, ranging from worst to best outcomes.

A critical step for any PRO measure is to evaluate its feasibility.<sup>18</sup> Accordingly, a study was conducted to investigate the feasibility of using a standardized GAS menu (GAS-Hēm) to set goals and track progress towards those goals in PwH, to assess the measurement properties of the GAS-Hēm tool, including near-term (12-week) responsiveness, and to obtain feedback from clinicians and patients regarding their experience of the GAS process.

## 2 | MATERIALS AND METHODS

### 2.1 | Participants

Study participants were recruited, screened and enrolled through five clinical sites at four centres in the United States and Canada. Paediatric (5-12 years), adolescent (13-18 years) and adult (19-65 years) PwH A (factor activity level <5%) who were on continuous prophylaxis therapy were eligible. PwH with an active inhibitor or a history of inhibitor and disabling joint damage were excluded. Eligible PwH were provided with study information, consent forms explaining the study parameters, and an opportunity to ask the principal investigator or his/her designee questions. All participants, or their legal guardian(s), were enrolled after providing informed consent.

### 2.2 | Study design

A 12-week, prospective, multicentre, non-interventional study was conducted at four haemophilia treatment centres (HTCs) in the United States and Canada (Figure 1). GAS-Hēm was available to investigators as an online platform, in which all documentation was completed. Participants were interviewed in person at the study start to set goals; follow-up interviews at 6 and 12 weeks were by telephone or in person. Interviews were conducted by HTC professionals, including the disciplines of social work, nursing, medicine and physical therapy. Participants set  $\geq 1$  goal during the initial interview and constructed a 5-point goal attainment scale for each, with baseline status assigned a value of -1 and the goal a value of 0 (-2 described a worse outcome, and +1 and +2 described outcomes better than the stated goal).

Goal attainment was evaluated by clinicians and participants (or by their parents/guardians for children aged <12 years) at

**TABLE 1** GAS-Hēm goal areas by domain

| Managing haemophilia   | Haemophilia complications   | Impact on life  |   |
|--|---|---|---|
| <ul style="list-style-type: none"> <li>• Being able to administer factor</li> <li>• Medication adherence</li> <li>• Procedure planning</li> <li>• Following treatment plan</li> <li>• Haemophilia care planning</li> <li>• Weight, exercise and nutrition</li> </ul> | <ul style="list-style-type: none"> <li>• Bleeds</li> <li>• Muscle bleeds</li> <li>• Pain</li> <li>• Joint problems</li> </ul> | <ul style="list-style-type: none"> <li>• General activities</li> <li>• Accessing resources</li> <li>• Daily personal care</li> <li>• Use of assistive devices</li> <li>• Relationship with significant other</li> <li>• Substance misuse</li> <li>• Narcotic use</li> <li>• Negotiating health insurance coverage</li> <li>• Work attendance</li> </ul> | <ul style="list-style-type: none"> <li>• Attending school</li> <li>• Career planning</li> <li>• Relationship with friends</li> <li>• Relationship with family</li> <li>• Leisure activities</li> <li>• Engaging in sports</li> <li>• Self-esteem</li> <li>• Depression</li> <li>• Feelings of anger</li> <li>• Feelings of sadness</li> </ul> |

12 weeks, with an interim assessment at 6 weeks. Following their input in the study's development, clinicians had the option to score goal attainment on a 9-point rather than a 5-point scale. This was meant to reflect cases in which meaningful change in a goal area might be noted but be insufficient to meet the criterion of a specified level of goal attainment (eg an outcome better than level -1 but not meeting the description for level 0 could be scored as -0.5).<sup>19</sup>

Training on GAS and the use of GAS-Hēm was provided at each centre and a study monitor was available for consultation throughout the study. Because this was a non-interventional study, no change in treatment was planned. If changes in treatment occurred while on the study, these were documented.

### 2.3 | Outcome measures

Feasibility and acceptability were measured quantitatively by the proportion of participants who completed GAS-Hēm interviews at baseline and at 6- and 12-week intervals; by achieving a ≥90% success rate of setting a goal, by the completeness of each participant's goals (ie clearly defined and measurable outcomes for the goal and all attainment levels), and by the time taken to complete interviews.

Content validity was assessed by conducting a formal qualitative evaluation of the goals set. Key elements of this evaluation included the degree to which GAS-Hēm menu goals were modified by participants, and how easily goals created by individual participants could be assigned to a GAS-Hēm menu goal area.

Construct validity of GAS-Hēm was evaluated by assessing the correlation at baseline and study end (12 weeks) of GAS-Hēm scores (range, 25-75) with widely used QoL measures: the Short Form Survey—physical health component score (SF-36 PCS) and —mental health component score (SF-36 MCS) in adults, and the Pediatric Quality of Life Inventory (PedsQL) in children and adolescents.<sup>4,20,21</sup> Correlations of GAS-Hēm scores with ABR were also considered.

**TABLE 2** Five-point\* goal attainment scale for goal: independent self-care management

| Attainment score | Definition                    | Descriptions of attainment levels   |
|------------------|-------------------------------|---|
| +2               | Much better than expected     | <i>Always</i> sets his own reminders to self-infuse and self-infuses; mother never needs to remind him  |
| +1               | Somewhat better than expected | <i>Usually</i> sets his own reminders to self-infuse, on cell phone or other method, and self-infuses   |
| 0 (goal)         | Expected                      | <i>Occasionally</i> sets his own reminders to self-infuse on his cell phone and self-infuses  |
| -1 (baseline)    | Somewhat worse than expected  | <i>Does not</i> currently remember to self-infuse. Mom has to remind him every time. Interested in learning new ways to remember independently to self-infuse |
| -2               | Much worse than expected      | <i>Not interested</i> in setting reminders to self-infuse independently   |

\*Clinicians had the option to score goal attainment on a 9-point scale (eg an outcome better than level -1 but not meeting the description for level 0 could be scored as -0.5).

Responsiveness of GAS-Hēm was evaluated using the standardized response mean (SRM),<sup>22</sup> which was calculated by dividing the mean change by the standard deviation (SD) of the change (SRM >0.2 corresponds to small, >0.5 to moderate and >0.8 to large effect). The GAS-Hēm SRM was compared with the SRM of SF-36 PCS and SF-36 MCS in adults, and with the PedsQL in paediatrics, and with the ABR in both populations.

### 2.4 | GAS-Hēm scoring

GAS-Hēm scoring used a formula accounting for the extent to which participants achieved their goals, the extent to which there was change from baseline, and the weights assigned to each goal:

$$50 + \left\{ \left[ 10 \sum (w_i x_i) \right] / \left[ 0.7 \sum w_i^2 + 0.3 \left( \sum w_i \right)^2 \right]^{1/2} \right\}$$

where  $w_i$  = weight assigned to the  $i$ th goal (this study did not include weighting, therefore  $w_i = 1$ );  $x_i$  = score of the  $i$ th goal; the 0.3 term in the denominator is an empirical estimate of rho (the usual degree of inter-correlation between goal areas); and  $0.7 = 1 - \rho$ .<sup>7,9,22</sup>

This formula results in a summary score of 50 when all goals are attained (individual goal attainment = 0). Scores >50 indicate better than expected outcomes (eg ≥1 goal scaled at +1/somewhat better or +2/much better). Scores <50 indicate overall worsening (eg ≥1 goal scaled as -1/somewhat worse or -2/much worse).

In summary, the intent of this measurement scale is that with a well-calibrated programme with well-calibrated scoring, the result is a mean score near 50 and a SD of approximately 10 (except at baseline).

### 2.5 | Statistical analyses

Given that this was a non-interventional study, the analysis focused on descriptive statistics. Data were analysed as observed at each visit without imputation of missing data. All analyses were stratified

by age group. An evaluable participant was defined as a participant who met the inclusion/exclusion criteria and provided GAS-Hēm data at baseline and at least one follow-up time point.

To compare clinicians' with subjects' GAS-Hēm scores, we adjusted the clinicians' 9-point GAS scores to 5-point GAS scores (derived by rounding each 0.5 score down). We also compared the 9-point with the 5-point scores using descriptive statistics and calculating Pearson's *r*.

Statistical tests, if performed, were two-tailed and interpreted as significant at 5% (*P*-value  $\leq .05$ ). Chi-square tests were applied to categorical data and *t* tests to continuous data. Data analyses were carried out by DGI Clinical Inc. in Halifax, Nova Scotia, Canada, using the R statistical programming environment, version 3.2.4 or later for Windows (R Core Team, 2016) for quantitative data. The SF-36 scores were calculated using QualityMetric Health Outcomes™ Scoring Software 4.5.1, Version 4.5.4330.22306.

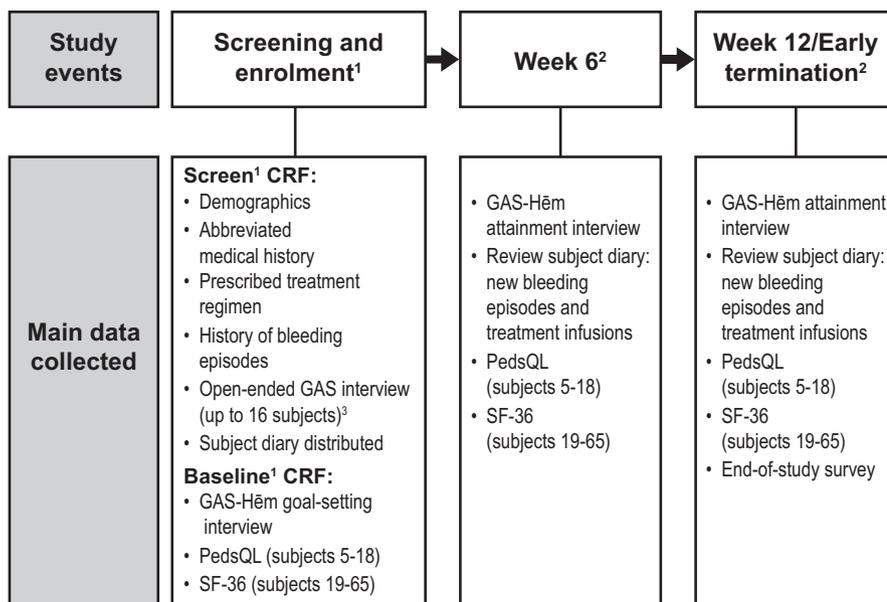
Sample size was determined based on expert opinion that approximately 36 evaluable participants would be sufficient to establish feasibility. Although balanced recruitment between age groups was not a requirement of the study, it was planned that a minimum of eight subjects should be enrolled in each group.

The study was conducted in accordance with the Declaration of Helsinki, all study materials were reviewed and approved by the institutional review boards of each participating HTC, and written informed consent was obtained.

### 3 | RESULTS

#### 3.1 | Patient characteristics

Overall, 44 patients were screened, 42 participants (41 males) enrolled in the study, and 41 completed. Most were White and had been on



1. The screening and baseline visits were combined into a single visit or conducted at separate visits up to 7 d apart.

2. Follow-up visits were scheduled within  $\pm 10$  d of the target visit date and could be conducted by telephone.

3. Interview done without using the GAS-Hēm tool during the interview.

CRF, case report form; GAS-Hēm, Goal Attainment Scaling for haemophilia; PedsQL, Pediatric Quality of Life Inventory; SF-36, 36-Item Short Form Survey—Physical Health Component Score

FIGURE 1 Study design

| Parameter   | Paediatric<br>n = 9 | Adolescent<br>n = 9 | Adult<br>n = 24 | All<br>n = 42 |
|---|---------------------|---------------------|-----------------|---------------|
| Age, year, median (range)                                       | 8 (5–12)            | 15 (13–18)          | 29 (19–64)      | 24 (5–6)      |
| Race, n %   |                     |                     |                 |               |
| White   | 7 (78)              | 8 (89)              | 20 (83)         | 35 (83)       |
| Asian   | 1 (11)              | 0                   | 2 (8)           | 3 (7)         |
| Black   | 1 (11)              | 1 (11)              | 0               | 2 (5)         |
| Other   | 0                   | 0                   | 2 (8)           | 2 (5)         |
| Total duration on any prophylaxis regimen, year, median (range) | 7 (4–9)             | 14 (11–17)          | 19 (1–29)       | 16 (4–29)     |

TABLE 3 Participant demographics

lifelong prophylaxis (Table 3). One participant was ineligible due to an active inhibitor and one was lost to follow-up. The majority of participants had haemophilia A ( $n = 40$ , 95%), except for two adults with haemophilia B. Thirty-eight (90%) participants had clotting factor levels below 1% and four (two paediatric, two adult) between 1% and 5%.

Most participants lived with family ( $n = 33$ , 79%) and most were unmarried ( $n = 33$ , 79%). All minors ( $n = 18$ ) had a caregiver/informant participating. The majority ( $n = 25$ , 60%) reported engaging in daily exercise; five (12%) were active  $\leq 2$  times per month. Common exercises reported were walking, running, swimming and body building. Fewer participants ( $n = 10$ , 24%) engaged in contact sports, with the exception of adolescents ( $n = 4/9$ , 44%).

Frequency of infusion varied; the most common schedule was three times per week ( $n = 14$ , 33%), including 6/9 adolescents. Adults infused more frequently, with 46% ( $n = 11/24$ ) infusing at least every second day. At baseline, participants had been on their current treatment regimens for an average of 5.9 years (SD, 5.5; median, 4.5, range, 0-26). At the screening/baseline visit, 81% ( $n = 34$ ) reported that they "always" infused the prescribed dose and only 24% ( $n = 10$ ) had missed

a dose in the past month. The proportion of participants who reported "always" infusing their prescribed dose increased to 92% ( $n = 37/40$ ) at Week 6 and 95% ( $n = 39/41$ ) at Week 12.

### 3.2 | Feasibility

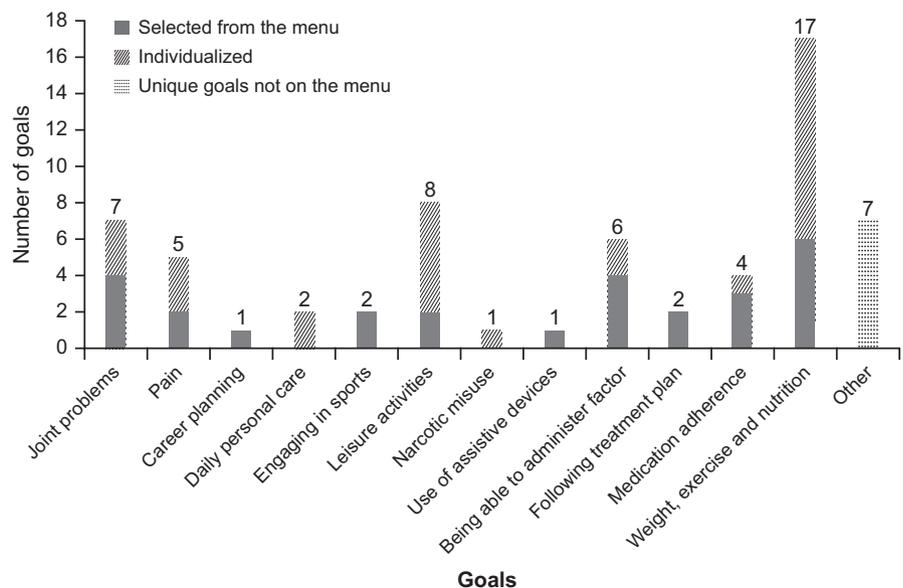
All participants completed all three interviews, except one paediatric patient who was lost to follow-up and one who completed the interview at Week 12 but not Week 6. On average, participants returned for the Week 6 and Week 12 interviews 7.1 and 13.9 weeks after the screening visit, respectively. The completion rate for goal setting and goal attainment scales (100% and 96%, respectively) exceeded the prespecified feasibility threshold of 90%.

Altogether, 63 goals were set; half of participants set one goal ( $n = 21$ ) and half set two goals ( $n = 21$ ). Adults were more likely to set two goals ( $n = 16/24$ ) than paediatric ( $n = 3/9$ ) or adolescent ( $n = 2/9$ ) participants. Participant- and clinician-rated goal attainment was scored and a current status description documented for all participants evaluated at Week 6 ( $n = 40$ ) and Week 12 ( $n = 41$ ).

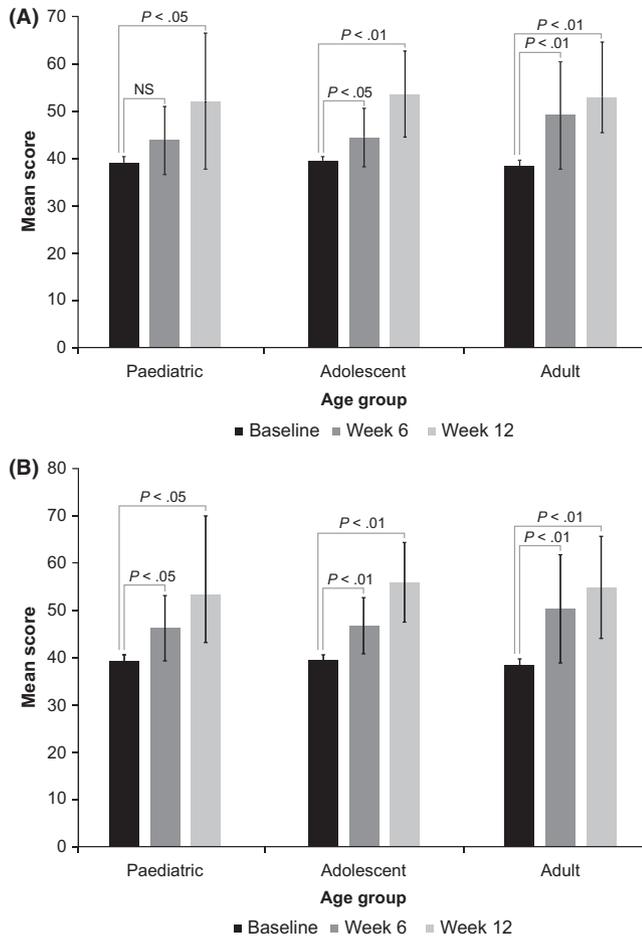
**TABLE 4** GAS-Hēm interview completion times by visit and age group

|                  | Age group  |             |            |             |
|------------------|------------|-------------|------------|-------------|
|                  | Paediatric | Adolescent  | Adult      | All         |
| Baseline         |            |             |            |             |
| n                | 9          | 9           | 24         | 42          |
| Median (Min-Max) | 25 (15-20) | 20 (10-105) | 45 (20-90) | 30 (10-120) |
| Week 6           |            |             |            |             |
| n                | 7          | 9           | 24         | 40          |
| Median (Min-Max) | 17 (10-40) | 10 (10-15)  | 20 (10-60) | 20 (10-60)  |
| Week 12          |            |             |            |             |
| n                | 8          | 9           | 24         | 41          |
| Median (Min-Max) | 18 (10-50) | 10 (10-60)  | 30 (10-60) | 20 (10-60)  |

Min, minimum; max, maximum.



**FIGURE 2** Distribution of goals set



**FIGURE 3** A, Subject-scored GAS-Hêm by visit and age group. B, Clinician-scored GAS-Hêm by visit and age group. Note: Error bars represent  $\pm 1$  SD

Initial interviews took longer than the 6- and 12-week follow-up interviews (median, 30 vs 20 minutes, respectively) (Table 4), although each age group had substantial variability. Interviews with adults took longer than those with paediatric or adolescent participants and each age group had substantial variability.

### 3.3 | Content validity

Most participants set their own goals (37/63; 59%) rather than selecting goals from the GAS-Hêm menu (26/63; 41%). For goals selected from the GAS-Hêm menu,  $\geq 1$  goal attainment description was individualized for 23 of 26 goals (all five for 20 of 23 goals). In short, only three participants did not customize some aspect of their goals. However, qualitative reviews revealed that 30 of the 37 participant-defined goals matched goals from the GAS-Hêm menu. For example, a unique goal titled "prevention of injuries and joint bleeds" was matched with the GAS-Hêm menu item "joint problems" (Figure 2).

Among all 63 goals, there were 29 (46%) from the domain of managing haemophilia, 15 (24%) from impact on life, 12 (19%) from haemophilia complications, and 7 (11%) other. The most common goal

**TABLE 5** Responsiveness of GAS-Hêm: baseline to 12 weeks

| Population                | Outcome measure                  | Standardized response mean |
|---------------------------|----------------------------------|----------------------------|
| Paediatric and adolescent | GAS-Hêm subject-rated            | 1.16                       |
|                           | GAS-Hêm adjusted clinician-rated | 1.36                       |
| Adult                     | GAS-Hêm subject-rated            | 1.25                       |
|                           | GAS-Hêm adjusted clinician-rated | 1.26                       |

from each domain, respectively, was: weight, exercise, and nutrition ( $n = 17$ ); leisure activities ( $n = 8$ ); and joint problems ( $n = 7$ ) (Figure 2).

### 3.4 | Responsiveness

Mean ( $\pm$ SD) subject- and clinician-scored GAS-Hêm scores at baseline ( $n = 42$ ), 6 weeks ( $n = 40$ ) and 12 weeks ( $n = 41$ ) are shown for each age group in Figure 3A and B. Results of clinicians' and participants' GAS-Hêm scores were closely aligned across all age strata and at each time point. Overall, improvement from baseline to 12 weeks was reported by both participants and clinicians, with mean GAS-Hêm scores for the total population of  $52.9 \pm 11.4$  and  $54.8 \pm 10.7$ , respectively ( $P < .01$  vs baseline for both). At Week 12, clinicians scored 18 of the 63 goals (28.6%) "on the line" (ie  $-1.5$ ,  $-0.5$ ,  $0.5$ , or  $1.5$ ); the total GAS-Hêm scores using this 9-point scale and the adjusted 5-point scale were highly correlated (Pearson's  $r = 0.97$ ), with similar ranges (35-75 and 38-75, respectively) and the same mode (50).

A large effect was observed on the GAS-Hêm in change from baseline to Week 12 in all participants, as shown by SRMs of 1.16-1.36 (Table 5). There were small differences in responsiveness of GAS-Hêm by rater and age group. SRM of GAS-Hêm scores were higher when carried out by clinicians (1.06-1.51) than by participants (0.89-1.51). Subject- and clinician-rated GAS-Hêm scores were most responsive in the adolescent age group (1.51 for both).

Of the QoL measures, the PedsQL showed good responsiveness in children (SRM = 0.78) and adolescents (SRM = 0.74), whereas the SF-36 PCS (SRM = 0.16) and SF-36 MCS (SRM = 0.24) were poorly responsive in adults. In all cases, the responsiveness of GAS-Hêm was greater than that of the QoL measures and ABR, which did not change during this study ( $P$  was not significant across all age strata).

### 3.5 | Construct validity

Correlations (Pearson's  $r$ ) were generally weak between GAS-Hêm and PedsQL scores in paediatric participants and between GAS-Hêm and SF-36 PCS and SF-36 MCS scores in adults at all time points. At 12 weeks, correlations between clinician- and subject-rated GAS-Hêm scores and total PedsQL scores ( $n = 17$ ) were  $-0.12$  and  $-0.16$ , respectively. Similarly, correlations between clinician- and subject-rated GAS-Hêm scores and SF-36 PCS and SF-36 MCS ( $n = 23$ ) were  $0.29$  and  $0.21$ , and  $-0.01$  and  $0.01$ , respectively. All correlations with ABR at Week 12 for the total population were weak ( $N = 40$ ,  $-0.06 < r < 0.0$ ).

### 3.6 | Treatment

Two participants switched products at Week 6; five others at Week 12. With respect to changes in infusion regimens, three continued to infuse the same dose and frequency with the new product while four changed frequency and/or units per infusion.

## 4 | DISCUSSION

In this study, the GAS-Hêm—a personalized, goal-oriented approach to measuring functional changes in PwH—was found to be feasible, valid and responsive. Feasibility was affirmed by the near-perfect study retention rate, high degree of completeness of goals set, goal attainment scales constructed and the acceptable time taken for the initial and follow-up GAS-Hêm interviews (30 and 20 minutes, respectively). Content validity was supported by the wide range of goals selected from the GAS-Hêm menu and the readiness with which most unique goals were able to be reclassified to existing GAS-Hêm goals when analysed qualitatively. GAS-Hêm proved to be a highly responsive measure of change, demonstrating similarly robust SRM for both subject- and clinician-rated goal attainment at study end, as well as significant change in GAS-Hêm scores by 6 weeks in all but the paediatric age group. The fact that correlations between GAS-Hêm and standard QoL measures were weak suggests that GAS-Hêm was tapping constructs not captured by these measures, and therefore construct validity could not be ascertained in this way.

These findings support the merit of the GAS-Hêm process and suggest that clinicians and patients would find value in using it. Besides complementing standard outcome measures to more effectively measure change, the large effect size observed suggests that GAS-Hêm may be considered a co-intervention. Other studies incorporating GAS have indicated that its use may have motivated some to address their challenges with greater effort and persistence.<sup>9,18,22</sup> Additionally, although no intervention was required, in some cases interventions (eg education, training and change in care plan) were implemented and may account for some of the observed changes. The fact that the most common changes were degrees of improvement, often considerable (eg 22% of adjusted clinician GAS scores at 12 weeks were >60), suggests that the goals were too readily accomplished. This experience will inform more widespread education and roll-out activities to ensure that goals are sufficiently challenging. Feedback to sites will also be important for future goal calibration.

Selecting and/or creating patient-centred goals and goal attainment scales were essential components of the GAS-Hêm process. A major finding was the extent of individualization of goals and descriptors of goal attainment levels, exceeding that observed when GAS has been used in other therapeutic areas.<sup>10,11,22</sup> There was a broad range of goals, including administering factor, managing diet and activity, and curbing drug use. Given the variability in patients' ages, life circumstances, illness burden, and individual needs and desires, it was understandable that such a diverse set of goals was observed. However, the almost unanimous customization of both goals and goal attainment

descriptors was surprising. Participants and caregivers consistently preferred individualization over simply selecting goals and descriptors from the GAS-Hêm menu. Not only does this verify the importance of personalizing goals; it also indicates the breadth of issues that patients are prepared to address with members of their HTC treatment team. Nonetheless, use of the menu played an important role in facilitating the process of goal setting and attainment scaling in almost all cases, particularly for the clinician.

Given the small size and limited duration of this study, these findings may not be generalizable to other centres or different patient populations. However, we did attain our target enrolment, which was as large as or larger than most feasibility studies that have introduced GAS in new disease areas.<sup>23-29</sup> Another potential limitation is that the participating study centres were highly motivated to use the GAS-Hêm and contribute to its development, perhaps creating some degree of favourable bias. In addition, inexperience using GAS may have led to goal setting that was insufficiently challenging, leading to a larger effect size. Lastly, the properties of the distribution of 5-point scale scoring suggest that it is best used, and on-the-line scoring discouraged, as has been suggested.<sup>19</sup>

The limited change in QoL measures, particularly in adults, was probably due to the short study duration and absence of an intervention. However, the change in QoL measures has been limited in interventional studies in haemophilia that were longer and where changes in bleed rate were large.<sup>4,21</sup> The minimal change in ABR was likely to have been the result of several factors: short duration, reliance on participants' recall of bleeding events to establish a baseline, and low baseline ABR in this highly adherent population.

## 5 | CONCLUSIONS

In summary, these findings suggest that GAS-Hêm has the potential to enhance patient-centred care and to complement methods for measuring personally and clinically meaningful change in PwH. The development of a revised instrument designed for broader clinical uptake (and renamed "GOAL-Hêm") is in progress, including technical improvements to facilitate the goal-setting process and to optimize the online platform for routine clinical use. Programmes for clinician training and support are also being developed. It is further anticipated that GOAL-Hêm may be applicable in other haemophilia patient populations, including haemophilia B, haemophilia carriers and inhibitor patients.

### ACKNOWLEDGEMENTS

The authors wish to acknowledge the invaluable contributions of Chere Chapman, Sherri Fay and Tumay Boxil of DGI Clinical Inc.; Elizabeth Schwartz and Jimena Goldstine of Shire; Sarah Gonzales and Shanna Beard of BCDI and Mandy Bouchard of IWK Health Centre. Funding for this study was provided by Shire. Professional medical writing support was provided by Bill Kadish, MD, of PAREXEL and funded by Shire.

## DISCLOSURES

JCR has acted as a paid consultant to Shire (formerly Baxalta), CSL Behring, HEMA Biologics and Octapharma; has received research funding from Shire (formerly Baxalta); and has served on a speaker's bureau for CSL Behring. MR has received research funding to his organization from Biogen, Genentech, Novo Nordisk and Shire. He has served on advisory boards for Biogen, CSL Behring, Genentech, Kedrion, Novo Nordisk, Pfizer and Shire. KR is President and Chief Science Officer of DGI Clinical, which has contracts with Shire on individualized outcome measurement. DG has received a speaker fee from Shire for talks on individualized prophylaxis. MD is a full-time employee of Shire. KSR, VP, SL, SS, SJ and SR have nothing to declare.

## AUTHORS CONTRIBUTIONS

JCR, MR, KSR, SJ and VP were principal investigators. SL, DG and SS conducted GAS-Hêm interviews. MD, KR and SR collaborated to lead the development of the GAS-Hêm. KR was principal author of the complete study report. All authors contributed to the drafting of the manuscript and approved the final version.

## ORCID

J. C. Roberts  <http://orcid.org/0000-0002-9415-9543>

## REFERENCES

1. Acharya SS. Advances in hemophilia and the role of current and emerging prophylaxis. *Am J Manag Care*. 2016;22(5 Suppl):s116-s125.
2. Berntorp E, Andersson NG. Prophylaxis for Hemophilia in the Era of Extended Half-Life Factor VIII/Factor IX Products. *Semin Thromb Hemost*. 2016;42:518-525.
3. Tagliaferri A, Feola G, Molinari AC, Santoro C, Rivolta GF, Cultrera DB, et al. Benefits of prophylaxis versus on-demand treatment in adolescents and adults with severe haemophilia A: the POTTER study. *Thromb Haemost*. 2015;114:35-45.
4. Bullinger M, Globe D, Wasserman J, Young NL, von Mackensen S. Challenges of patient-reported outcome assessment in hemophilia care—a state of the art review. *Value Health*. 2009;12:808-820.
5. Poonnoose P, Keshava S, Gibikote S, Feldman BM. Outcome assessment and limitations. *Haemophilia*. 2012;18(Suppl):4125-4130.
6. Recht M, Konkle BA, Jackson S, Neufeld EJ, Rockwood K, Pipe S. Recognizing the need for personalization of haemophilia patient-reported outcomes in the prophylaxis era. *Haemophilia*. 2016;22:825-832.
7. Kiresuk TJ, Pipe S, Jackson S, Roberts J, Munn J, Denne M. et al. Goal Attainment Scaling: a general method for evaluating comprehensive community mental health programs. *Community Ment Health J*. 1968;4:443-453.
8. Recht M, Pipe S, Jackson S, Roberts J, Munn J, Denne M. et al. Goal Attainment Scaling for Life - GOAL Hêm: an innovative PRO. *Haemophilia*. 2016;22(Suppl. 4):91.
9. Turner-Stokes L. Goal Attainment Scaling (GAS) in rehabilitation: a practical guide. *Clin Rehabil*. 2009;23:362-370.
10. Hanssen KT, Salyte BJ, Beiske AG, Landro NI, Hessen E. Goal attainment in cognitive rehabilitation in MS patients. *Neuropsychol Rehabil*. 2015;25:137-154.
11. Moorhouse P, Theou O, Fay S, McMillan M, Moffatt H, Rockwood K. Treatment in a Geriatric Day Hospital improve individualized outcome measures using Goal Attainment Scaling. *BMC Geriatr*. 2017;17:9.
12. Rockwood K. An individualized approach to tracking and treating Alzheimer's disease. *Clin Pharmacol Ther*. 2010;88:446-449.
13. Rockwood K, Howlett S, Stadnyk K, Carver D, Powell C, Stolee P. Responsiveness of goal attainment scaling in a randomized controlled trial of comprehensive geriatric assessment. *J Clin Epidemiol*. 2003;56:736-743.
14. Rockwood K, Fay S, Song X, MacKnight C, Gorman M. Attainment of treatment goals by people with Alzheimer's disease receiving galantamine: a randomized controlled trial. *CMAJ*. 2006;174:1099-1105.
15. Reuben DB, Tinetti ME. Goal-oriented patient care—an alternative health outcomes paradigm. *N Engl J Med*. 2012;366:777-779.
16. Turner-Stokes L. Goal attainment scaling and its relationship with standardized outcome measures: a commentary. *J Rehabil Med*. 2011;43:70-72.
17. Yip AM, Gorman MC, Stadnyk K, Mills WG, MacPherson KM, Rockwood K. A standardized menu for Goal Attainment Scaling in the care of frail elders. *Gerontologist*. 1998;38:735-742.
18. Lewis VJ, Dell L, Matthews LR. Evaluating the feasibility of Goal Attainment Scaling as a rehabilitation outcome measure for veterans. *J Rehabil Med*. 2013;45:403-409.
19. Kiresuk TJ, Smith A, Cardillo JE (eds). Goal Attainment Scaling: applications, Theory, and Measurement. *Psychometric Issues* (pp. 201-204). Hillsdale, NJ: Lawrence Erlbaum Associates. 1994.
20. Blanchette VS, O'Mahony B, McJames L, Mahlangu JN. Assessment of outcomes. *Haemophilia*. 2014;20(Suppl):4114-4120.
21. Pocoski J, Benjamin K, Michaels LA, Flood E, Sasane R. An overview of current trends and gaps in patient-reported outcome measures used in haemophilia. *Eur J Haematol*. 2014;93(Suppl):751-758.
22. Turner-Stokes L, Williams H. Goal attainment scaling: a direct comparison of alternative rating methods. *Clin Rehabil*. 2010;24:66-73.
23. Rockwood K, Joyce B, Stolee P. Use of goal attainment scaling in measuring clinically important change in cognitive rehabilitation patients. *J Clin Epidemiol*. 1997;50:581-588.
24. Rockwood K, Stolee P, Fox RA. Use of goal attainment scaling in measuring clinically important change in the frail elderly. *J Clin Epidemiol*. 1993;46:1113-1118.
25. Khan F, Pallant JF, Turner-Stokes L. Use of goal attainment scaling in inpatient rehabilitation for persons with multiple sclerosis. *Arch Phys Med Rehabil*. 2008;89:652-659.
26. Ashford S, Turner-Stokes L. Goal attainment for spasticity management using botulinum toxin. *Physiother Res Int*. 2006;11:24-34.
27. Owen C, Ziebell L, Lessard C, Churcher E, Bourget V, Villeneuve H. Interprofessional group intervention for parents of children age 3 and younger with feeding difficulties: pilot program evaluation. *Nutr Clin Pract*. 2012;27:129-135.
28. Stolee P, Awad M, Byrne K, Deforge R, Clements S, Glenny C, et al. A multi-site study of the feasibility and clinical utility of Goal Attainment Scaling in geriatric day hospitals. *Disabil Rehabil*. 2012;34:1716-1726.
29. Toto PE, Skidmore ER, Terhorst L, Rosen J, Weiner DK. Goal Attainment Scaling (GAS) in geriatric primary care: a feasibility study. *Arch Gerontol Geriatr*. 2015;60:16-21.

**How to cite this article:** Roberts JC, Lattimore S, Recht M, et al. Goal Attainment Scaling for haemophilia (GAS-Hêm): testing the feasibility of a new patient-centric outcome measure in people with haemophilia. *Haemophilia*. 2018;00:1-8. <https://doi.org/10.1111/hae.13454>