

# Responsiveness and Construct Validity of Goal Attainment Scaling for Haemophilia (GAS-Hēm): A Novel, Personalised, Patient-reported Outcome for Haemophilia

Michael Recht,<sup>1</sup> Shannon Jackson,<sup>2</sup> Jonathan C. Roberts,<sup>3</sup> K. Sue Robinson,<sup>4</sup> Victoria Price,<sup>4</sup> Susan Lattimore,<sup>1</sup> Deborah Gue,<sup>5</sup> Sandra Squire,<sup>5</sup> Michael B. Denne,<sup>6</sup> Kenneth Rockwood<sup>7</sup>

<sup>1</sup>The Hemophilia Center at Oregon Health & Science University, Portland, OR, USA; <sup>2</sup>University of British Columbia, Department of Hematology, Vancouver, BC, Canada; <sup>3</sup>Bleeding & Clotting Disorders Institute, Peoria, IL, USA; <sup>4</sup>Dalhousie University, Halifax, NS, Canada; <sup>5</sup>St. Paul's Hospital, Vancouver, BC, Canada; <sup>6</sup>Shire, US Medical Affairs, Chicago, IL, USA; <sup>7</sup>DGI Clinical Inc., Halifax, NS, Canada

## Introduction

- People with haemophilia in the developed world have experienced major gains in bleeding control and joint health status since the advent of prophylaxis as the standard of care<sup>1</sup>
- However, people with haemophilia continue to have complex medical issues that require a multi-dimensional assessment. Standard measures used to monitor health status and patient outcomes, such as annualised bleeding rate (ABR) or Quality of Life (QoL) instruments, are often not able to discriminate clinically and personally meaningful change in people with haemophilia who are on prophylaxis<sup>1</sup>
- To address the need for a novel outcome measure to complement standard measures, an approach based on Goal Attainment Scaling (GAS)<sup>2</sup> was developed to create a goal-based, personalised instrument for haemophilia: the GAS-Hēm<sup>3,4</sup>
  - The core of this method is setting measurable, meaningful goals and assessing the degree to which they are attained over specified intervals<sup>5,6</sup>
  - These goals should relate directly or indirectly to the challenges posed by having haemophilia
- The GAS-Hēm comprises 29 pre-specified goals in three domains (Table 1)<sup>7</sup>

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> <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>

- For each goal, the patient and clinician collaborate to create a five-point goal attainment scale that is used to measure progress (Table 2)
- To facilitate this process, the GAS-Hēm includes descriptors of attainment levels for each goal, ranging from worst to best outcomes<sup>7</sup>

Table 2: Example of a Five-Point Goal Attainment Scale for the Goal: Independent Self-Care Management

Attainment Score	Definition	Descriptors of Attainment Levels
+2	Much better than expected	Always sets his own reminders to self-infuse and self-infuses; mother never needs to remind him
+1	Somewhat better than expected	Usually sets his own reminders to self-infuse, on cell phone or other method, and self-infuses
0 (goal)	Expected outcome	Occasionally sets his own reminders to self-infuse on his cell phone and self-infuses
-1 (baseline)	Somewhat worse than expected	Does not 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	Not interested in setting reminders to self-infuse independently

- A feasibility study was conducted at four haemophilia treatment centres in the United States and Canada to evaluate the acceptability, feasibility, validity and responsiveness (sensitivity to change) of the GAS-Hēm<sup>8</sup>
- Data on the content validity and feasibility of GAS-Hēm were previously presented<sup>8</sup>

## Objective

- To establish the construct validity and responsiveness (sensitivity to change) of the GAS-Hēm in adult and paediatric patients with moderate or severe haemophilia in a real-world clinical setting

## Methods

- Patients aged 5–65 years with haemophilia A or B of any severity and who were receiving prophylaxis were recruited from four North American centres into the 12-week non-interventional study. Participants set one or more goals during an interview with a clinician trained in the use of GAS-Hēm and constructed a five-point goal attainment scale
- Goal attainment was evaluated by clinicians and by participants (or by their parents/guardians for children aged < 12 years) at 12 weeks, with an interim assessment at 6 weeks
  - GAS-Hēm scores were derived using a formula that accounts for the extent to which individual patients and groups of patients achieve their goal(s)<sup>2,5</sup>
  - This formula resulted in a score of 50 when each goal was reached (0), with scores < 50 representing failure to achieve at least one goal (-1 or -2) and scores > 50 indicating better than expected outcome (+1 or +2) on at least one goal
  - GAS-Hēm scores were normalised, with a standard deviation of 10
  - This method of scoring was designed to result in scores very near 50 when the goal attainment scale is well calibrated
- Construct validity of GAS-Hēm was evaluated by assessing the correlation at baseline and study end (12 weeks) of GAS-Hēm scores (range, 0–100) with widely used QoL measures: the Short Form Survey – Physical Health Component Score (SF-36 PCS) and SF-36 Mental Health Component Score (SF-36 MCS)<sup>9</sup> in adults, and the Pediatric Quality of Life Inventory (PedsQL)<sup>10</sup> in children and adolescents

- Responsiveness of GAS-Hēm was evaluated using the standardised response mean (SRM), which is calculated by dividing the mean change by the standard deviation of the change (SRM > 0.2 corresponds to small, > 0.5 to moderate and > 0.8 to large effect)
  - Responsiveness of GAS-Hēm was compared with the SRM of SF-36 PCS and SF-36 MCS in adults, and with the PedsQL in children and adolescents
- Statistical analysis focussed on descriptive statistics
  - Data were analysed as observed at each visit without imputation of missing data

## Results

- Participants
  - Overall, 44 patients were screened; 42 participants (41 males) enrolled in the study, of whom 41 completed the study (Table 3)
  - The majority had been on prophylaxis for most of their lives (Table 3)

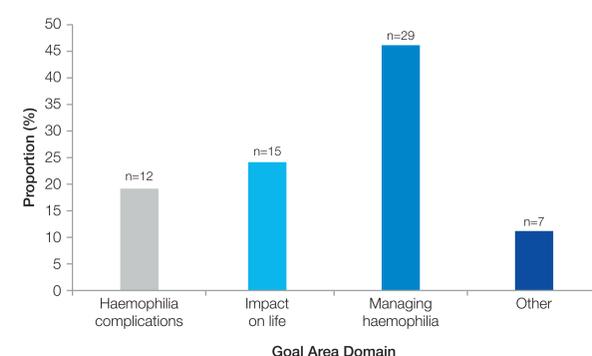
Table 3: Participant Demographics

Parameter	Paediatric n=9	Adolescent n=9	Adult n=24	All N=42
Age, y, median (range)	8 (5–12)	15 (13–18)	29 (19–64)	24 (5–64)
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, y, median (range)	7 (4–9)	14 (11–17)	19 (1–29)	16 (4–29)

## Goals

- Setting goals and monitoring goal attainment
  - A total of 63 goals were set in various domains (Figure 1)
  - 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 subjects, 80%) than paediatric (n=3/9, 33%) or adolescent (n=2/7, 22%) participants

Figure 1: Distribution of Goals Set by Domain



## GAS-Hēm Outcomes

- Mean (± SD) subject- and clinician-scored GAS-Hēm scores at baseline (n=42), 6 weeks (n=40) and 12 weeks (n=41) are shown in Figures 2A and 2B

Figure 2A: Subject-Scored GAS-Hēm by Visit and Age Group

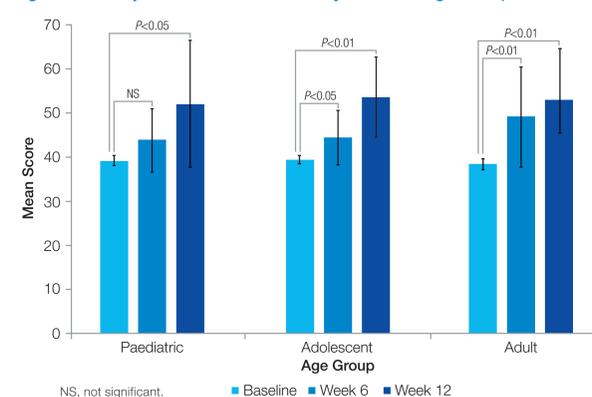
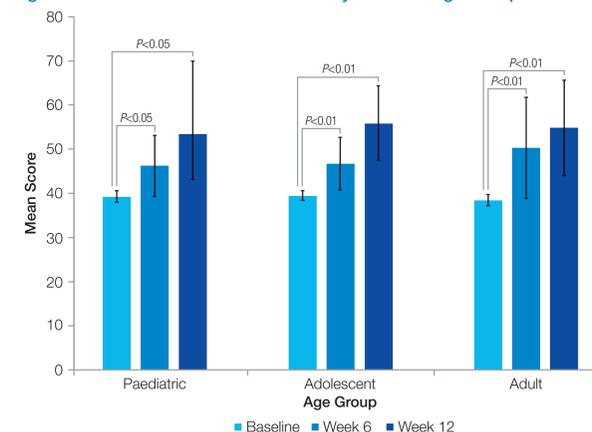


Figure 2B: Clinician-Scored GAS-Hēm by Visit and Age Group



- Overall improvement from baseline to 12 weeks was reported by both participants and clinicians with GAS-Hēm scores of 52.9 ± 11.4 and 54.8 ± 10.7, respectively (P<0.01 vs baseline for both)
- Results of clinicians' and participants' GAS-Hēm scores were closely aligned across all age strata and at each time point (Figures 2A and 2B)
- A large effect was observed for responsiveness of the GAS-Hēm to change from baseline in both paediatric and adult participants, as shown by SRMs of 1.16–1.50 (Table 4)

Table 4: Responsiveness of GAS-Hēm (Baseline to 12 Weeks)

Population	Outcome Measure	SRM
Paediatric and adolescent	GAS-Hēm subject-rated	1.16
	GAS-Hēm clinician-rated	1.42
Adult	GAS-Hēm subject-rated	1.25
	GAS-Hēm clinician-rated	1.50

- In contrast, SRMs for SF-36 PCS and SF-36 MCS in adults (0.16 and 0.18, respectively) showed no responsiveness
  - The PedsQL did demonstrate moderate responsiveness, with SRM = 0.78; results were similar in children (SRM = 0.74) and adolescents (SRM = 0.78)
- Correlations (Pearson's r) were generally weak between GAS-Hēm and PedsQL scores in paediatric participants and between GAS-Hēm 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-Hēm scores and total PedsQL scores (n=16) were -0.12 and -0.16, respectively
  - At 12 weeks, correlations between clinician- and subject-rated GAS-Hēm scores and SF-36 PCS and SF-36 MCS (n=23) were 0.29 and 0.21, and -0.01 and 0.01, respectively

## Conclusions

- The data from this feasibility study of GAS-Hēm – a novel, patient-centred outcome measure based on the proven methodology of GAS – provide evidence that GAS-Hēm was able to show personally and clinically meaningful change over a short time frame in adults, adolescents and children with haemophilia
- GAS-Hēm was a highly responsive measure of change in adults, adolescents and children, with similarly robust SRM for both subject- and clinician-rated goal attainment
- Correlations between GAS-Hēm and standard outcome measures were weak, suggesting that GAS-Hēm is tapping constructs not captured by standard outcome measures
  - The individualised goal setting facilitated by GAS-Hēm may account for these findings
  - Although no intervention was required or expected as part of the protocol, in some cases interventions (eg, education, training, changes in care plan) were implemented as a result of having gone through this goal-setting process, and these may also account for some of the observed change
- Given the small size and limited duration of the study, these findings should not be considered definitive or generalisable
- In summary, these findings suggest that GAS-Hēm may become a useful tool to enhance patient-centred care, as well as being a complementary method for measuring personally and clinically meaningful change in people with haemophilia

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## Contributorship

MR, JR, SJ, KSR and VP were principal investigators. SL, DG and SS did GAS-Hēm interviews. MD and KR have collaborated to lead the development of the GAS-Hēm. KR is principal author of the CSR. All authors contributed to the drafting of the poster and approved the final version.

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