Management of US adult men and women patients with hemophilia (PWH): the bridging hemophilia B experiences results and opportunities into solutions (B-HERO-S) study

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Objective

- To better understand the clinical presentation and management of men/women with mild, moderate or severe hemophilia B.

Introduction

- The global HERO (Hemophilia Experiences Results and Opportunities) program initiated in 2009 and sponsored by Novo Nordisk sought to investigate the psychosocial issues impacting persons with hemophilia (PWH) and those that care for and support them.[1,2]
- The B-HERO-S study was conducted in patients with mild, moderate or severe hemophilia B including female patients, caregivers of girls with hemophilia and pregnant participants.

Methods

- The B-HERO-S survey was conducted in the United States from 24 September 2015 to 3 November 2015, as a one-time participation in an online one-hour internet-based Independent Review Board (IRB)-approved survey.
  - Adult PWH (≥18 years) with mild, moderate or severe hemophilia B were identified by web-posted IRB-approved study via three advocacy organizations (National Hemophilia Foundation, Hemophilia Federation of America and Coalition for Hemophilia B).
  - Participants completed an electronic case report form consisting of approximately 100 multiple choice questions, including some questions that were contingent on prior answers.
- Descriptive statistical analyses were performed on the full analysis set, which included questionnaires of all participants who had completed the survey.

Results

- Characteristics of the 299 PWH who completed the survey are shown in **Table 1**.
  - Median age of the participants was 29 years (range 18−70 years).

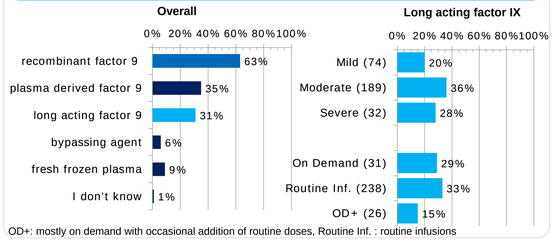
Table 1 Characteristics of PWH

	Total (N=299)	Male (N=213)	Female (N=86)
Hemophilia Severity, n (%)			
Mild (factor IX level 6−50%)	74 (25%)	49 (23%)	25 (29%)
Moderate (factor IX level 1−5%)	189 (63%)	133 (62%)	56 (65%)
Severe (factor IX level <1%)	32 (11%)	25 (13%)	5 (6%)
Inhibitor	4 (1%)	4 (2%)	0 (0%)
Age of Respondent, n (%)			
Age <30	180 (60%)	135 (63%)	45 (52%)
Age 30-45	104 (35%)	68 (32%)	36 (42%)
Age >45	15 (5%)	10 (5%)	5 (6%)
Self-reported Illnesses, n (%)			
Viral Illness	45 (15%)	41 (19%)	4 (5%)
Arthritis	148 (50%)	91 (43%)	57 (66%)
Pain	122 (41%)	106 (50%)	16 (19%)
Anxiety/depression	113 (38%)	64 (30%)	49 (57%)

N: number of respondents, n: number of responses

- PWH received recombinant, plasma-derived or extended-half-life factor; 6% received bypassing-agents **(Figure 1)**.

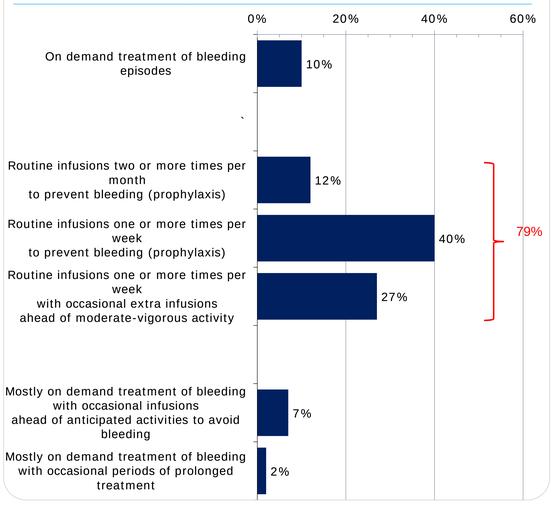
Figure 1 Type of product



OD+: mostly on demand with occasional addition of routine doses, Routine Inf. : routine infusions

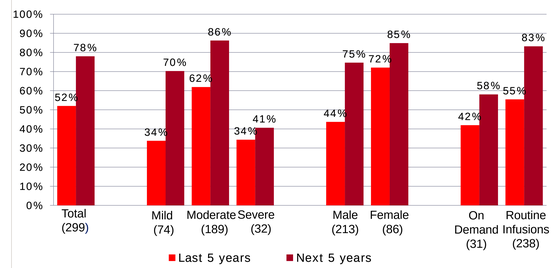
- Only 27% of PWH reported being responsible for their care vs. hematologists and nurses who were responsible for 55% and 12% of PWH, respectively.
- The decision on bleed treatment was made in coordination with the physician/nurse at the hemophilia treatment center (HTC)/clinic (61%, mild/moderate/severe: 66%/65%/28%) vs. the patient alone (16%, mild/moderate/severe: 15%/7%/66%).
- The treatment location was mostly at the hemophilia clinic/hospital but sometimes at home (56%, mild/moderate/severe: 49%/ 67%/16%) vs. always at home (10%, mild/moderate/severe: 7%/3%/63%).
- The majority of PWH were treated with routine infusions to prevent bleeding (~80%), but routine treatment varied by severity (mild/moderate/severe: 64%/86%/81%), **Figure 2**.

Figure 2 Treatment regimen



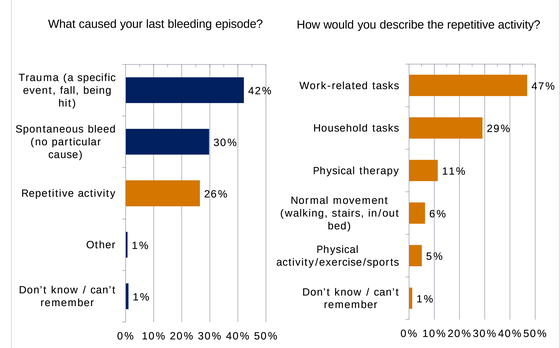
- Over the past 5 years, 52% of the PWH had factor access issues; 78% have concerns about access in the next 5 years **(Figure 3)**.

Figure 3 Issues with access to factor due to availability or affordability in the last 5 years and next 5 years



- PWH reported a median of 4 bleeds in the prior year (mild /moderate /severe / inhibitor: 1/4/4/6, male/female: 4/4) and a median of 2 joint and 2 muscle bleeds in the prior year.
- Most bleeds were joint (62%) vs. muscle (27%), mucosal (7%) or other (3%). Men reported a lower percentage of joint bleeds (57% men vs. 73% women) and a higher percentage of muscle bleeds (30% men vs. 21% women).
- Only 27% of PWH reported having a specific joint affected. This was higher for patients with severe (64%) than for those with mild (15%) or moderate (22%) hemophilia. Most commonly affected joint was knee.
- Most recent bleeding was traumatic, spontaneous or due to repetitive activity **(Figure 4)**. For PWH, most (47%) repetitive activity-related bleeding was associated with work (39% of those employed performed manual labor, 35% worked in construction/manufacturing/production).

Figure 4 Last bleeding episode

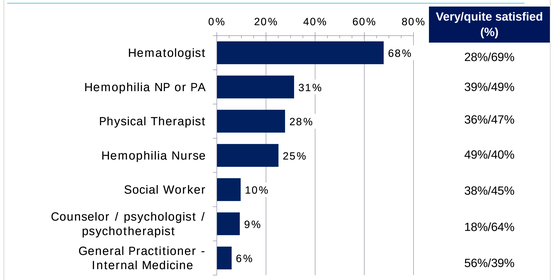


Conclusions

- B-HERO-S provides insights into the phenotype and management of men/women with mild, moderate or severe hemophilia B.
- While most PWH were responsible for their own care, most mild/moderate PWH were not as familiar with self-infusion despite reporting higher than expected use of routine treatment ("prophylaxis") strategies.
- Issues around access to factor were much higher for patients with moderate hemophilia and for women.

- Median perceived disease control was 7 (0:not-at-all, 10:extremely-well).
- PWH reported median 4 (mean:5.4, IQR: 3−6) hemophilia treatment center visits in the past year; 14% did not go to a HTC or went to a hematologist/hematology clinic.
- Most (82%) reported very/quite easy access to a HTC; length of time to get to the HTC was the most common difficulty (62%).
- Sixty-eight (68%) percent of the PWH reported seeing a hematologist for hemophilia treatment or any complications related to treatment **(Figure 5)**.
- Rates of inclusion of visits of PWH with physical therapists (PT) and social workers (SW) were lower than expected based upon a comprehensive care model, and were seen throughout in patients with mild/moderate/severe (PT: 23%/26%/50%; SW: 11%/5%/31%) hemophilia.
- Most were very/quite satisfied with care **(Figure 5)**.

Figure 5 Healthcare professionals seen for hemophilia/complications



NP: Nurse practitioner, PA: Physician's assistant

References

1. Forsyth AL, *et al. Patient Prefer Adherence* 2015 Oct 29;9:1549-60.
2. Witkop M, *et al. Am J Hematol* 2015 Dec;90 Suppl 2:S3-10.

Conflict of interest disclosure

All authors have potential conflicts of interest. Please refer to the abstract.

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