Understanding the Impact of Hemophilia B on Activity of US Children With Hemophilia (CWH) From Their Caregivers: The Bridging Hemophilia B Experiences, Results and Opportunities Into Solutions (B-HERO-S) Study

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Objective

- B-HERO-S was designed to better understand the psychosocial impact of hemophilia on adults with mild-moderate-severe hemophilia B and caregivers (CGs) of children with hemophilia (CWH), and in particular, to explore the impact of hemophilia on engagement in activities.

Introduction

- Initiated in 2009, the global HERO program and subsequent 10-country HERO quantitative study sought to investigate the psychosocial issues affecting people with hemophilia (PWH) and CGs/families.[1,2]
- The HERO study highlighted challenges with engagement in activities for PWH and CWH, with a large/moderate negative impact in the mostly moderate-severe population studied.
- B-HERO-S was designed in collaboration with health care professionals, advocacy organizations, and patients/CGs to assess the needs in this population and to address gaps identified by the HERO quantitative study data:
  - Impact on people with mild-moderate hemophilia, including affected women/girls
  - Assessment of the vigorousness and duration of participation in activities
  - Treatment modifications to support ongoing participation in activities

Methods

- US PWH (either gender, age ≥18 years) and CGs of CWH (either gender, age <18 years) were recruited through patient organizations to complete distinct (PWH/CG), IRB-approved, Internet surveys from September 26 to November 3, 2015.
- Surveys assessed in part the impact of hemophilia on activities. CGs responded in describing the current, past, and aspirational activities of their CWH (selected from a list of 35 different activities), duration and vigorousness of current activities, reasons why CWH were unable to participate in activities currently, impact of hemophilia on participation in activities, and specific changes to treatment regimens around activities.

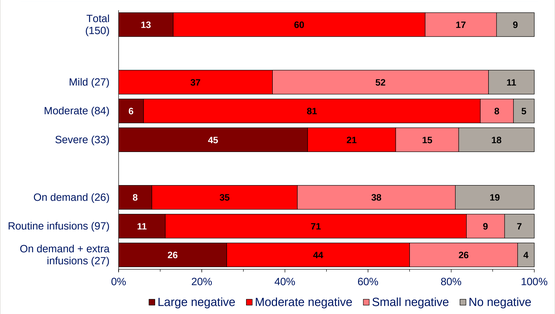
Results

- CG respondents (150) were mothers (116) or fathers (34) with a median age of 35 years (range, 21-51 years).
- CGs' oldest CWH (age <18 years) described were mostly boys (121, 81%) but also girls (29, 19%) with mild (27, 18%), moderate (84, 56%), or severe (33, 22%) hemophilia or with inhibitors (6, 4%). Median age of CWH was 10 years (range, 0-18 years) and was similar for boys and girls.

Impact of hemophilia on recreational activities

- Nearly all CWH (90%) had experienced a negative impact of hemophilia on engagement in activities (**Figure 1**).
  - Impact was significant in children with severe hemophilia (66% large or moderate impact); 37% of children with mild and 87% with moderate hemophilia experienced a large or moderate impact on activities.
  - CWH currently being treated with routine infusions to prevent bleeding and those treated for bleeding with occasional extra infusions were most frequently reported to have experienced a large or moderate impact (82% and 70%, respectively); these treatment groups may reflect activity-related treatment adjustments.

Figure 1 Impact on participation in recreational activities

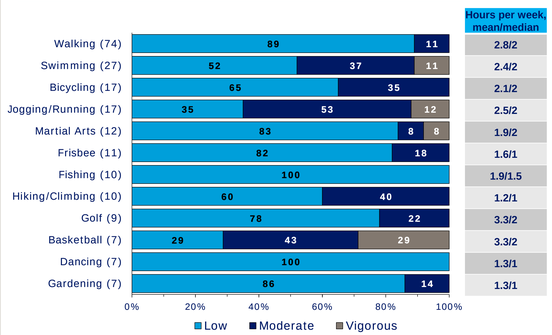


Parentheses indicate the numbers of CWH in each category.

Current and past activities

- The top 5 current activities for CWH were walking (49%), swimming (18%), bicycling (11%), jogging/running (11%), and martial arts (8%).
- The reported intensity of current activities (low/moderate/vigorous) and the mean/median duration of participation per week are shown in **Figure 2**.

Figure 2 Intensity and duration of participation in current activities

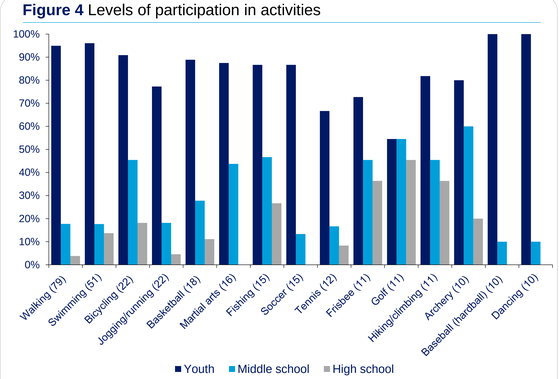


Parentheses indicate the numbers of CWH in each category.

- The top 3 past activities (>5%) were swimming (16%), basketball (7%), and soccer (6%).
- The top reasons for discontinuing past activities (reported by ≥10% of participants) are shown in **Figure 3**.
- The top 5 aspirational (desired) activities in which CWH were unable to participate due to hemophilia were football (17%), basketball (17%), soccer (16%), tennis (9%), and skateboarding (9%).
- More activities and greater percentages of participation were reported for CWH at younger ages ("youth") than during middle and high school (**Figure 4**). However, given the median CWH age of 10 years, some attrition may also be due to not having as many CWH old enough to provide data.

Figure 3 Reasons for discontinuing past activities

Figure 4 Levels of participation in activities



Parentheses indicate the numbers of CWH in each category.

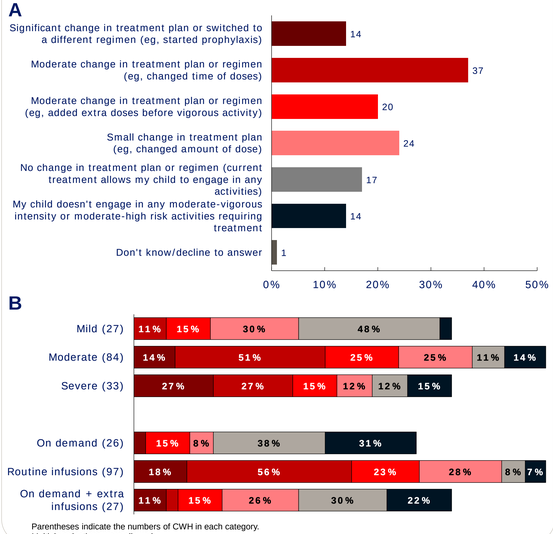
Changes to treatment around activity

- Changes to treatment regimen around activity to allow participation in particular recreational activities, including sports, are shown in **Figure 5**.
- More than half of CWH reported significant or moderate changes to treatment to accommodate activities.
  - Changes were most common/significant in severe and moderate hemophilia, yet many CGs of children with mild hemophilia reported changing treatment around activity.
  - Those currently treated with routine infusions reported more treatment changes than those treated on demand for bleeding, suggesting that the reason for routine infusions may be related to activity participation.

Conclusions

- B-HERO-S provides new insights into the impact of hemophilia on activities in CWH; large and/or moderate impact was seen in children with mild/moderate/severe hemophilia.
- While most CWH were not engaging in high-risk activities, the extent of participation (weekly hours, vigorousness) suggests active engagement in lower-risk activities.
- Moderate to significant treatment changes, including use of routine infusions, were reported among CWH, including those with mild-moderate hemophilia.
- Taken with recent epidemiologic data on risk of bleeding, these data suggest that treatment and/or treatment modification is needed to accommodate CWH participation in even normal (low/moderate risk) childhood activities.

Figure 5 Changes to treatment regimen around activity, overall (A) and by hemophilia severity and treatment regimen (B)



Parentheses indicate the numbers of CWH in each category.
Multiple selections were allowed.

Conflict of interest disclosure

GH has received grant/research support from Bayer and Novo Nordisk and has served on advisory boards for Biogen Idec and on the speakers bureau for Emergent BioSolutions. KB has served as a consultant for Bayer and Novo Nordisk and as a speaker for Baxter, Bayer, and Novo Nordisk. CG is on nursing advisory boards of Baxter/Baxalta, Biogen Idec, Grifols, Novo Nordisk, and Octapharma and is on speakers bureaus for Baxalta, Novo Nordisk, and Solution Sight. SC has received honoraria from Novo Nordisk and Solution Sight. MF has been on advisory boards for Baxalta, Bayer, Kedrion, Novo Nordisk, Octapharma, and Pfizer and on speakers bureaus for Baxalta and Novo Nordisk. SD has nothing to declare. DR has a son with hemophilia B. DC has nothing to declare. CE and DLC are employees of Novo Nordisk Inc.

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2. Forsyth AL et al. *Patient Prefer Adherence.* 2015;9:1549-1560.

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