Association between Health Status and Consumption of Factor IX Concentrate among Persons with Hemophilia B

Hemophilia Utilization Group Study - Part Vb (HUGS Vb)

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**Disclosures for:** Xiaoli Niu

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<table>
<thead>
<tr>
<th>CONFLICT</th>
<th>DISCLOSURE — IF CONFLICT OF INTEREST EXISTS</th>
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* European Accreditation Council for Continuing Medical Education
Introduction

- Due to the availability of the regular factor replacement therapy, significant improvements in treatment outcomes have been observed, including less frequent bleeding, less pain and less arthropathy [1]

- Improvements in treatment outcomes may influence patients’ social activity and quality of life [2]

- Although the reported factor IX use varies across national economics, there is an overall increase in the amount of factor IX used in the treatment of hemophilia B [3]

- With the data from a multi-center study and patients with varied treatment pattern, we hope to be able to explore the association between the consumption of factor and health status

Objectives

- To describe the consumption of factor IX concentrate among a sample of people with hemophilia B within the United States (US)

- To examine the relationship between health status and factor consumption within this sample from the Hemophilia Utilization Group Study Part Vb (HUGS-Vb)
Methods

Data Source:

• HUGS Vb, a prospective, observational, multi-center cohort study

• Data was collected from 2009 to 2013 among hemophilia B patients from 10 Hemophilia Treatment Centers (HTCs) caring for patients across 11 states in the US

• Collected information included: socio-demographics, disease severity, treatment regimen (prophylaxis or on-demand), bleeding episodes, health status/HRQoL

• Data was either self-reported by adult patients or reported by patient-proxies (parents) for children <18 years of age
Methods

Study Sample:

Inclusion criteria:
- Patients with hemophilia B (factor IX level ≤30%)
- Between 2 and 64 years of age
- Receiving 90% of their hemophilia care at the participating HTCs
- Seen at the HTC within two years prior to the beginning of the study
- English/Spanish speaking

Exclusion criteria:
- Patients with an inhibitor during the study period
- Patients without any dispensing records during the study period
- Patients enrolled in clinical trials for any long-acting products
Methods

Outcome Assessment Measures:

• Health status was assessed using the utility scores derived from the EQ-5D index and VAS at the initial interview (baseline) and at one-year follow-up (higher scores indicate better health)

• Weekly factor consumption was estimated from one-year factor dispensing records

Statistical Analysis:

• Based on the observed factor consumption (mean consumption: 48.9±54.7 IU/kg/week), patients were categorized into one of the following two groups:

  ➢ Mean factor consumption ≥50 IU/kg/week
  ➢ Mean factor consumption <50 IU/kg/week

• Covariates adjusted (ANCOVA) utility scores were used to compare health status between the two groups as well as between baseline and one-year follow-up scores

  ➢ Covariates: age, race, education, severity, treatment regimen, bleeding frequency and factor consumption level

• Propensity score matching was also conducted and the results were compared. Only the results from the covariates adjusted analysis are reported here as the results were similar between two methods.
Results

Table 1: Patient characteristics at the beginning of the study

<table>
<thead>
<tr>
<th>Variables</th>
<th>Total (N=96)</th>
<th>Factor consumption &lt;50 IU/kg/week (N=60)</th>
<th>Factor consumption ≥50 IU/kg/week (N=36)</th>
<th>P value</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Weekly factor consumption, IU/kg</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mean (SD)</td>
<td>48.9 (54.7)</td>
<td>13.0 (12.1)</td>
<td>106.2 (46.8)</td>
<td></td>
</tr>
<tr>
<td>Range</td>
<td>0.5-267.3</td>
<td>0.5-42.4</td>
<td>51.1-267.3</td>
<td></td>
</tr>
<tr>
<td><strong>Age group, N (%)</strong></td>
<td></td>
<td></td>
<td></td>
<td>0.01</td>
</tr>
<tr>
<td>Children</td>
<td>52 (54)</td>
<td>26 (43)</td>
<td>26 (72)</td>
<td></td>
</tr>
<tr>
<td>Adults</td>
<td>44 (46)</td>
<td>34 (57)</td>
<td>10 (28)</td>
<td></td>
</tr>
<tr>
<td><em><em>Education &gt;12 years</em>, N (%)</em>*</td>
<td>69 (72)</td>
<td>48 (80)</td>
<td>21 (58)</td>
<td>0.02</td>
</tr>
<tr>
<td><strong>Race, N (%)</strong></td>
<td></td>
<td></td>
<td></td>
<td>&lt;0.01</td>
</tr>
<tr>
<td>White</td>
<td>63 (66)</td>
<td>48 (80)</td>
<td>15 (42)</td>
<td></td>
</tr>
<tr>
<td>Others*</td>
<td>33 (34)</td>
<td>12 (20)</td>
<td>21 (58)</td>
<td></td>
</tr>
<tr>
<td><strong>Disease severity, N (%)</strong></td>
<td></td>
<td></td>
<td></td>
<td>&lt;0.01</td>
</tr>
<tr>
<td>Mild/Moderate</td>
<td>43 (45)</td>
<td>36 (60)</td>
<td>7 (19)</td>
<td></td>
</tr>
<tr>
<td>Severe</td>
<td>53 (55)</td>
<td>24 (40)</td>
<td>29 (81)</td>
<td></td>
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<tr>
<td><strong>Prophylaxis, N (%)</strong></td>
<td>31 (32)</td>
<td>7 (12)</td>
<td>24 (67)</td>
<td>&lt;0.01</td>
</tr>
<tr>
<td><em><em>Bleeding frequency</em>, mean (SD)</em>*</td>
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<tr>
<td>Prophylaxis</td>
<td>4.52 (5.42)</td>
<td>1.22 (2.52)</td>
<td>5.48 (5.69)</td>
<td>0.01</td>
</tr>
<tr>
<td>On-demand</td>
<td>6.61 (6.78)</td>
<td>5.46 (6.07)</td>
<td>11.68 (7.68)</td>
<td>0.01</td>
</tr>
</tbody>
</table>

*Applies for adult patients or parents of children age <18 years; *Others include: Black, Hispanic, Asian/Pacific Islander, American Indian or Alaskan Native and Others; *Indicates the number of bleeding episodes during the past 12 months.
• Higher factor consumption was observed among persons with severe hemophilia, versus mild/moderate hemophilia, and among those with prophylactic regimen versus on-demand regimen.
Results

- No statistically significant differences were observed on both tools between factor consumption subgroups. A slightly higher VAS scores were observed among those with factor consumption ≥50 IU/kg/week, indicating a better quality of life.

*Vertical lines in figures denote standard deviation.
Discussion/Limitations

• Preliminary information on the association between factor consumption and health status were provided in this study. Factor consumption may not reflect the prescribed dose

• The threshold of factor consumption was based on the distribution of annual factor dispensed within a sample of mild, moderate and severe hemophilia patients

• EQ-5D index score reflects a trade-off utility, while VAS measures a general perception of the health. Patients with factor consumption ≥50 IU/kg/week may feel that they had a better protection while the actual utility may remain unchanged

• The differences in utility scores between subgroups were very small, potential confounder may include treatment history prior to the enrollment between the two groups, which we were unable to control for in this study
Conclusion

- Higher factor consumption (IU/kg/week) was significantly associated with younger age, fewer years of education, being non-white, severe disease, receiving prophylaxis regimen and a higher bleeding frequency in hemophilia B patients.

- Only small differences were found between two factor consumption subgroups in both EQ-5D and VAS among the two factor consumption groups.

- Further assessment of how treatment patterns (prophylaxis vs. on-demand, high dose regimen vs. low dose regimen, high infusion frequency vs. low infusion frequency) impact health status needs to be done.
Acknowledgement

We thank our sponsors: Pfizer (formerly Wyeth) for funding the HUGS Vb project. In addition, we would like to thank Biogen Idec for sponsoring a research fellowship for Xiaoli Niu.

- **The Hemophilia Utilization Group Study Part-Vb (HUGS-Vb)** (ranked by study center ID):

  - **University of Southern California**: Michael B. Nichol, PhD (Principal Investigator), Kathleen A. Johnson, PharmD MPH, PhD (late Principal Investigator), Mimi Lou, MS, Joanne Wu, MS, Zheng-Yi Zhou, PhD, Jiat Ling Poon, PhD, Xiaoli Niu, Jason N. Doctor, PhD;
  - **Children’s Hospital Los Angeles, Hemostasis and Thrombosis Center**: Cathilyn Buranahirun, PsyD (Site Principal Investigator), Robert Miller, PA (former Site Principal Investigator), Jennifer Hanley, Wendy Leung;
  - **Children’s Hospital of Orange County, Hemophilia Treatment Center**: Amit Soni, MD (Site Principal Investigator), Heather Huszti, PhD (former Site Principal Investigator), James Fabella;
  - **University of Colorado Denver**: Brenda Riske, MS, MBA, MPA (Site Principal Investigator), Julie Smith, Kristi Norton;
  - **Indiana Hemophilia & Thrombosis Center, Hemophilia Treatment Center**: Amy Shapiro, MD (Site Principal Investigator), Natalie Duncan, MPH, Melissa Meyer, Neelam Thukral, Brandy Trawinski, Jayme Harvey;
  - **UMASS Memorial Hospital, New England Hemophilia Center**: Ann D. Forsberg, MA, MPH (Site Principal Investigator), Patricia Forand;
  - **University of Texas at Houston, Gulf States Hemophilia and Thrombophilia Center**: Megan M. Ullman, MA, MPH (Site Principal Investigator);
  - **Akron Children’s Hospital Medical Center, Ohio**: Jeffrey Hord, MD (Site Principal Investigator), Erin Cockrell, DO (former Site Principal Investigator), Dawn Ali, Felicia Lewis, Janice Kakish;
  - **Michigan State University, Center for Bleeding and Clotting Disorders**: Roshni Kulkarni, MD (Site Principal Investigator), Cheryl Robins, Sue Adkins, Laura Carlson;
  - **Hemophilia Program, Puget Sound Blood Center, Washington**: Barbara A. Konkle, MD (Site Principal Investigator), Sarah Ruuska, Sarah Galdzicka;
  - **University of Mississippi Medical Center**: Suvankar Majumdar, MD (Site Principal Investigator), Linnea McMillan;

- **The authors thank the HUGS Steering Committee**: Randall G. Curtis, MBA, Shelby L. Dietrich, MD and Marion A. Koerper, MD, Brenda Riske, MS, MBA, MPA, Megan M. Ullman, MA, MPH, Judith R. Baker, DrPH, MHSA, and Consultants Femida Gwadry-Sridhar, BSPhm, MS, PhD and Jason N. Doctor, PhD.