

# Understanding Burden of Illness of People with Hemophilia A with or without a Current Inhibitor

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## BACKGROUND

- The Community Having Opportunity to Influence Care Equity (CHOICE) Project was conducted in partnership between the Centers for Disease Control and Prevention (CDC) and Hemophilia Federation of America (HFA) to survey persons with bleeding disorders (PWBD) in the US, including those who do not receive care at federally-funded hemophilia treatment centers (non-HTC PWBD).
- HFA and CDC collaborated on the development of the CHOICE survey questions with input from members of the bleeding disorders community.
- From April 2013 to July 2015, HFA recruited US PWBD ≥18 years old (adults) and caregivers of children with bleeding disorders for CHOICE through email, social media, print, and word-of-mouth to take an approximately 20-minute survey in English or Spanish, online or on paper.
- Non-HTC PWBD were solicited specifically but others were not excluded from participation.
- Survey elements included diagnosis, treatment regimen and products used, inhibitor status, joint function and disease (invasive orthopedic procedures, use of pain medication), bleeding history, HIV and hepatitis infection and other comorbidities, health services utilization (usual source of care, frequency of care, barriers to regular care, delay of care, emergency room utilization and hospitalizations within the past 12 months), demographics, and patient satisfaction (RAND PSQ-18).<sup>1</sup>
- Participants' status as non-HTC PWBD was determined by algorithm.

## OBJECTIVES

- To retrospectively characterize clinical utilization and bleeding among people with hemophilia A (PwHA) with or without a current inhibitor against FVIII to better understand burdens of illness and how to address them.

## METHODS

- PWBD who took the CHOICE survey and reported single diagnosis of hemophilia A (PwHA) were divided into cohorts of with or without a current inhibitor.
- CHOICE survey metrics of (1) patient characteristics, (2) symptoms and treatments, and (3) care location and physician information were reviewed to gain a better understanding of burden of illness.
- Survey elements analyzed included demographics, diagnosis, treatment regimen and treatment products used, health services utilization (usual source of care, frequency of care, barriers to regular care, delay of care, hospital emergency room [ER] utilization, and hospitalizations within the past 12 months), and patient satisfaction (RAND PSQ-18).
- CHOICE survey participants were only required to respond to survey questions for gender, age, and whether they took the survey for themselves or their child. Questions for other criteria included the multiple response options which may not appear in the figures, including "Prefer not to respond" and providing no response. This leads to different numbers of patients (N's) for each survey question; thus, percentage calculations are unique for each question.
- Descriptive statistical analyses of PwHA were performed based on the chi-square test (p values).
- XLSTAT version 18.07<sup>2</sup> year 2017 and Excel 10<sup>3</sup> were used to compute frequencies for all elements, as well as the Pearson chi-square or Kruskal Wallis test to check for the association of inhibitor status with certain outcome variables.

### ACKNOWLEDGMENTS AND DISCLOSURES

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### REFERENCES

<sup>1</sup> The RAND Patient Satisfaction Questionnaire Short Form (PSQ-18). [https://www.rand.org/health/surveys\\_tools/psq.html](https://www.rand.org/health/surveys_tools/psq.html)

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## RESULTS

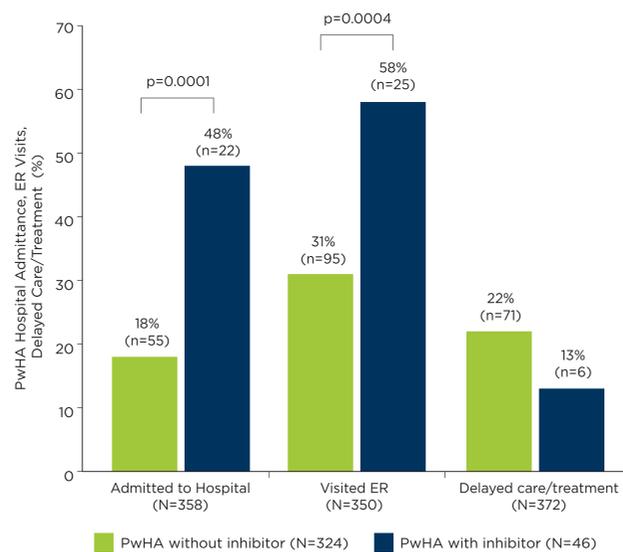
### Patient population

- Of 439 PwHA, 57 (13%) are non-HTC PwHA and 11% had a current inhibitor.

### Hospitalization and ER visits

- In the last 12 months, 48% PwHA with an inhibitor and 18% PwHA without an inhibitor were admitted to a hospital (p=0.001), and 58% of PwHA with an inhibitor and 31% of PwHA without an inhibitor visited an ER (p=0.0004) due to their bleeding disorder (**Figure 1**).
  - PwHA with an inhibitor had significantly more ER visits than PwHA without an inhibitor (3.7 vs. 2.8, p=0.004).
- In the same period, delayed care was reported in 13% of PwHA with an inhibitor vs. 22% PwHA without an inhibitor (**Figure 1**).

**Figure 1. Hospital admittance, ER visits, delayed care/treatment for PwHA with or without a current inhibitor\***

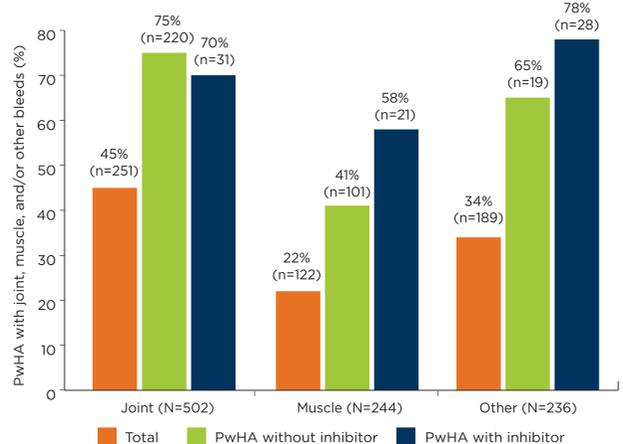


\*Response options to questions about hospital admittance, ER visits, and delays in care and treatment were yes, no, or not applicable.

### Bleed types

- 70% of PwHA with an inhibitor and 75% of PwHA without an inhibitor had a joint bleed, 58% of PwHA with an inhibitor and 41% of PwHA without an inhibitor had a muscle bleed, and 78% of PwHA with an inhibitor and 65% of PwHA without an inhibitor had another type of bleed (**Figure 2**).

**Figure 2. Bleed types in past 12 months for PwHA with or without a current inhibitor\***



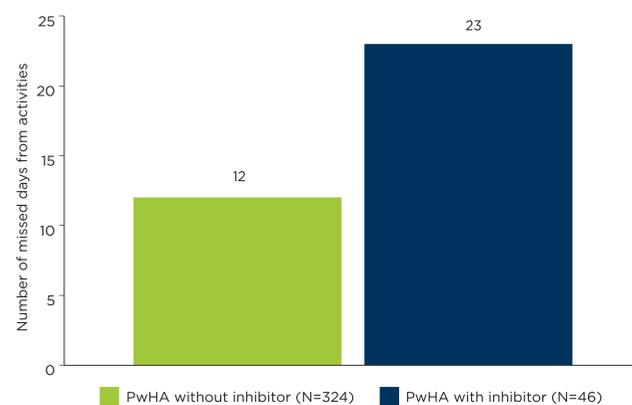
\*More than one response was allowed.

- The average of number of bleeds in a 12-month period was 7.6 for PwHA with an inhibitor and 5.3 for PwHA without an inhibitor.
- 19% of PwHA with an inhibitor and 26% of PwHA without an inhibitor always had joint problems (e.g. pain, stiffness, loss of motion, or weakness), which limited daily activities, like work, school, or recreational activities in 27% of PwHA with an inhibitor and 16% of PwHA without an inhibitor.

### Absence from work, school, or usual activities

- In a 12-month period, the average number of days missed from work, school, or usual activities because of the bleeding disorder were 12 days for PwHA without an inhibitor and 23 days for PwHA with an inhibitor (**Figure 3**).

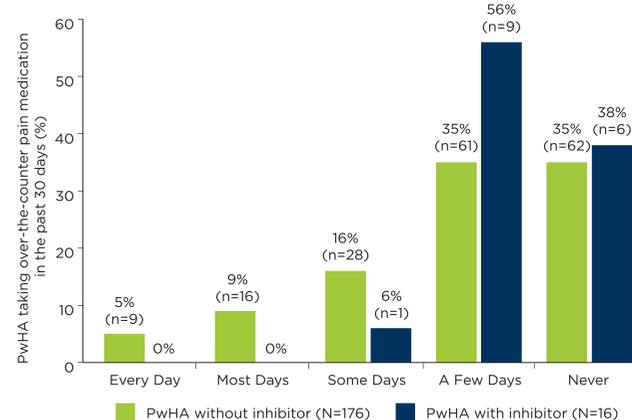
**Figure 3. Average number of days PwHA with or without a current inhibitor missed activities in a 12-month period due to bleeding disorder**



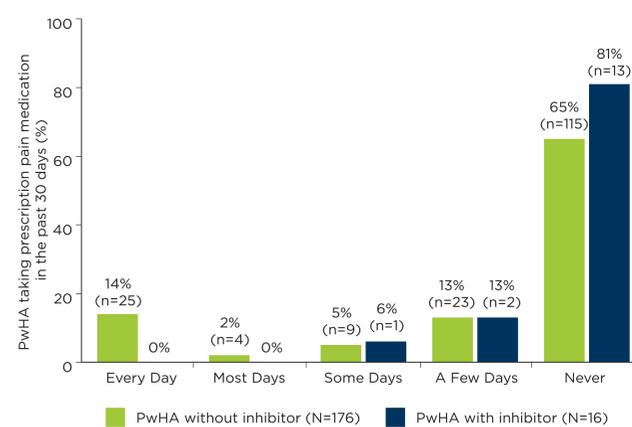
### Pain treatment

- Participants reported frequency of use of pain treatment in a 30-day period as every day, most days, some days, few days, or never.
- 56% of PwHA with an inhibitor and 35% of PwHA without an inhibitor took over-the-counter medication on a few days (**Figure 4**), and 13% of PwHA with an inhibitor and 13% of PwHA without an inhibitor took prescription pain medication on a few days (**Figure 5**).

**Figure 4. Use of over-the-counter pain medication by PwHA with or without a current inhibitor over a 30-day period**



**Figure 5. Use of prescription pain medication by PwHA with or without a current inhibitor over a 30-day period**



## CONCLUSIONS

- Burden of illness for PwHA with an inhibitor is reflected in hospital admittance and emergency care in addition to the types and number of bleeds.
- Additional burden in PwHA with an inhibitor is reflected in delay of care, joint problems that impact regular daily activities, and use of both over-the-counter and prescription pain medication.
- Additional analyses are needed to investigate the disease burden associated with different treatment regimens for PwHA.
- This sample may not necessarily represent all PwHA, as targeted outreach in some regions may have led to over-representation of some participant characteristics.