

# A real-world survey of healthcare resource utilisation, treatment usage, and unmet need for haemophilia patients in Spain

Authors: Sheena Thakkar<sup>1</sup>, Carmen Peral<sup>2</sup>, Irene Francisco<sup>2</sup>, Patricia Tarilonte<sup>2</sup> Rabiyah Sahar<sup>3</sup>, Chris Blazos<sup>3</sup>, Sophie Lai<sup>3</sup>, Ella Morton<sup>3</sup>, Nathan Ball<sup>3</sup>

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<sup>1</sup>Pfizer Inc <sup>2</sup>Pfizer SLU, Spain <sup>3</sup>Adelphi Real World, Bollington, UK

## Introduction

Haemophilia is an inherited bleeding disorder where defects in clotting factor VIII cause haemophilia A (HA) and defects in clotting factor IX cause haemophilia B (HB). These coagulation deficiencies result in bleeds and joint issues<sup>1</sup>.

Treatment is received by people with haemophilia (PwH) prophylactically or on-demand and includes standard-half life (SHL) or extended-half life (EHL) factor therapies as well as non-factor therapies (NFTs)<sup>2</sup>.

Despite treatment, PwH frequently have high healthcare resource utilisation (HCRU) due to persisting bleeding complications and joint-related health issues<sup>3</sup>.

Further data is needed to examine the unmet need among haemophilia patients in terms of treatment utilisation, treatment satisfaction and efficacy in the reduction of HCRU.

## Methods

Data were drawn from the Adelphi Real World Disease Specific Programme (DSP)<sup>TM</sup>, a cross-sectional survey with retrospective data collection of physicians and male HA and HB patients in Spain (September 2023-April 2024).

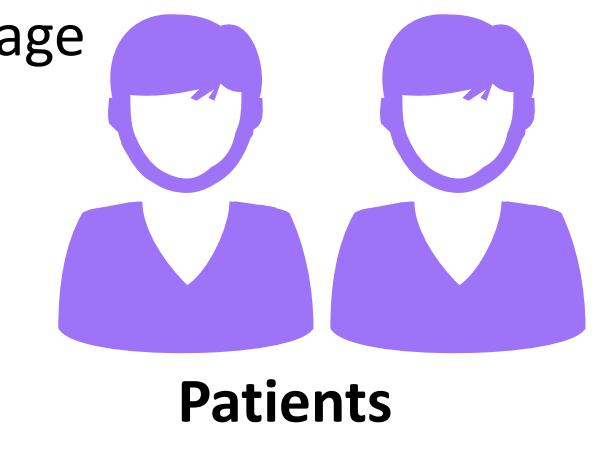
Analyses were descriptive.

DSP<sup>TM</sup> are cross-sectional surveys with retrospective data collection of a geographically representative sample of physicians, a methodology which has been published and validated (4,5,6,7).

### Electronic record forms

Completed by physicians, capturing:

- Demographics including type of healthcare insurance coverage
- Clinical characteristics including bleeds and mortality risk
- SHL/EHL/NFT treatment patterns and adherence
- Haemophilia related consultations and HCRU
- Clinical unmet needs
- Caregiver support



- Confirmed haemophilia A or B diagnosis
- Male
- Baseline clotting factor <5.0%

In Spain, 36 physicians provided data for 183 patients (83% (n=151) HA, 17% (n=32) HB)

## Objective



To describe healthcare resource utilisation (HCRU), treatment usage, and remaining unmet need among male haemophilia patients in Spain.

## Conclusions



Nearly half of PwH in Spain had bled in the 12 months preceding the survey, and/or had joint problems



High numbers of consultations and test usage was reported, resulting in burden for patients.



Future therapies should address the need for supportive medication and clinical unmet need which in turn could reduce HCRU.

## Limitations

- Physicians completed surveys for their next consecutively consulting patients, meaning more frequently consulting patients and those with a more severe disease state are more likely to be captured within the DSP<sup>TM</sup>.
- As data was reported retrospectively, there is a possibility of recall bias; this was reduced by collecting data at earliest time point following patients' consultation.
- Physician participation was determined by the DSP<sup>TM</sup> inclusion criteria, was voluntary and was influenced by respondents willingness to participate.

Presenting author: Sheena Thakkar



Email for more information  
Nathan.Ball@omc.com



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## Acknowledgements:

- Data was collected by Adelphi Real World via the Haemophilia III Disease Specific Programme<sup>TM</sup>, an independent survey whereby all data are the intellectual property of Adelphi Real World. Pfizer Inc. subscribed to access this data source.
- Adelphi Real World and Pfizer Inc. would like to thank the physicians that participated in this survey.

## Disclosures:

- ST is an employee of Pfizer Inc., New York, United States of America.
- CP, IF and PT are employees of Pfizer SLU, Madrid, Spain.
- CP, IF and PT have Pfizer stocks and stock options
- NB, EM, SL, CB and RS are employees of Adelphi Real World, Bollington, United Kingdom.

## References:

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## Results

Table 1. Patient characteristics

|   | Overall (n=183)  | HA (n=151)       | HB (n=32)        |
|---|------------------|------------------|------------------|
| Age (years), median (IQR)   | 23.0 (17.0-40.0) | 23.0 (17.0-40.0) | 25.0 (18.3-41.0) |
| ≥12 years old, n (%)  | 154 (84.2)       | 127 (84.1)       | 27 (84.4)        |
| Inhibitor status, n (%)   |                  |                  |                  |
| No inhibitors at time of survey   | 164 (89.6)       | 133 (88.1)       | 31 (96.9)        |
| Inhibitors at time of survey  | 19 (10.4)        | 18 (11.9)        | 1 (3.1)          |
| Number of tests to monitor, mean (SD)   | 6.8 (2.95)       | 6.7 (3.06)       | 7.2 (2.42)       |
| Types of test to monitor (top 3 overall), n (%)   |                  |                  |                  |
| Complete Blood Count  | 162 (88.5)       | 134 (88.7)       | 28 (87.5)        |
| Activated partial thromboplastin time   | 148 (80.9)       | 120 (79.5)       | 28 (87.5)        |
| Prothrombin time  | 118 (64.5)       | 95 (62.9)        | 23 (71.9)        |
| Number of times any physician was seen for the patient's haemophilia in the last 12 months, mean (SD) | 14.4 (34.06)     | 12.5 (23.60)     | 23.6 (63.34)     |

HA=Haemophilia A, HB=Haemophilia B, IQR= Interquartile range, SD=Standard deviation

- In the 12 months prior to survey, PwH had a mean (SD) 14.4 (34.06) consultations with any physician specialty; with a mean (SD) 6.8 (2.95) test/assessments used to monitor patient's haemophilia since diagnosis.
- Physician-reported prophylactic treatments received by PwH at time of survey and treatment satisfaction for these patients are summarized in Figures 1 and 2.
- At the time of survey, 90% of patients received prophylactic therapy (HA: 38% SHL, 28% EHL, 26% NFT; HB: 56% EHL, 19% SHL); of which 79% were also prescribed on-demand therapy.
- Physicians reported that they weren't 'completely satisfied' for 49% of PwH's prophylactic therapy.

Figure 1. Physician-reported prophylactic treatment at time of survey

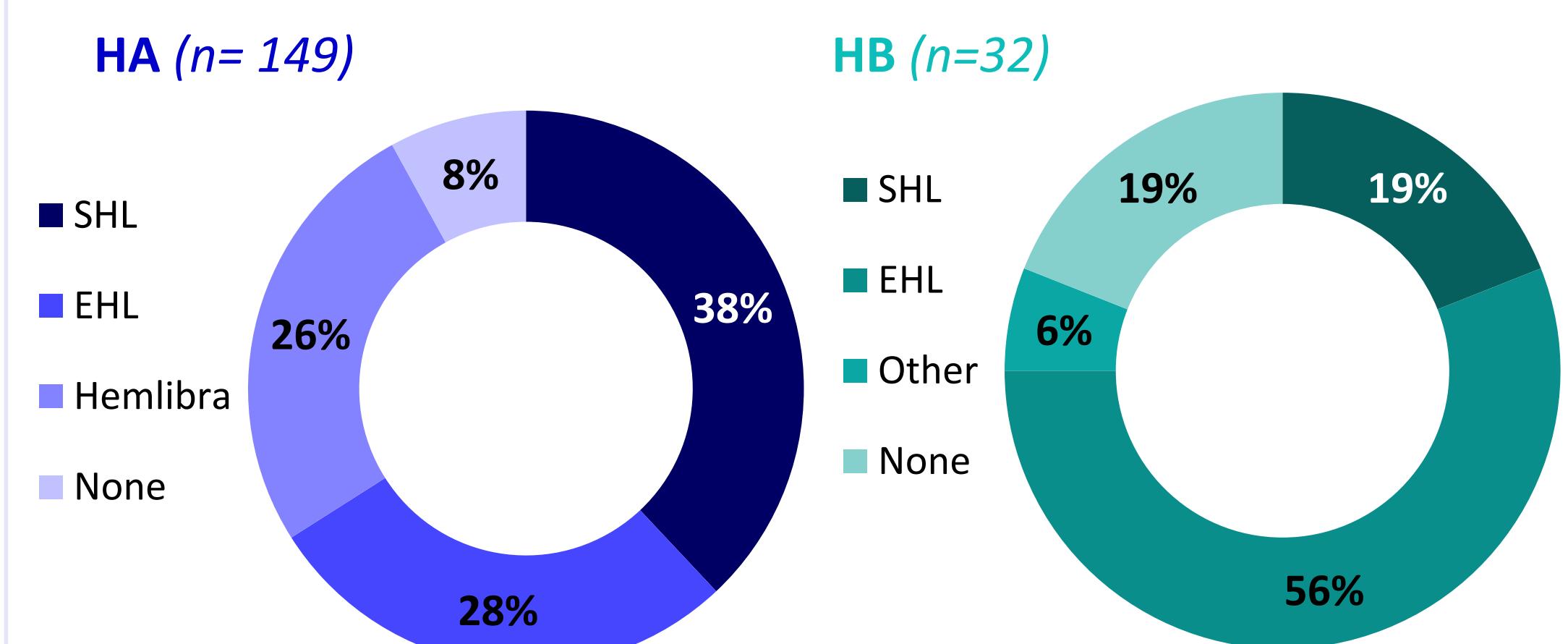


Figure 2. Physician-reported satisfaction with prophylactic treatment (proportion of patients)

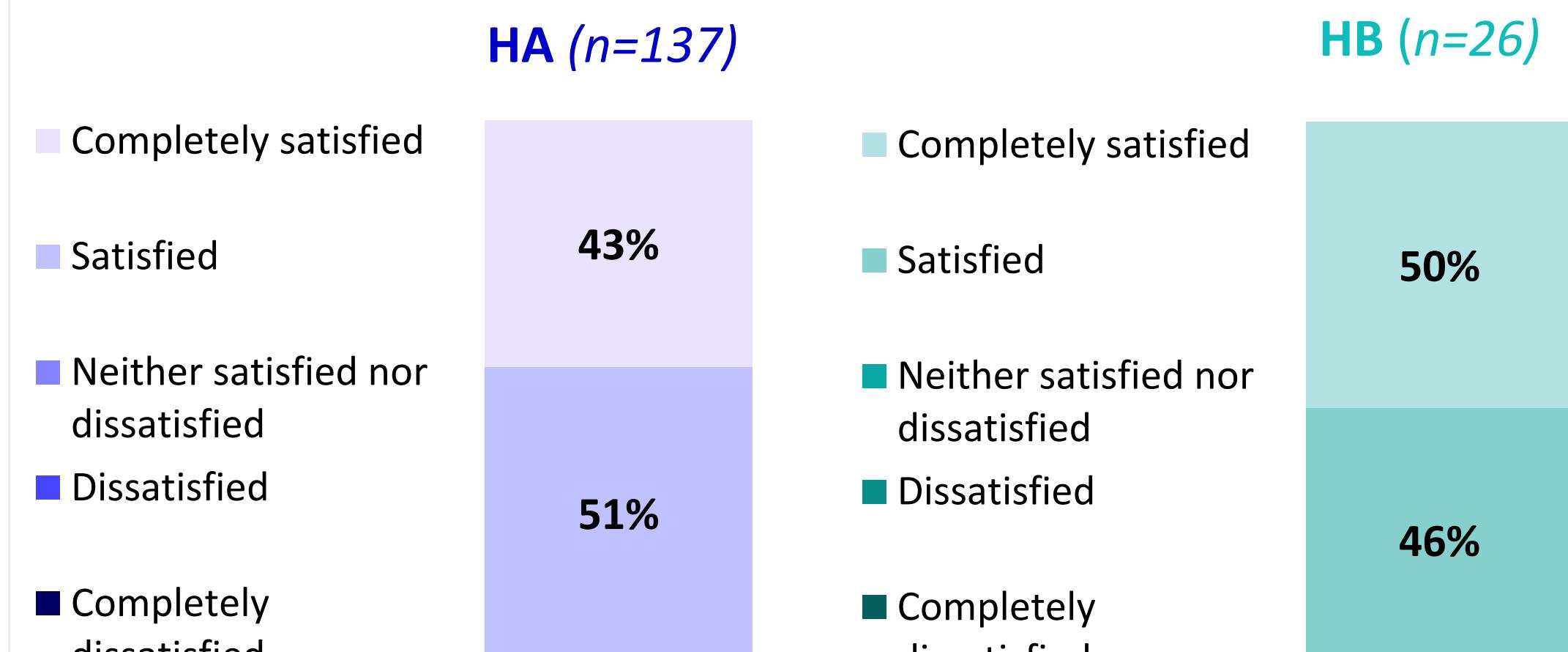
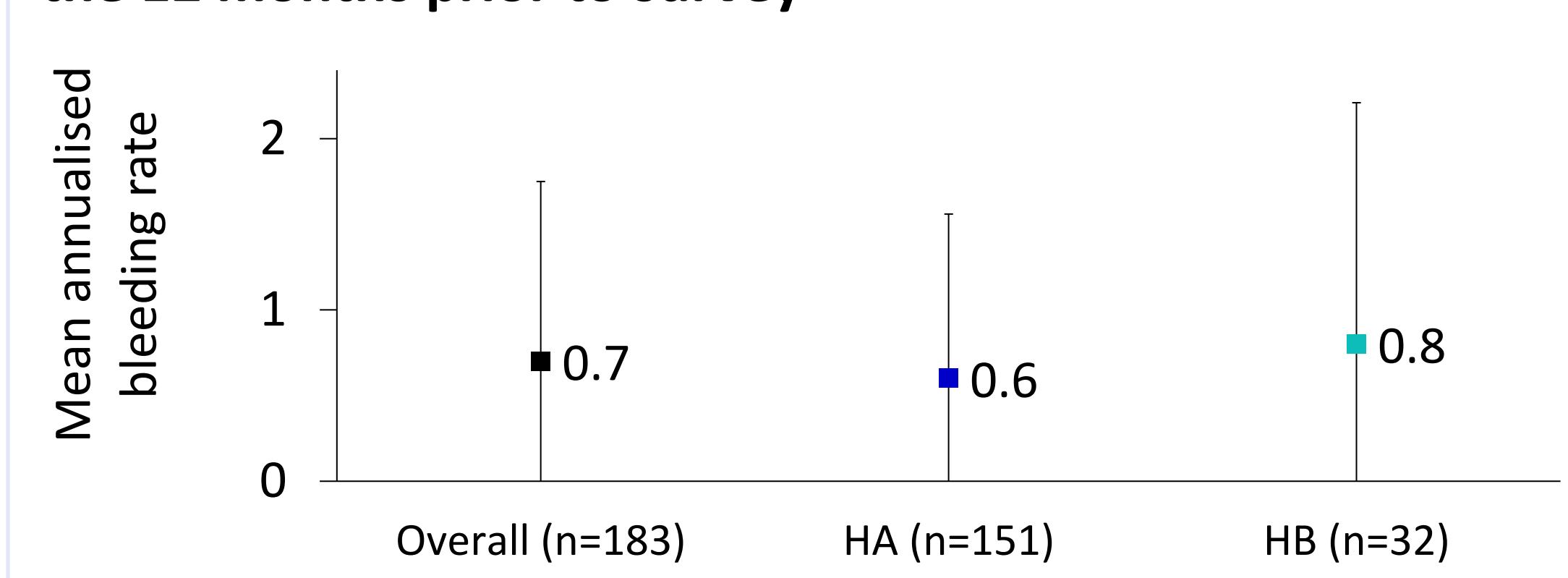


Figure 3. Physician-reported mean annualised bleed rate in the 12 months prior to survey



- Physician-reported bleeds experienced by PwH in the 12 months prior to survey are shown in Figure 3.
- In the 12 months prior to survey, PwH had a mean (SD) 0.7 (1.05) bleeds, with 40% experiencing ≥1 bleed.
- Physician-reported joint problems experienced by PwH at any time point, and joint health status at time of survey are summarized in Figures 4a and 4b, respectively.
- Of the 42% of PwH who had suffered from joint problems, 58% were reported to have a target joint.
- Ahead of a physical activity, 59% of patients took preventative measures, most commonly administering a dose of factor (32%).

Figure 4a. Physician-reported history of joint problems

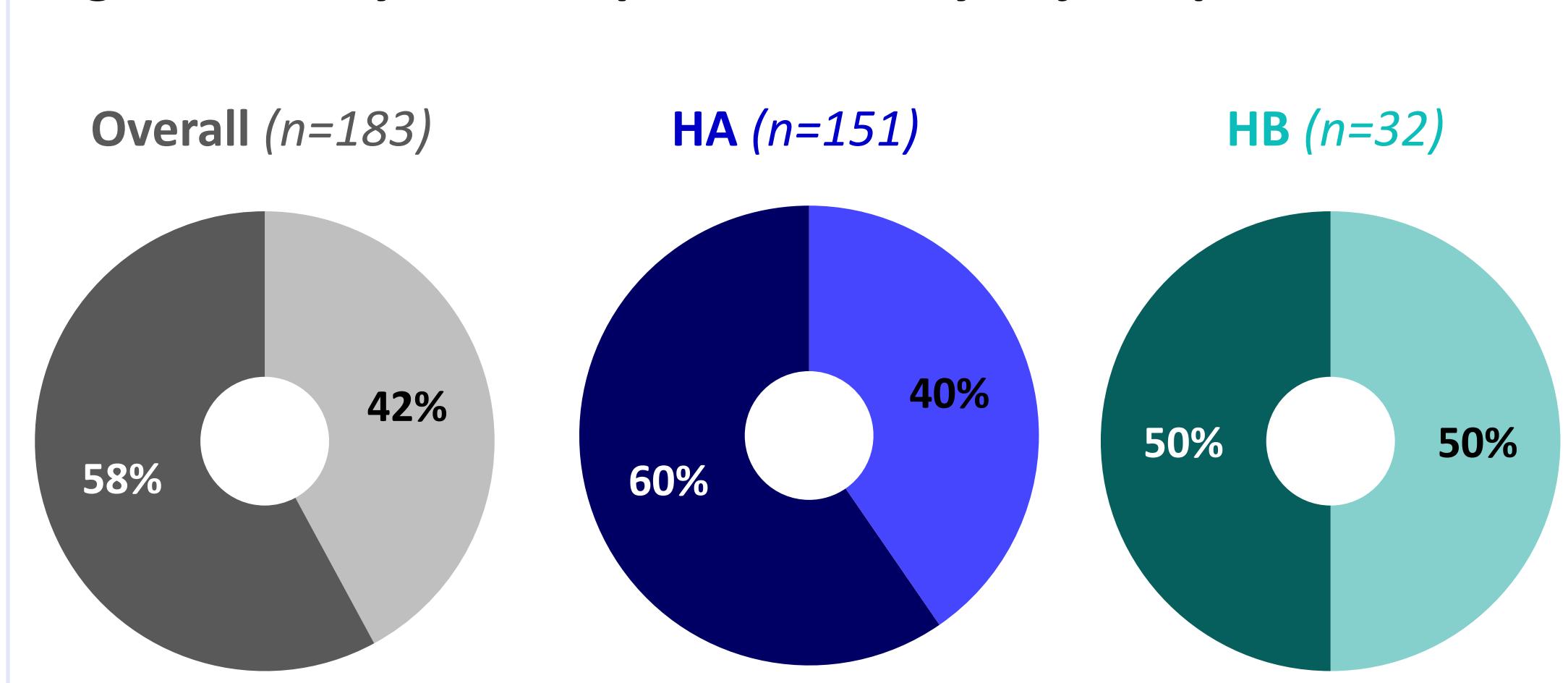


Figure 4b. Physician-reported severity of joint health problems

