PO314

# Treatment Preferences in People with Severe Haemophilia A: a Discrete Choice Experiment in the United States

W. Miesbach[1]; L. A. Valentino[2]; B. O'Mahony[3]; K. Forsyth[4]; M. Bullinger[5]; JM Gonzalez[6]; E. Dashiell-Aje[7]; S. Santos[8]; H.B. Lewis[9]; D. Latibeaudiere-Gardner[9]; T. Tervonen[10]; M. Karimi[8]

[1]Department of Haemostasis/Haemophilia Centre, Medical Clinic 2, University Hospital Frankfurt, Germany; [2]National Bleeding Disorders Foundation, New York, USA; [3]Irish Haemophilia Society, Dublin, Ireland; [4]The Royal London Hospital, London, UK, [5]Independent, Hamburg, Germany; [6]Duke University School of Medicine, Durham, USA; [7]BioMarin Pharmaceutical Inc. San Raphael, USA; [8]BioMarin Pharmaceutical Inc. London, UK; [9]ICON plc, Reading, UK; [10]Kielo Research, Zug, Switzerland

## Background

- The evolving severe haemophilia-A treatment landscape with new modalities such as gene therapy requires understanding the aspects of treatment that drive patients' treatment choices.

- Understanding how people with severe Haemophilia-A (PwSHA) value aspects of treatments is critical in assessing the role of new therapies for severe haemophilia A.

- This study assesses the treatment preferences of PwSHA using a discrete choice experiment (DCE).

## Methods

- Six attributes and their levels were developed based on qualitative interviews[1] and input from a steering committee of experts in haemophilia and preference research (Table 1).

- PwSHA, recruited via patient organizations in the United States, self-reporting severe haemophilia-A were invited to complete the online DCE.

- Data were analysed using a multinomial-logit model (MNL).

  - Results from the MNL are considered preference weights that quantify how preferences for treatments change when the attribute levels vary.

  - Attribute relative importance was calculated as the greatest change in treatment preferences that was obtainable through changes in the study attribute levels. The differences were normalized to add up to 100%.

- The value of a reduction in treatment burden can be expressed as an equivalent to reduction in treated bleeds to better understand the value for patients of this attribute.

### Table 1. Attributes and Levels

| Attribute | Levels |
|---|---|
| Treated Bleeds per Year | No change (5 bleeds) \| Reduction to 2 bleeds \| Reduction to 1 bleeds \| Reduction to 0 bleeds |
| Treatment Frequency and Route of Administration | Intravenous injections twice every week (IV BIW) \| Subcutaneous injections once every 4 weeks (SC Q4W) \| Intravenous injection once that lasts for 5 years, then treatment switch (IV Q5Y) \| Intravenous injection once that lasts for 10 years, then treatment switch IV Q10Y) |
| 1st Year Treatment Requirements | Additional clinic visits – once every week for the first 6 months, then once every two weeks for the next 6 months (Visits 1/w for 6m, then 1/2w) \| (Visits 1/w for 6m, then 1/2w AND use of steroids) \| Use of steroids \| No additional requirements |
| Increased Theoretical Risk of Cancer | 0.01% \| 0.1% \| 1% \| 5% |
| 1st Year Treatment Response | Chance of treatment rejection during the 1st year: 1% \| 5% \| 10% |
| Impact on Daily Life | No change in physical activity and worry about bleeds \| Improvement in physical activity \| Improvement in worry about bleeds \| Improvement in limitations/difficulties of physical activity AND improvement in worry about bleeds |

## Results

- A total of 77 PwSHA who completed the survey were included. Most respondents were currently on subcutaneous treatment (Table 2).

- **PwSHA valued a reduction in treated bleeds as the most important attribute** (relative attribute importance: 32%), **but treatment choices were also strongly influenced by non-bleed attributes** in the study (Fig 1).

- **Providing PwSHA with an 'Intravenous injection once that lasts for 5 years'** instead of an 'Intravenous injections twice every week' **is as beneficial as a reduction in 2.5 treated bleeds per year** from baseline (5 bleeds per year) (Fig 2).

- On average, PwSHA prefer 'Intravenous injection once that lasts for 5 years' over 'Subcutaneous injections once every 4 weeks' (Fig 2).

### Table 2. Sample characteristics

| Characteristic | Response |
|---|---|
| Median age (IQR) | 35 (29, 39) |
| Employment Status n (%) | |
| • Employed, retired, or student | 41 (53%) |
| • Unemployed or unable to work | 36 (47%) |
| Number of target joints; n (%) | |
| • 0 | 9 (12%) |
| • 1 | 20 (26%) |
| • 2 | 24 (31%) |
| • >3 | 24 (31%) |
| Treated bleeds in past 12 months; n (%) | |
| • 0-2 | 25 (32%) |
| • 3-5 bleeds | 33 (43%) |
| • >5 bleeds | 19 (25%) |
| Currently on subcutaneous injections treatment; n (%) | 48 (62%) |

Abbreviation: IQR: Interquartile range

**Figure 1. Relative importance of attributes category**
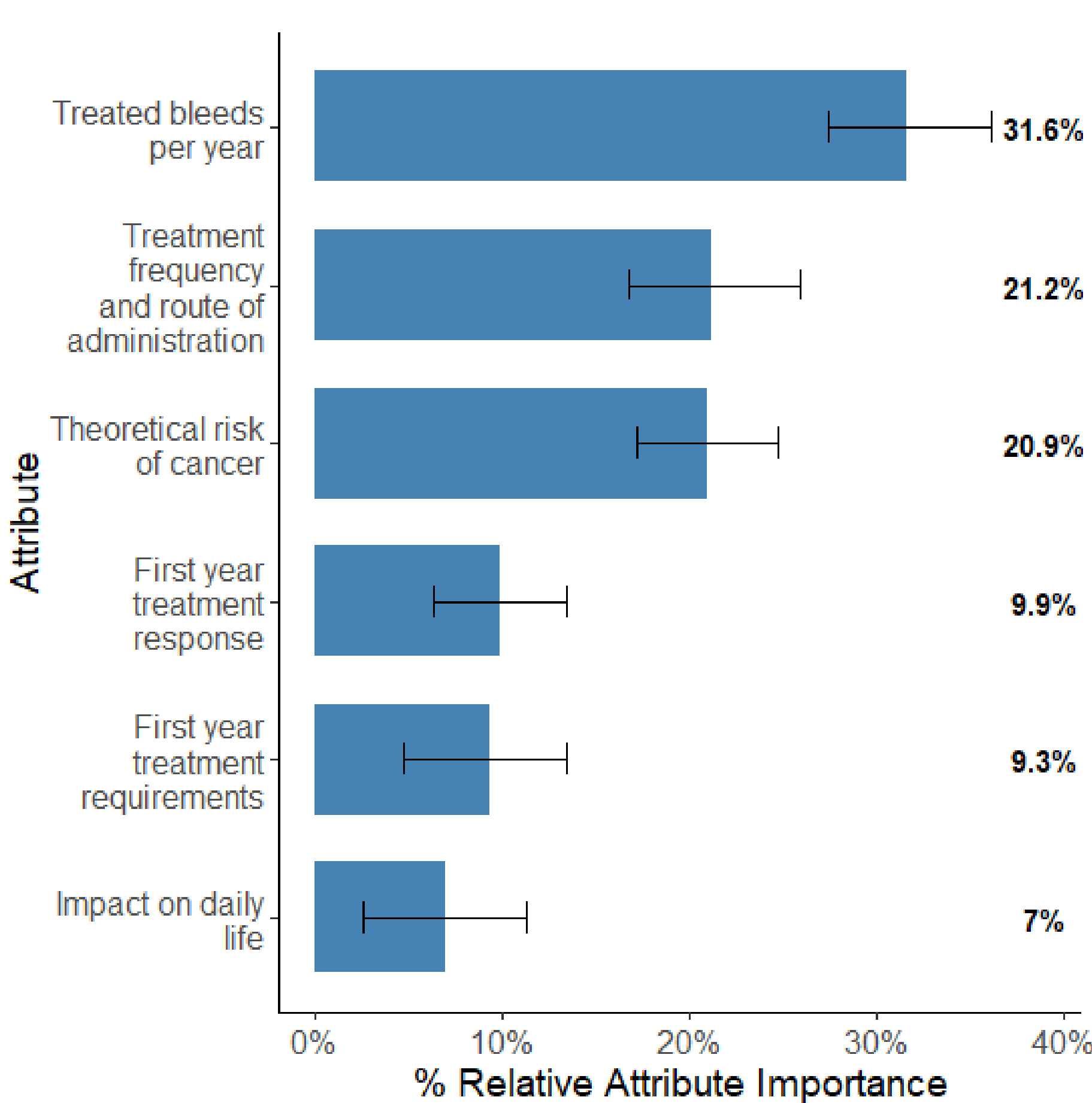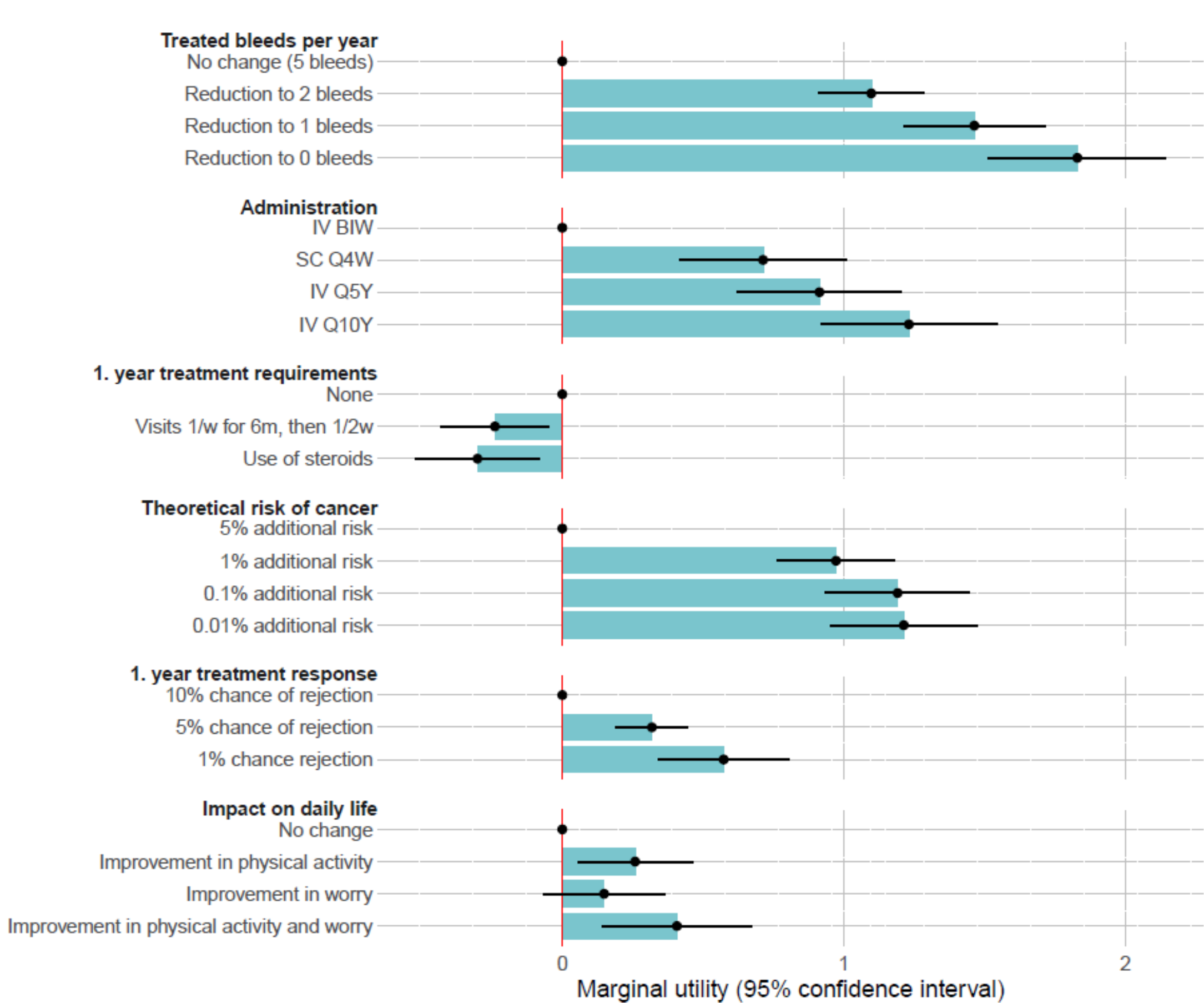


**Figure 2. Preferences for attribute levels**



## Discussion

- This study provides insights which may help inform clinical decision-making.

- Reducing treatment frequency is a key driver for PwSHA, including for those on subcutaneous treatment. On average, PwSHA preferred a one-time IV therapy with 10 or 5-year durability over monthly subcutaneous injections or weekly IV injections.

- The sample used in this study was recruited via patient organizations and cannot be considered fully representative of the patient population. The sample consists of people only in the US. This study is currently being conducted in other geographical regions. Further analyses are planned using a range of statistical models.

## References

Qualitative research evaluating patient preference for haemophilia therapy; Miesbach et al.; EAHAD 2022; Haemophilia. 2022

## Acknowledgements

## Disclosures