National Hemophilia Foundation (NHF) - Posters

Non-severe hemophilia is not benign? - Insights from the PROBE Study

**OBJECTIVES**
- This study aimed to investigate the overall health status of people living with non-severe hemophilia, compared to people without bleeding disorders.

**METHODS**
- Respondents were enrolled through non-government organizations (NGO) working in hemophilia and bleeding disorders.
- Among these, 862 self-reported as having no bleeding disorder (NoBD), or mild or moderate hemophilia, and without history of an inhibitor were included in the analysis.
- Respondents were asked to complete the PROBE questionnaire, which comprise general health domain, hemophilia specific domain and health-related quality of life (HRQoL) domain.
- The primary outcome of the study was to compare the health status and HRQoL of people with NoBD and people living with non-severe (mild and moderate) hemophilia.

**RESULTS**
- Figure 1a. Breakdown of bleeding rates in the past 12 months in people with mild and moderate hemophilia. (Figure 1a. Male, Figure 1b. Female)
- Figure 2. Reported acute and chronic pain by gender

**CONCLUSIONS**
- Majority of respondents with mild and moderate hemophilia did not receive prophylaxis. They reported varied amount of annualized bleeding episodes.
- They reported significant negative impact on pain and HRQoL compared to respondents without bleeding disorders.
- Future research is needed to identify the optimal care management of patients with mild and moderate hemophilia.

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Objective:
There are limited data on the impact of haemophilia on health status and health-related quality of life (HRQL) in people affected by non-severe haemophilia. Aim is to evaluate the health status of people living with mild or moderate haemophilia.

Methods:
A cross-sectional, multinational study was conducted as part of the Patient Reported Outcomes, Burdens and Experiences (PROBE) project. Respondents without bleeding disorder (NoBD) and those with mild or moderate haemophilia were included. Respondents were asked to complete the PROBE questionnaire, which contains haemophilia-related questions, general health questions and HRQL. Results were compared between unaffected individuals and people with mild or moderate haemophilia.

Summary:
A total of 862 respondents, of whom 144 with moderate haemophilia, 143 with mild haemophilia and 575 with NoBD were included. Median age (first-third quartile) was 33 (23-46), 42 (25-55) and 43 (35-54), respectively. In relation to bleeding in the previous 12 months, respondents with mild reported less bleeding frequency than those with moderate haemophilia, with similar patterns of bleeding frequency seen in both male and female cohorts. Reporting of acute pain is less in those with NoBD compared to the mild to moderate cohorts for both genders (male - 33%, 67%, 77%; female - 38%, 52%, 67%, respectively). Thirteen percent of those with NoBD reported an impact on activities of daily living compared with mild and moderate haemophilia who reported of 35% and 61%, respectively. The impact on quality of life due to mild haemophilia compared to those with NoBD was a reduction of 5.2%, 5.0% and 9.3% in VAS, EQ-5D-5L and PROBE Score respectively (p<0.001).

Conclusions:
People affected by mild or moderate hemophilia encountered a significant amount of haemophilia related sequelae. Future research is needed to identify the optimal management of moderate and mild hemophilia patients, with particular focus on early identification of patients with a severe clinical phenotype.