



Health status of people living with non-severe hemophilia

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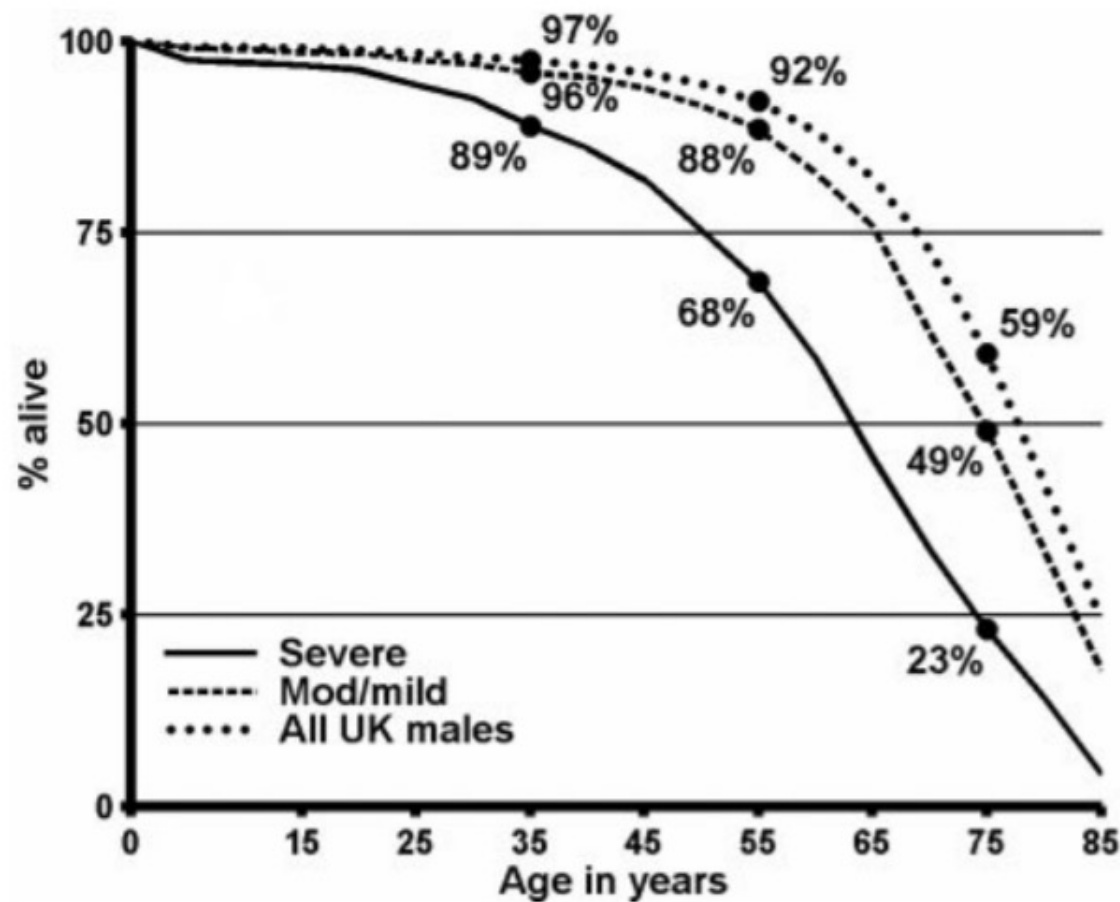
Disclosures

- No potential conflicts of interest

Background

- Non-severe hemophilia
 - Moderate hemophilia: reduced factor level of 1-5 IU/dL
 - Mild hemophilia: reduced factor level of <40 IU/dL
- Clinical manifestations of non-severe hemophilia
 - Rare spontaneous bleeding
 - Excessive bleeding after surgical procedures or trauma

Life expectancy of PWH compared to non-hemophilia males (UK)



Knowledge gap

- Limited data on the health status of people living with non-severe hemophilia

Objective

- To investigate the health status of people living with non-severe hemophilia comparing with people without bleeding disorders

Methods-participant enrollment and data collection

- Participants were enrolled through patient organizations from 2016-2017
- Inclusion criteria
 - PWH (non-severe) and people without bleeding disorders
 - Age > 10 years
 - To be able to complete the questionnaire by themselves
- Study procedure and data collection
 - Participants were instructed to answer the questionnaire either paper- or web-based version
 - All questionnaires were collected by participants organizations then transferred to McMaster University for data management

Methods-PROBE questionnaire

- PROBE is a patient-lead research initiative with the main objective to develop a standardised questionnaire to assess health status in PWH
- PROBE questionnaire (29 questions in 3 major domains)
 - General health problems
 - Hemophilia-related problems
 - Health-related quality of life
- PROBE has been validated in both PWH and participants without bleeding disorders

Chai-Adisaksopha C, et al. BMJ Open 2018;8:e021900.

PROBE items on pain assessment

- During the past 12 months, have you experienced acute pain?
 - ☐ Yes
 - ☐ No

If yes, when did your acute pain occur?
(Please check all that apply)

- ☐ Walking
- ☐ Stair climbing
- ☐ Nighttime (such as waking you up/keeping you awake)
- ☐ Resting
- ☐ Weight bearing
- ☐ Playing (including playing with children) or participating in sports / exercising
- ☐ After falling or a trauma
- ☐ Other (Describe): _____

If yes, does your acute pain interfere with any of the following? (Please check all that apply)

- ☐ General activity
- ☐ Mood
- ☐ Walking ability
- ☐ Normal work (including both work outside the home and housework)
- ☐ Attending school
- ☐ Relations with others
- ☐ Sleep
- ☐ Enjoyment of life
- ☐ Playing (including playing with children) or participating in sports / exercising
- ☐ Lifting
- ☐ Other (Describe): _____

Health-related quality of life

- EQ-5D-5L

Work or school life

- Work or school status
- Sick days per year

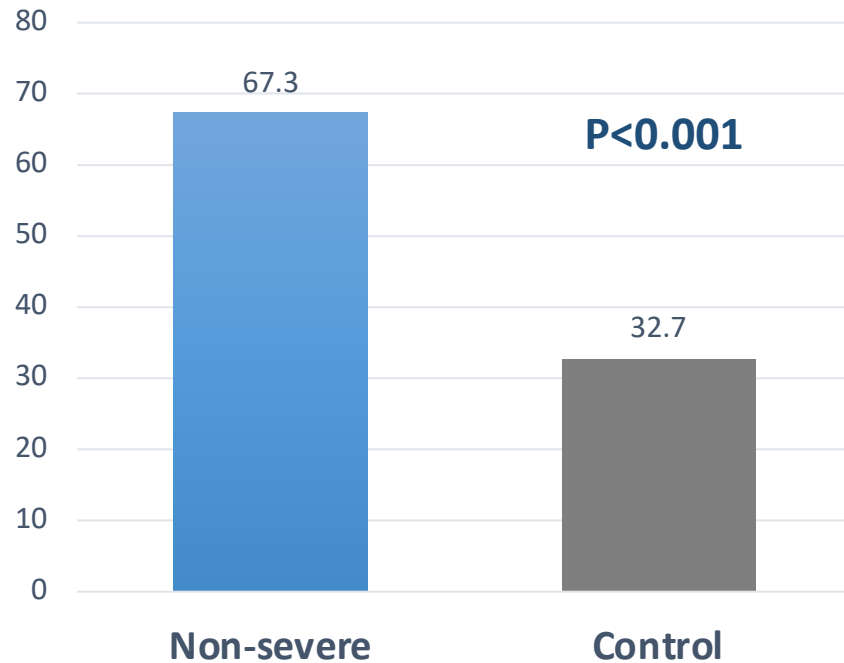
Statistical analysis

- 1:1 age and sex matched
- Descriptive data were reported as a proportion and mean (standard deviation) as appropriate.
- Study outcomes
 - General health problems
 - Health-related quality of life
 - Work and school life

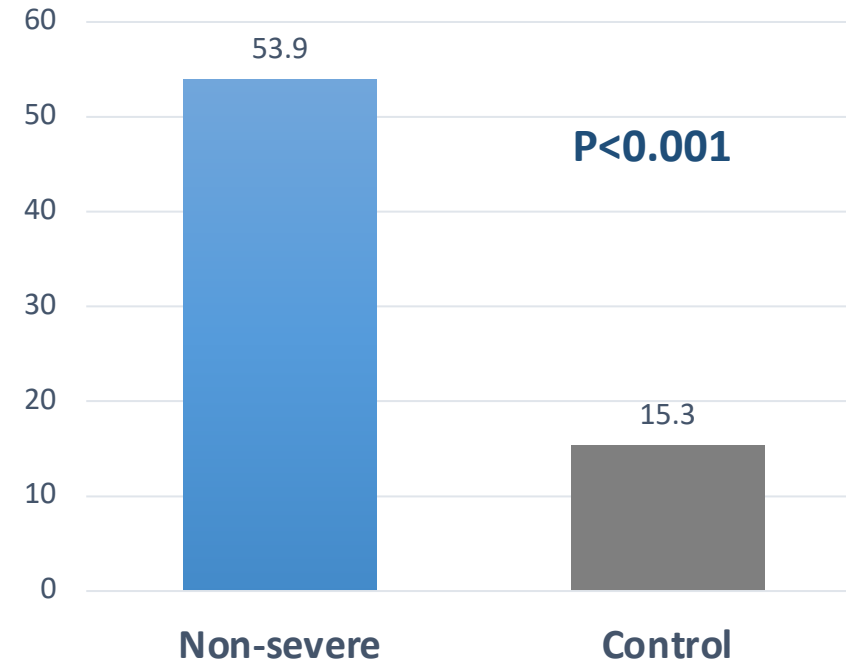
Characteristics of participants

Characteristics	Non-severe hemophilia (n=183)	No bleeding disorder (n=183)
Age (mean, SD)	44.7 (\pm 17.4)	45.7 (\pm 14.4)
Sex (male, %)	153 (83.6)	153 (83.6)
Geographical region, n(%)		
• Africa	2 (1.1)	0 (1.1)
• Western Pacific	48 (26.2)	40 (21.9)
• South America	51 (27.9)	47 (25.7)
• Europe	56 (30.6)	38 (20.8)
• North America	26 (14.2)	58 (31.7)
Disease status		
• Hemophilia A	144 (78.7)	N/A
• Hemophilia B	39 (21.3)	

Health related problems

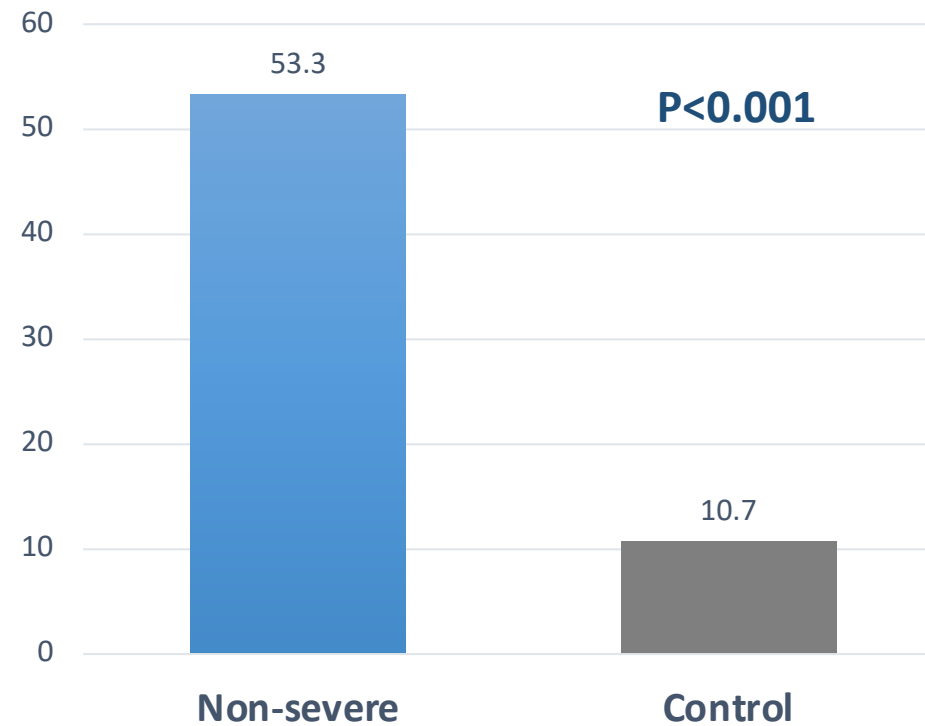


Experiencing problems related to health

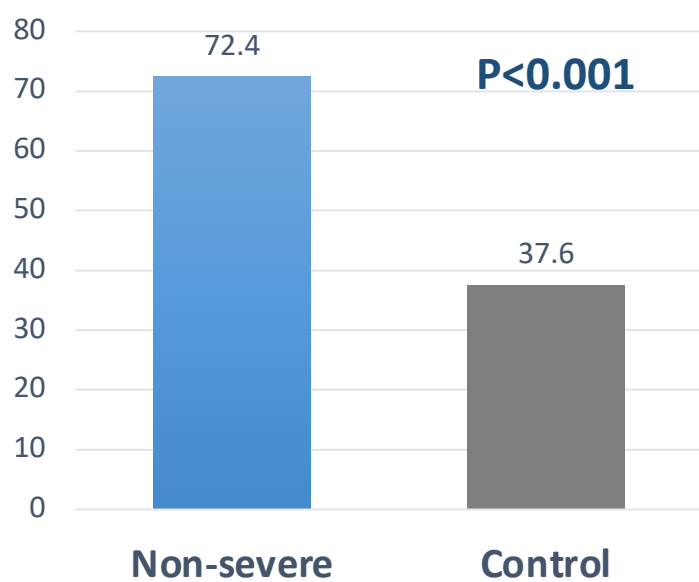


Using mobility aids

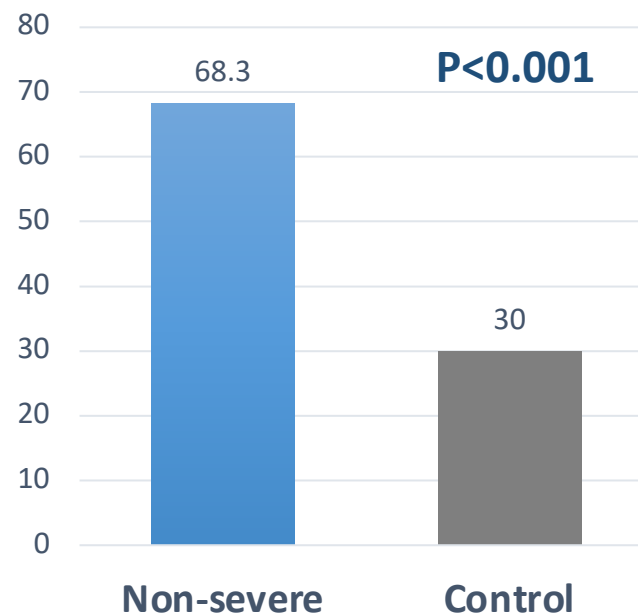
Difficulty with activities of daily living



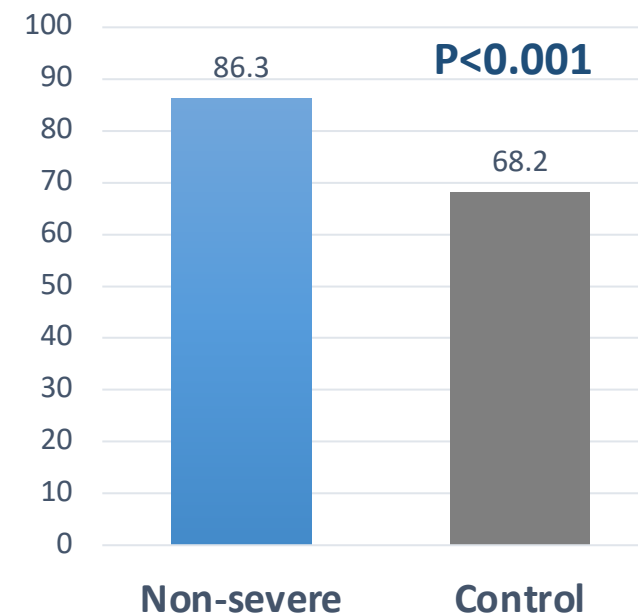
Health related problems



Acute pain

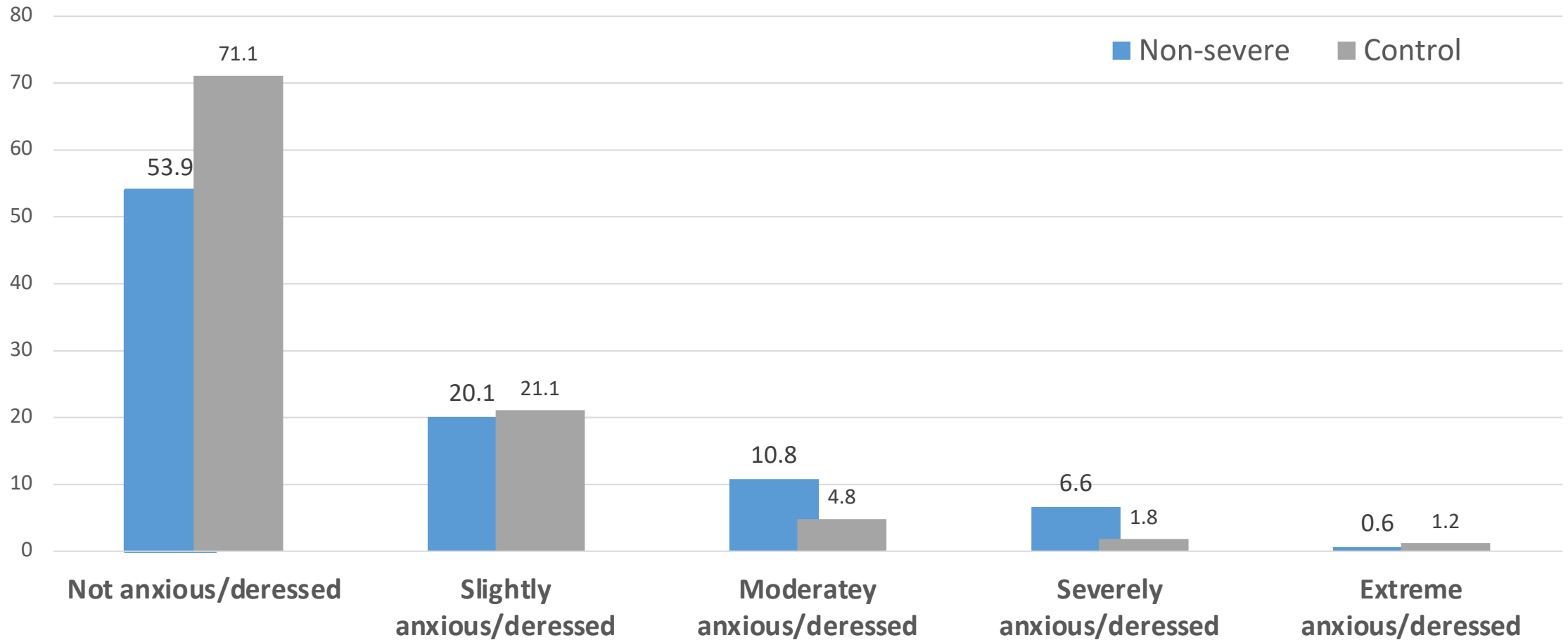


Chronic pain

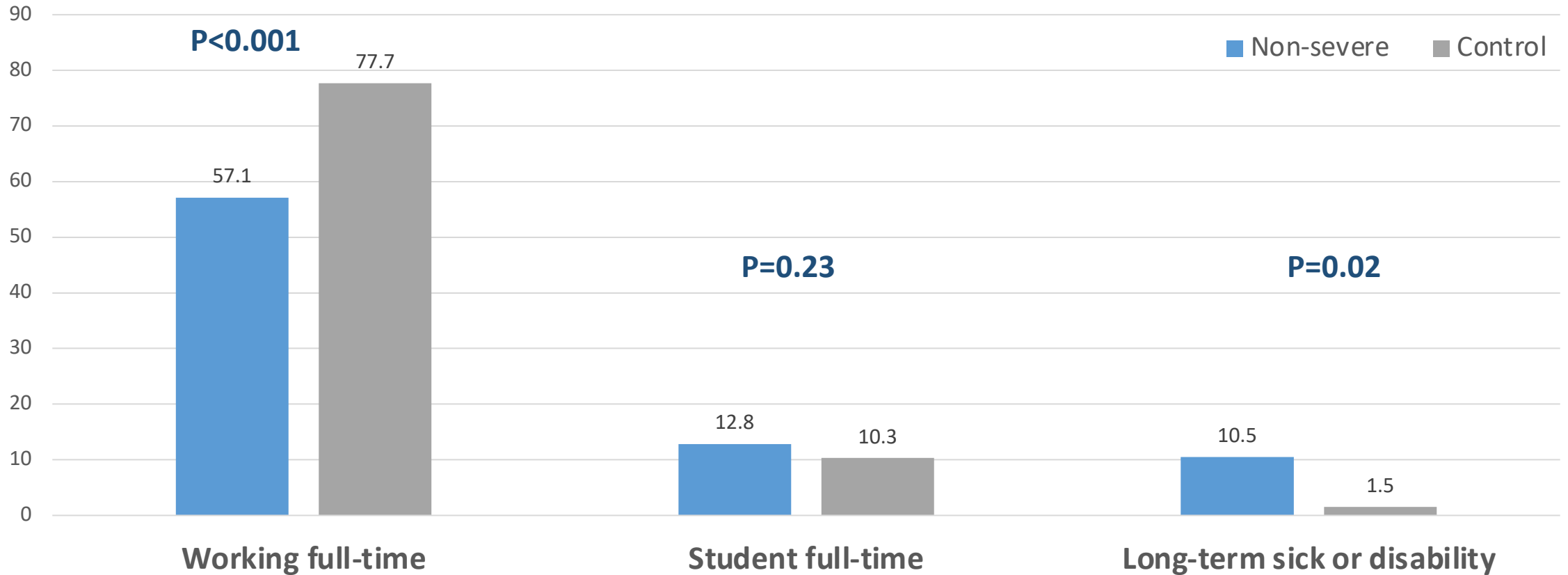


Use of pain medications

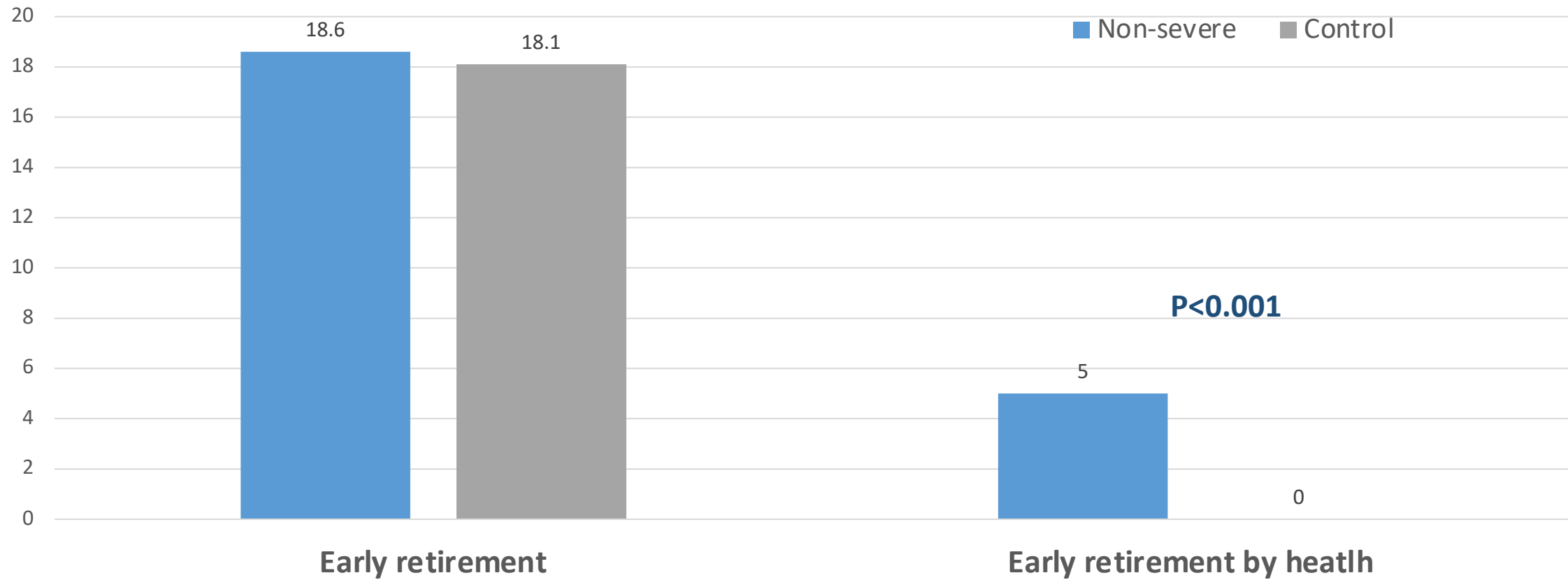
EQ-5D-5L on anxiety/depression (%)



Work and school life



Work and school life

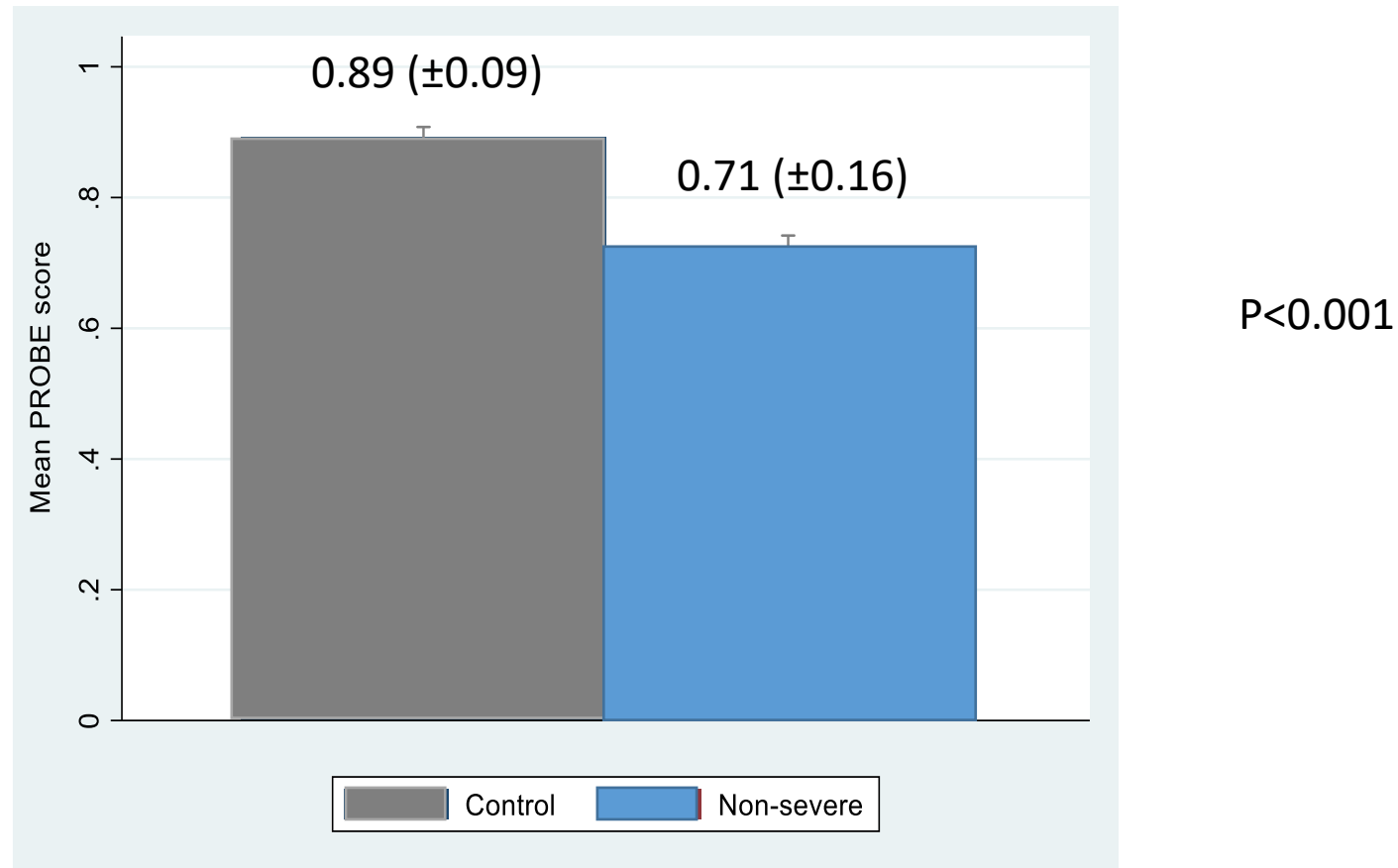


Work and school life (continued)

- Sick days per year

Sick days per year	Non-severe hemophilia (n=183)	No bleeding disorder (n=183)	P-value
Median (interquartile range)	7 (0-25)	0 (0-3)	<0.001
Mean (SD)	44.9 (8.7)	3.7 (1.5)	<0.001

Overall health status assessed by PROBE score (Higher score indicates better health status)



Conclusions

- Compared to participants without bleeding disorders, people with non-severe hemophilia had much worse health status
 - Health-related problems
 - Work and school life
 - Health-related quality of life
- Non-severe hemophilia is not a benign disease.

Conclusions

- Clinical outcomes, such as, annualized bleeding rate or mortality are less sensitive to detect health status in these populations.
- We encourage investigators and clinicians to use a patient-reported outcome tool for assessing the health status of PWH

Collaborating Patient Organizations

- Fundación de la Hemofilia (Argentina) Cordoba Chapter
- Hemophilia Foundation Australia
- Federação Brasileira de Hemofilia (Brazil)
- Canadian Hemophilia Society
- Association Française des Hémophiles (France)
- Deutsche Hämophiliegesellschaft (Germany)
- Magyar Hemofilia Egyesület (Hungary)
- Irish Haemophilia Society
- Federazione delle Associazioni Emofilici (Italy)
- National Hemophilia Network of Japan
- Federación de Hemofilia de la República Mexicana (Mexico)
- Nederlandse Vereniging van Hemofilie-Patiënten (The Netherlands)
- Haemophilia Foundation of New Zealand
- Haemophilia Foundation of Nigeria
- Polish Hemophilia Society
- Federación Española de Hemofilia (Spain)
- The Haemophilia Society (United Kingdom)
- National Hemophilia Foundation (US)
- Asociación Venezolana para la Hemofilia (Venezuela)
- Vietnamese Hemophilia Association

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 - Novo Nordisk (HERO)
 - Roche
 - Shire
 - Sobi
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For more information

If you have questions or would like more information about PROBE you may directly contact the PROBE investigator team:

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