

The effect of haemophilia on activities of daily living (ADL): PROBE cohort

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INTRODUCTION

Knowledge about the impact of haemophilia on a person's activities of daily living (ADL) is a critical component of a patient centred approach to the management of this disease. The aim of this analysis is to examine the impact of haemophilia on ADL based on data from the PROBE (Patient Reported Outcomes, Burdens and Experiences) project.

METHODS

The PROBE questionnaire asks a dedicated question to explore the impact of haemophilia on ADL, with 22 pre-coded ADL as possible answers. PROBE also includes a measure of QoL (EQ5D-5L). Descriptive analyses are presented as n (%) or mean (95% C.I.), as appropriate. Standard t-tests and chi squared tests were conducted to test for between-group differences.

RESULTS

The results, shown in Table 1, are derived from a sub-population of the PROBE survey of 1261 patients, aged 11-91, 70% males, classified based on diagnosis as not having a bleeding disorder (NOBD), or having mild, moderate or severe haemophilia. Patients with a current or history of an inhibitor were excluded from the present analysis due to the impact that this may have on QoL and ADL.

In total, 528 (42%) people with NOBD, 109 (9%) with mild, 129 (10%) with moderate and 495 (39%) with severe haemophilia filled the questionnaire. Severe haemophilia was associated with the lowest mean EQ5D utility of 0.729 (0.708-0.751), with moderate, mild and NOBD mean EQ5D utilities being 0.773 (0.732-0.813), 0.849 (0.816-0.883) and 0.916 (0.905-0.926), respectively.

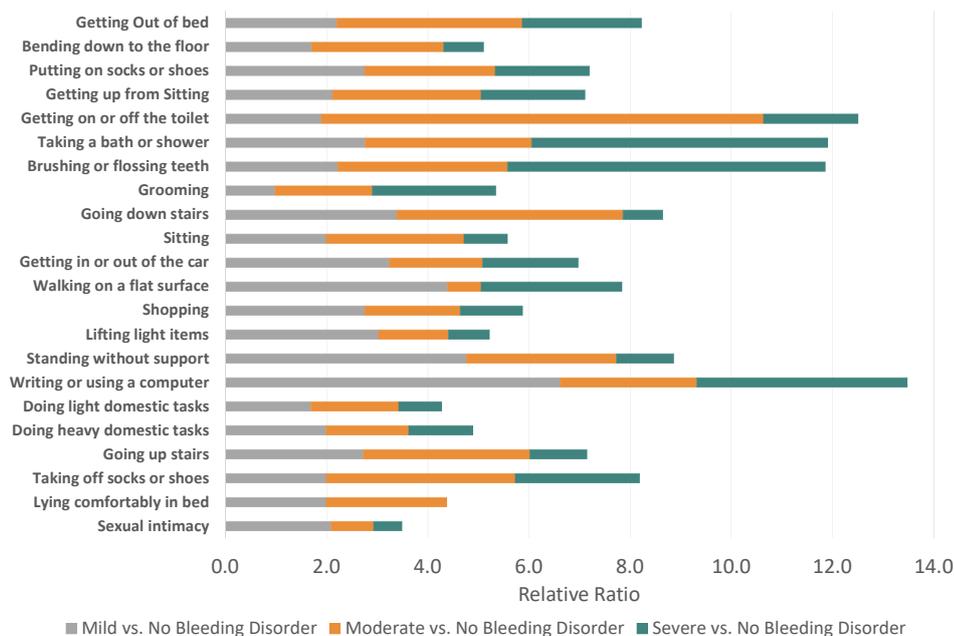
Overall, 14.7% of the NOBD, 30.6% of the mild, 60.9% of the moderate and 64.7% of the severe haemophilia patients reported ADL being affected. The mean number of ADL affected was 0.75 (0.58-0.98), 1.88 (1.11-2.64), 3.78 (2.94-4.62) and 4.87 (4.38-5.35) for NOBD, mild, moderate and severe patients, respectively. Figure 1 shows the relative ratio in patients with haemophilia reporting difficulties with ADLs.

There was a significant difference between the NOBD group and severe cohorts across all 22 ADL ($p < 0.001$) and 20 ADL ($p < 0.001$) in the moderate cohort (except for teeth brushing and grooming activities).

Table 1. Participant Characteristics

	n	EQ-5D utility (range)	People reporting ADL being affected	Mean number of ADL affected per patient
No Bleeding Disorder	528	0.916 (0.905-0.926)	14.7%	0.75 (0.58-0.98)
Mild Haemophilia	109	0.849 (0.816-0.883)	30.6%	1.88 (1.11-2.64)
Moderate Haemophilia	129	0.773 (0.732-0.813)	60.9%	3.78 (2.94-4.62)
Severe Haemophilia	495	0.729 (0.708-0.751)	64.7%	4.87 (4.38-5.35)

FIGURE 1. Relative Ratio of patients with bleeding disorders reporting difficulties with Activities of Daily Living compared to people without bleeding disorders



There were 5 ADL that were impacted in the mild cohort compared to the NOBD cohort ($p < 0.001$) which included, going down stairs, getting in/out of a car, walking on a flat surface and going up stairs.

When considering the increase of reported impairment in any of the activities as compared to the rate in the NOBD group, mild patients had a mean increase of 2.08 times (range 1.0-6.6), moderate patients 5.5 times (range 2.9-10.6) and severe patients 7.5 times (range 3.5-13.5).

CONCLUSIONS

The reported prevalence of affected ADL is significantly greater across all severities of haemophilia compared to those without a bleeding disorder. The gradient of impairment across levels of severity increases our confidence in the results. The relatively small difference between severe and moderate patients, and the larger difference between mild patients and those with NOBD will be further explored by using patient and treatment level covariates.

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