PROBE Results Show Continued Burden of Disease in Canadians with Hemophilia A and B

David Page[1], Alexandra Kucher[2], Federico Germini[3]
1 Canadian Hemophilia Society, Montreal, Canada
2 Patient Outcomes Research Group Ltd.
3 McMaster University, Hamilton, Canada

PROBE®
Patient Reported Outcomes Burdens and Experiences Study

Canadian Hemophilia Society
Help Stop the Bleeding
Société canadienne de l'hémophilie
Arrêtons l'hémorragie

INTRODUCTION

Prophylaxis with recombinant factor concentrates has been the standard of care for clinically severe hemophilia A and B in Canada since the early 1990s and has resulted in significantly improved quality-of-life. People with severe hemophilia A and B (PwH), report to be on regular prophylaxis at a rate of 88 and 85% respectively, though the rates are much lower in moderate and mild disease.

METHODS

Patients who report their home infusions through the patient portal of the Canadian Bleeding Disorders Registry (CBDR) are invited annually via email sent to their CBDR account to complete the online Patient Reported Outcomes Burdens Experiences (PROBE) questionnaire via the on-line app. Those not using CBDR are recruited through communications from the Canadian Hemophilia Society. Up until April 2022, 555 Version 3 questionnaires, the latest version, had been completed by PwHA, including 446, 63 and 46 with severe, moderate and mild disease respectively. 87 questionnaires had been completed by PwHB, including 53, 21 and 13 with severe, moderate and mild disease respectively. These represent a particularly good sample of over 40% of the severe patient population – 1073 PwSHA and 198 PwSHB – in the Canadian Hemophilia Registry. The survey was also completed by 201 Canadians with no bleeding disorder who serve as controls.

The data is viewed and analyzed via the PROBE on-line dashboard, which is available to all patient associations registered with PROBE.

CONCLUSIONS

Despite the high rate of prophylaxis in severe disease and the excellent network of Canadian treatment centres, the PROBE study observes that burden of disease remains significant in Canadian PwH compared to the general population. PwH report greater burden of disease – mobility limitations, pain and difficulties conducting activities of daily living – and lower PROBE and EQ5D scores compared to controls. Interestingly, the data show a lower psychosocial burden of depression and anxiety in PwH. More research is planned to better understand this phenomenon.

RESULTS

	Hemophilia A				Hemophilia B				Controls
	Severe	Moderate	Mild	All	Severe	Moderate	Mild	All	
Respondents (all ages)	446	63	46	555	53	21	13	87	201
Regular prophylaxis	88%	43%	14%	77%	85%	57%	8%	67%	NA
Use of mobility aids	29%	33%	24%	29%	29%	31%	21%	28%	8%
Use of pain medication in last 12 months	67%	78%	67%	68%	72%	83%	41%	70%	55%
Difficulties with activities of daily living	36%	40%	41%	37%	43%	33%	23%	38%	14%
Clinically diagnosed depression	4%	6%	11%	5%	2%	0%	0%	1%	13%
Anxiety disorder	9%	12%	11%	10%	6%	0%	7%	5%	23%
% of those retired due to health	81%	80%	25%	71%	25%	67%	100%	56%	17%
Working full- or part-time (22-64 years)	61%	67%	67%	62%	67%	76%	61%	68%	70%
Days missed annually from school/work (11-64 years)	32	28	54	34	22	5	33	19	13
PROBE score	0.79	0.77	0.79	0.79	0.75	0.78	0.83	0.77	0.89
EQ5D score	0.81	0.76	0.81	0.81	0.81	0.82	0.88	0.82	0.90

Not surprisingly, PwH, compared to the control group, report …

- greater need for mobility aids: 29% in HA, 28% in HB, 8% in controls;
- higher use of pain medication in last 12 months: 68% in HA, 70% in HB, 55% in controls;
- more difficulties with activities of daily living: 37% in HA, 38% in HB, 14% in controls.

Although the sample size for mild and moderate HA and HB is quite small, there is remarkably little difference in reported burden of disease compared to those with severe disease, likely due to the widespread use and mitigating effect of prophylaxis in severe disease.

Employment levels in those aged 22-64 are slightly lower in PwH (62% in HA, 68% in HB) than in controls (70%). Days missed annually from school/work were higher: 34 in HA, 19 in HB vs. 13 in controls. Those PwH who have retired were much more likely to have done so due to health (71% in HA, 56% in HB) than controls (17%).

Surprisingly, levels of clinically diagnosed depression are reported to be much lower in PwH than in controls: 5% in HA, 1% in HB vs. 13% in controls. Idem for anxiety disorder: 10% in HA, 5% in HB vs. 23% in controls. Is the benefit of regular medical follow-up in PwH a possible explanation? Or is this an example of the disability paradox, where psychosocial issues are minimized in importance given the physical burden of disease?

The PROBE scores, based on a subset of key outcomes, were consistent across all severities of HA and HB, and 0.10 lower than controls. EQ5D scores were also quite similar across patient groups and 0.08 to 0.09 lower than controls. Note that a higher score indicates better quality of life, and 1 is the maximum.

ACKNOWLEDGEMENTS

Thanks to all those who completed the questionnaire.

PROBE is an independent investigator led research project with grant / research support from: Bayer, CSL, Novo Nordisk, Roche, Sanofi, Sobi and Takeda.

CONTACT INFORMATION

David Page, dpage@hemophiliia.ca

info@probestudy.org

myprobe.org