

Assessments of Functional Impairment in US Adults With Hemophilia Across Patient-Reported Outcomes in the Pain, Functional Impairment, and Quality of Life (P-FiQ) Study

P-M-131

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Objective

- To assess functional impairment in adult people with hemophilia (PWH) through patient-reported outcome (PRO) instruments

Introduction

- Hemophilia is marked by frequent joint bleeding, resulting in pain and functional impairment
- Standardized and disease-specific PRO instruments have been used in clinical studies, but rarely to individualize treatment

Methods

- Adult males with mild to severe hemophilia and a history of joint pain/bleeding were enrolled during routine visits
- Participants and treatment center staff provided assessments of functional status using the 6-category Centers for Disease Control and Prevention Universal Data Collection (CDC-UDC) scale¹
- Participants completed 5 PRO instruments and underwent an evaluation of joint range of motion (6 index joints)
- Functional impairment was assessed by PROs using EQ-5D-5L with visual analog scale (VAS), SF-36v2, and Hemophilia Activities List (HAL)

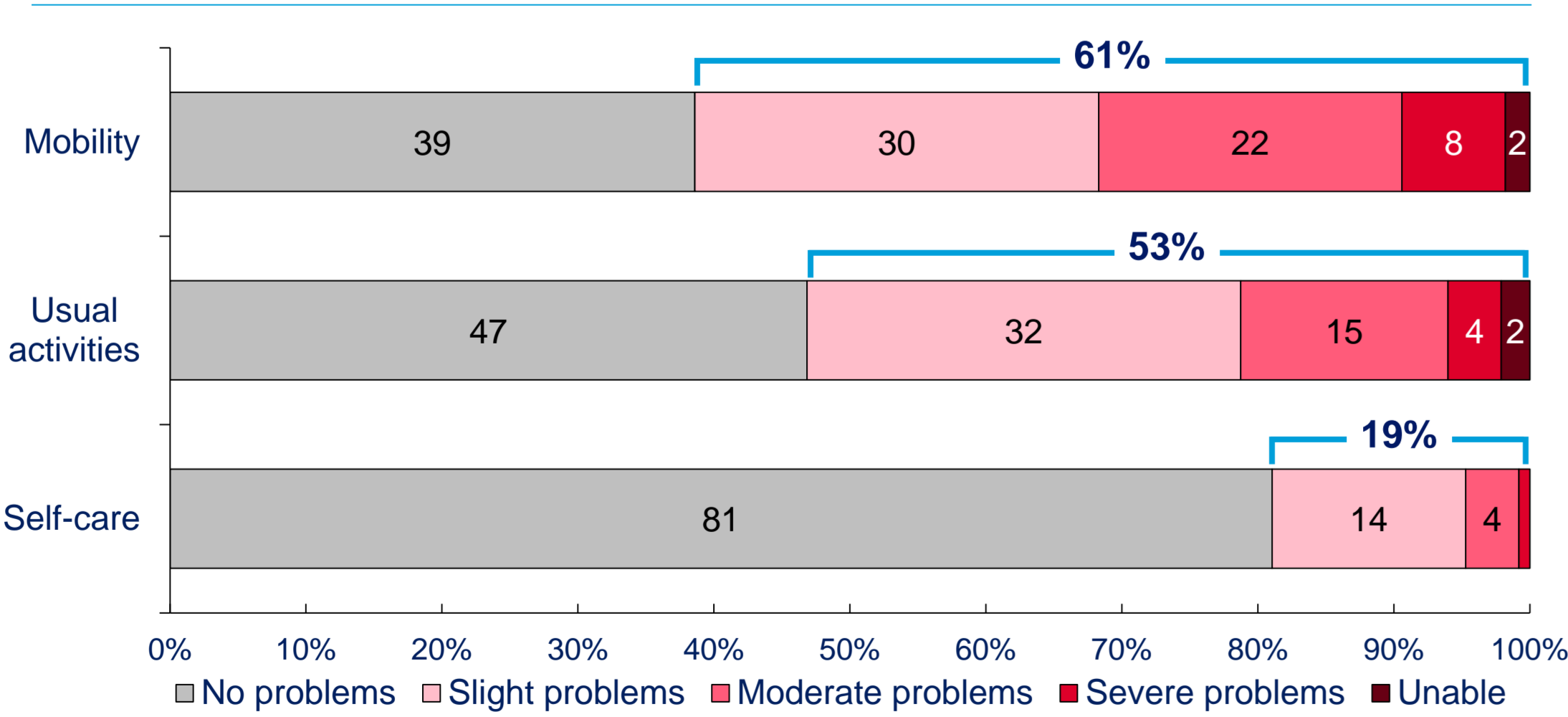
Results

- Overall, 381 PWH enrolled; median age was 34 years
- Most participants were employed (77%), and 65% were overweight/obese
- Most participants self-reported arthritis/bone/joint problems (65%), and half (50%) reported a history of joint procedures/surgeries
- Patient- and site-reported rates of functional disability in the past 6 months (CDC-UDC scale) were 66% and 59%, respectively

EQ-5D-5L

- Participants reported problems "today" with mobility (61%), usual activities (53%), and self-care (19%) (Figure 1)

Figure 1 EQ-5D-5L functional domains ("today")



SF-36v2

- Functional domain/summary scores are presented in Table 1

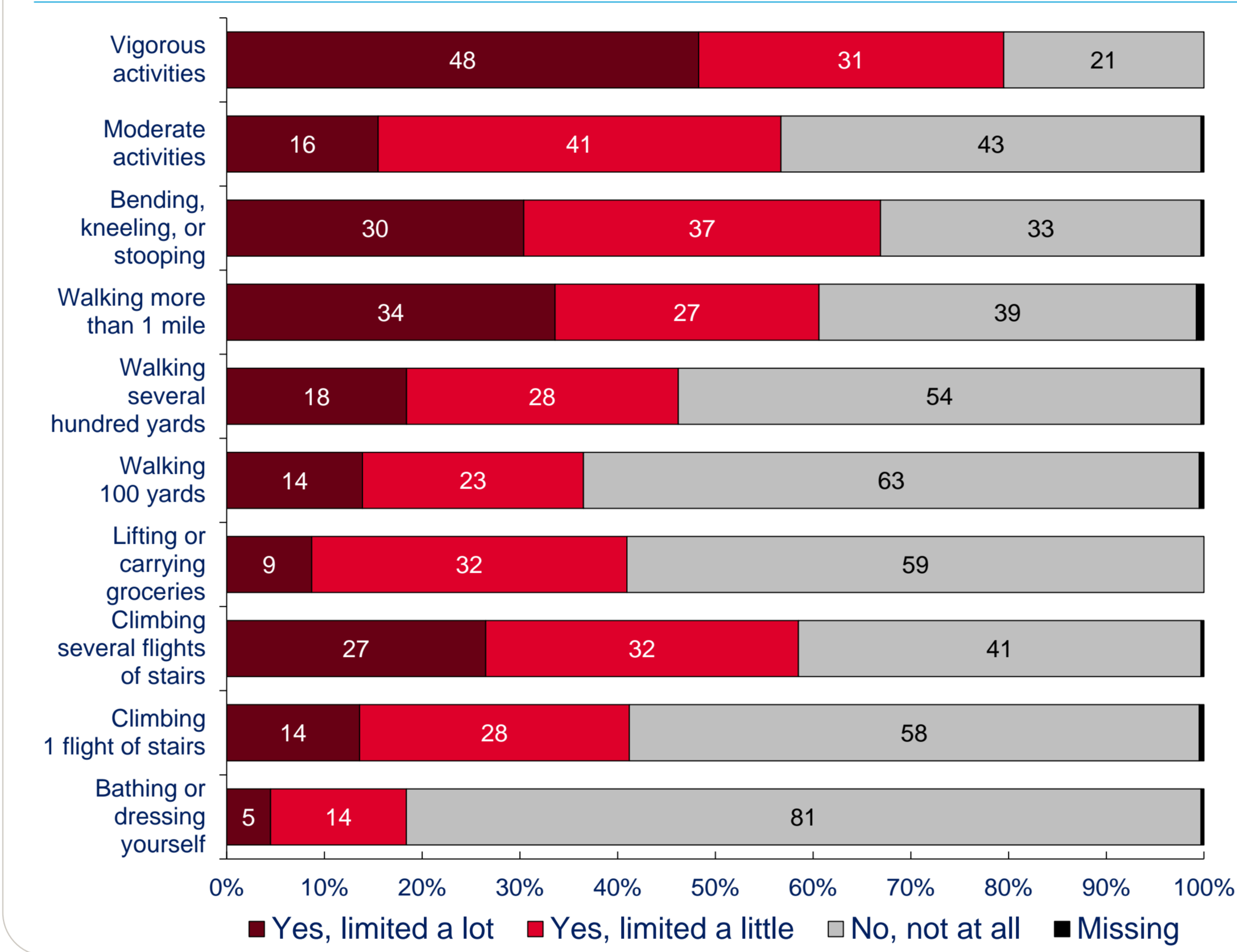
Table 1 SF-36v2 functional domain/summary scores

Domain/Summary Score	Median (Q1, Q3) ^a
Physical functioning (PF)	44.4 (29.7, 52.8)
Role physical (RP)	44.6 (32.4, 56.9)
Physical health component summary (PCS)	39.2 (29.5, 49.4)

^aRange 0 to 100; higher scores indicate better quality of life.

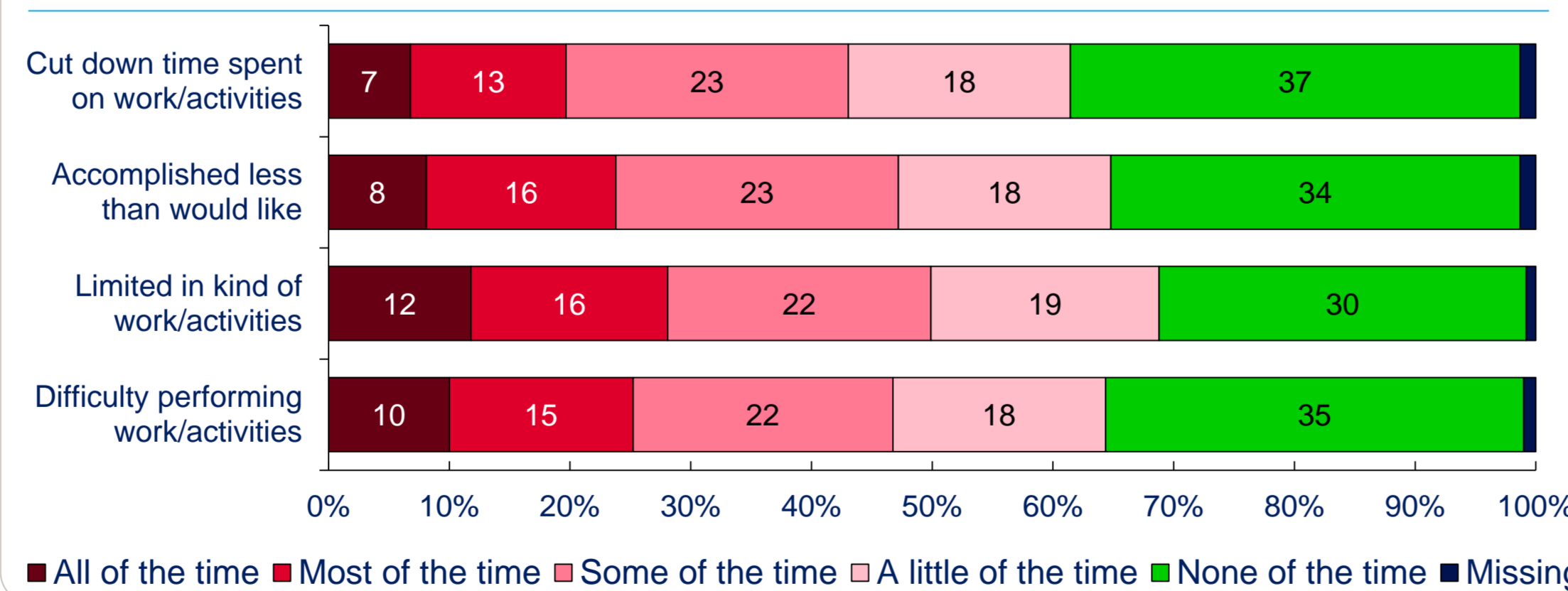
- The activities in the past 4 weeks that were most frequently limited during a typical day were vigorous activities (80%), bending, kneeling, or stooping (67%), walking more than a mile (61%), and climbing several flights of stairs (59%) (Figure 2)

Figure 2 SF-36v2 activity limitations (over the past 4 weeks)



- Physical problems caused participants to limit kinds of work/activities (69%), accomplish less than they would like (66%), have difficulty in performing work/activities (65%), and cut down time spent on work/activities (62%) (Figure 3)

Figure 3 SF-36v2 physical problems due to hemophilia (over the past 4 weeks)



HAL

- Domain/composite scores related to functional impairment are presented in Table 2; in general, greater difficulties were seen for the lower vs upper extremity functions/activities

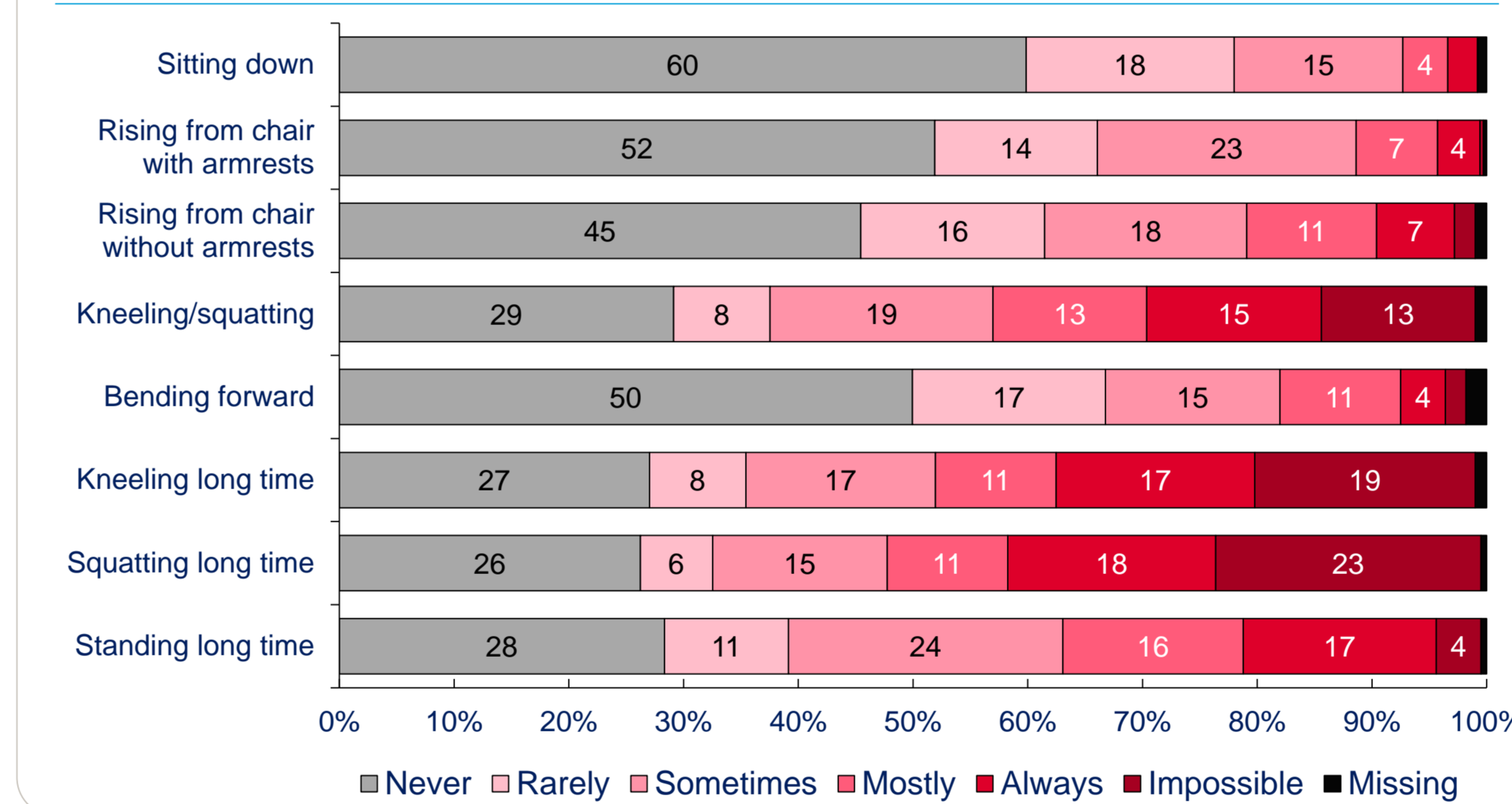
Table 2 HAL functional domain/composite scores

Domain/Composite Score	Median (Q1, Q3) ^a
Domain scores	
Lying down/sitting/kneeling/standing	67.5 (45.0, 95.0)
Functions of the legs	66.7 (40.0, 95.6)
Functions of the arms	80.0 (60.0, 100.0)
Use of transportation	86.7 (60.0, 100.0)
Self-care	100.0 (84.0, 100.0)
Household tasks	88.0 (64.0, 100.0)
Leisure activities and sports	80.0 (56.7, 100.0)
Composite scores	
Upper extremity activities	88.9 (73.3, 97.8)
Basic lower extremity activities	73.3 (46.7, 96.7)
Complex lower extremity activities	55.6 (30.0, 88.9)
Overall sum score	76.6 (58.0, 94.3)

^aRange 0 to 100; higher scores indicate better quality of life.

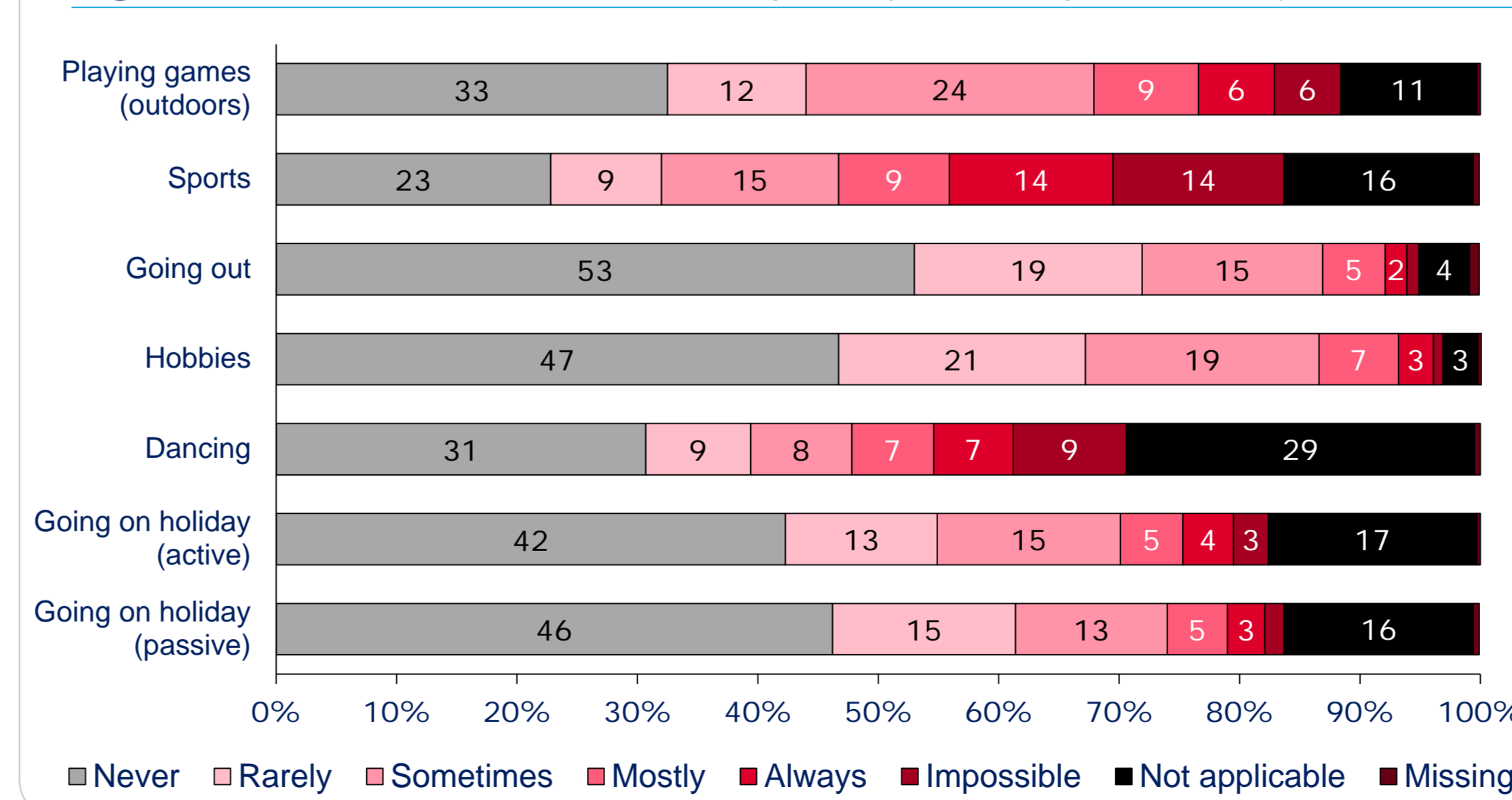
- Within the lying down/sitting/kneeling/standing domain, the most frequent problems in the previous month were squatting for a long time (74%), kneeling for a long time (73%), standing for a long time (72%), and kneeling/squatting (70%) (Figure 4)

Figure 4 HAL lying, sitting, kneeling, and standing (over the past month)



- Within the leisure activities and sports domain, the most frequent problems in the previous month were sports (61%) and playing games outdoors (56%) (Figure 5)

Figure 5 HAL leisure activities and sports (over the past month)

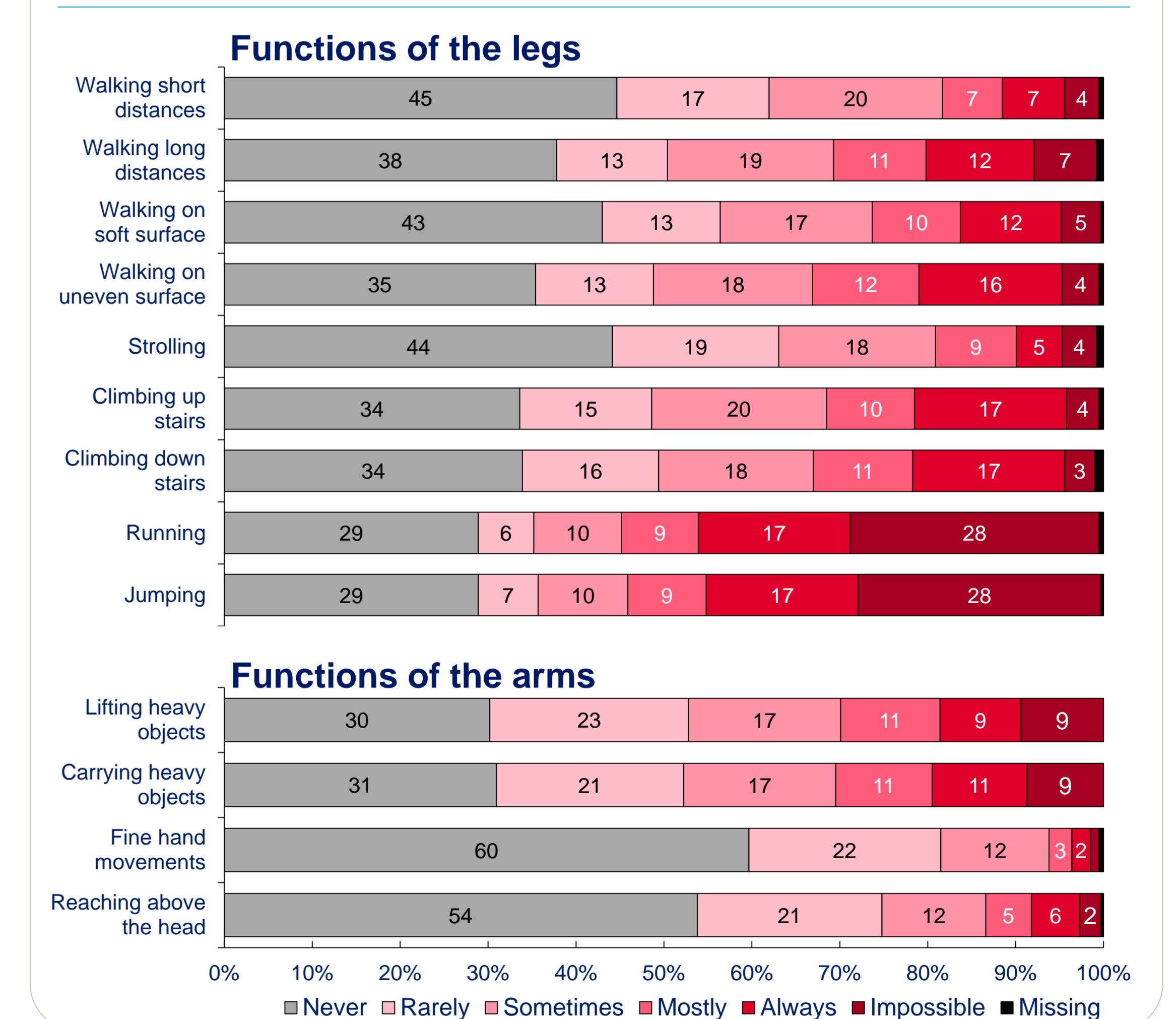


Conclusions

- P-FiQ data from 3 PROs highlight the challenges of functional impairment among adult PWH and the higher burden of lower vs upper extremity issues
- More detailed instruments (eg, HAL) may be helpful in identifying specific functional limitations for targeted physical therapy interventions
- Enhanced patient-provider dialogue and consistent functional assessment in the comprehensive care setting may help to drive improved outcomes in adult PWH

- Within the functions of the legs and arms domains, the most frequent problems in the previous month were jumping (71%), running (71%), lifting heavy objects (70%), and carrying heavy objects (69%) (Figure 6)

Figure 6 HAL functions of the legs and arms (over the past month)



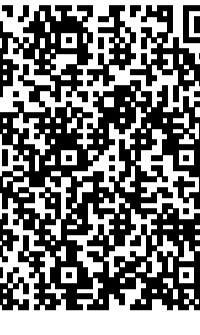
Reference

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Conflict of interest disclosure

M Witkop has received grant funding from Pfizer, is on advisory boards with Baxter BioScience, CSL Behring, Novo Nordisk, Octapharma, and Pfizer, and is on the speakers bureau at Novo Nordisk. G Hernandez has received grant/research support from Bayer and Novo Nordisk, has served on advisory boards for Biogen Idec, and is on the speakers bureau for Emergent BioSolutions. M Recht has received grant/research support from Biogen Idec, Novo Nordisk, and Pfizer and served as a consultant for Kedron and Novo Nordisk. M Wang has served as a consultant for Baxter, Biogen, CSL Behring, and Novo Nordisk. K Baumann has served as a consultant for Bayer and Novo Nordisk and as a speaker for Baxter, Bayer, and Novo Nordisk. D Cooper is an employee of Novo Nordisk Inc. C Kempton has served as a consultant for Baxter, Biogen, CSL Behring, Hoffman-La-Roche, and Kedron and received grant/research support from Novo Nordisk.

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Orthopedic issues
David Cooper