



Short-term Health State Change in Older Persons with Hemophilia from the Hematology Utilization Group Studies Part VII (HUGS VII)

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Disclosures for: Randall G. Curtis, MBA

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Director, Officer, Employee	
Shareholder	
Honoraria	
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Introduction

- Effective treatment has lengthened life expectancy for persons with hemophilia (PWH)
- Hemophilia-related complications impact health-related quality of life (HRQoL)
- Understanding which dimensions of health are changing can provide important information about the effects of patients' management of treatment



Objectives

- We assessed HRQoL changes within a six-month period using data from HUGS VII
- We explored the variables associated with worsened health states



Methods

- Study collected data from persons with hemophilia A or B aged ≥ 40 years who obtained care from three US Hemophilia Treatment Centers from 2018 – 2020
- Participants completed the EQ-5D-3L survey at baseline (enrollment) and follow-up at six months
- Medical chart reviews documented hemophilia severity and complications during the six months prior to and after enrollment



Methods cont'd

- The EQ-5D-3L measures patient self assessment of their quality of life in five domains: mobility, self-care, usual activities, pain/discomfort and anxiety/depression.
- Each dimension has 3 levels: no problems, some problems, and extreme problems.
- The digits for the five dimensions can be combined into a 5-digit index number that describes the patient's health state.
- The EQ VAS (Visual Analog Scale) records the patient's self-rated health on a 100 point visual scale from 'Best imaginable health state' to 'Worst imaginable health state'.



Methods cont'd

- Paretian classification of health change (PCHC) was used to judge overall health improvement or worsening in EQ-5D profile
 - No change: all health state dimensions had no change
 - Improved: at least one dimension of health state improved, and no worse in any other dimensions
 - Worsened: at least one dimension of health state decreased, and no other dimensions improved
 - Mixed (some profiles included both improved and worsening health states)



Statistical Analysis

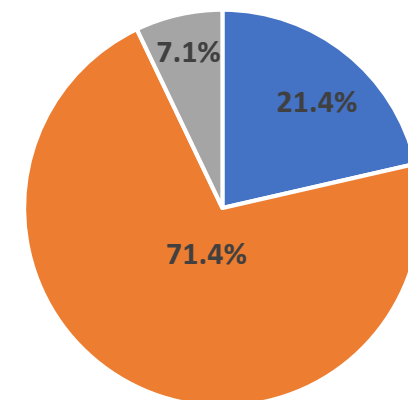
- EQ-5D-3L VAS and index score changes between baseline and follow-up were analyzed using paired Student T-tests
- EQ-5D-3L profile data were analyzed for both baseline and follow-up using Chi-square tests
 - Compared baseline and follow-up
 - Stratified by age group: 40-49 vs ≥ 50 years old
- We explored the variables associated with worsened health states using Chi-square tests
 - Severe medical problems (medical chart documented)
 - New diagnosis of hemophilia complications (medical chart documented)
 - Joint bleeding (Self-reported)



Participants' Characteristics

Variable	Age 40-49 N=20	Age ≥ 50 N=36	Total N=56	P value*
Mean age (SD)	44.9 (2.8)	63.9 (7.7)	57.1 (11.2)	<0.0001
Hemophilia severity (%)				0.04
Mild/moderate	7 (35.0)	23 (63.9)	30 (53.6)	
Severe	13 (65.0)	13 (36.1)	26 (46.4)	
Mean Dif. EQ VAS (SD)†	2.7 (16.9)	-0.3 (16.6)	0.71 (16.6)	0.54
Mean Dif. EQ Index (SD)†	0.01 (0.13)	0.04 (0.17)	0.03 (0.16)	0.57

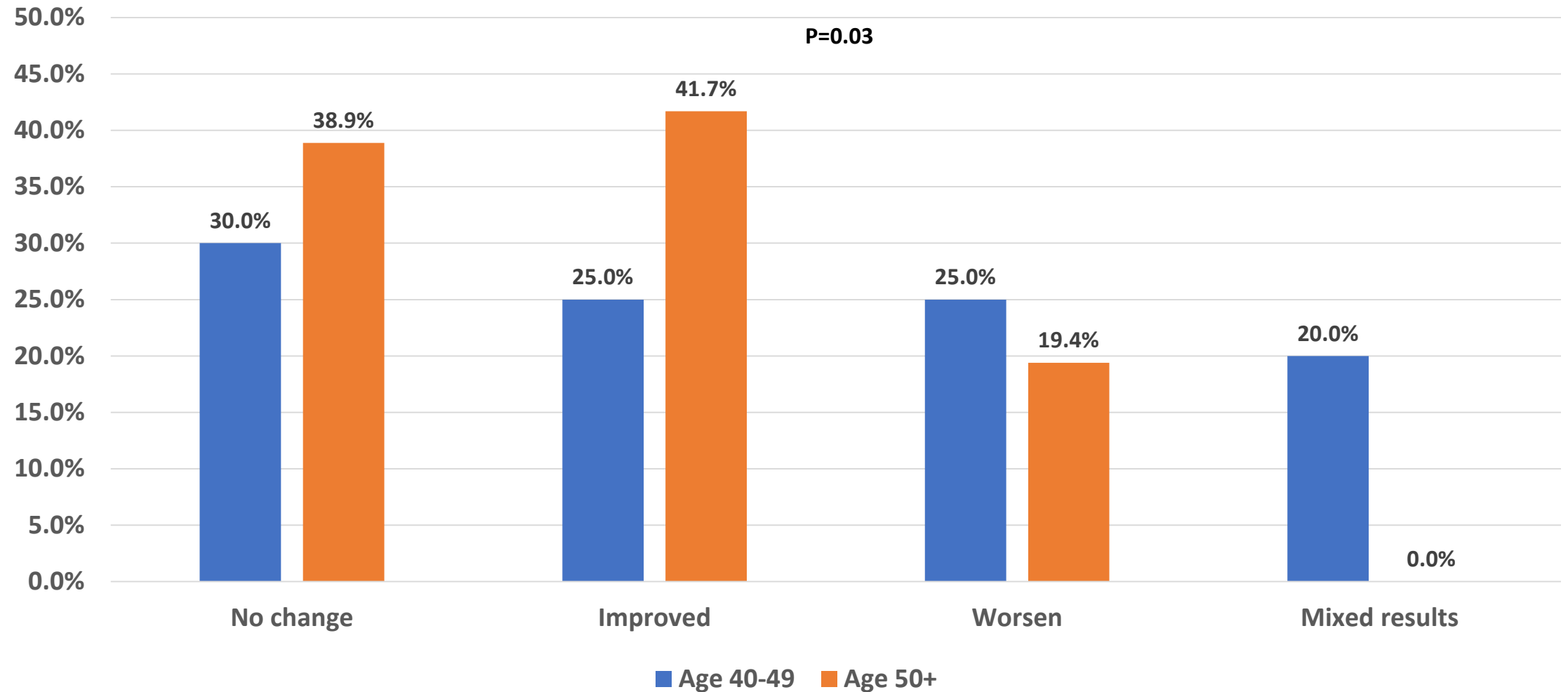
PCHC Classification of Health States



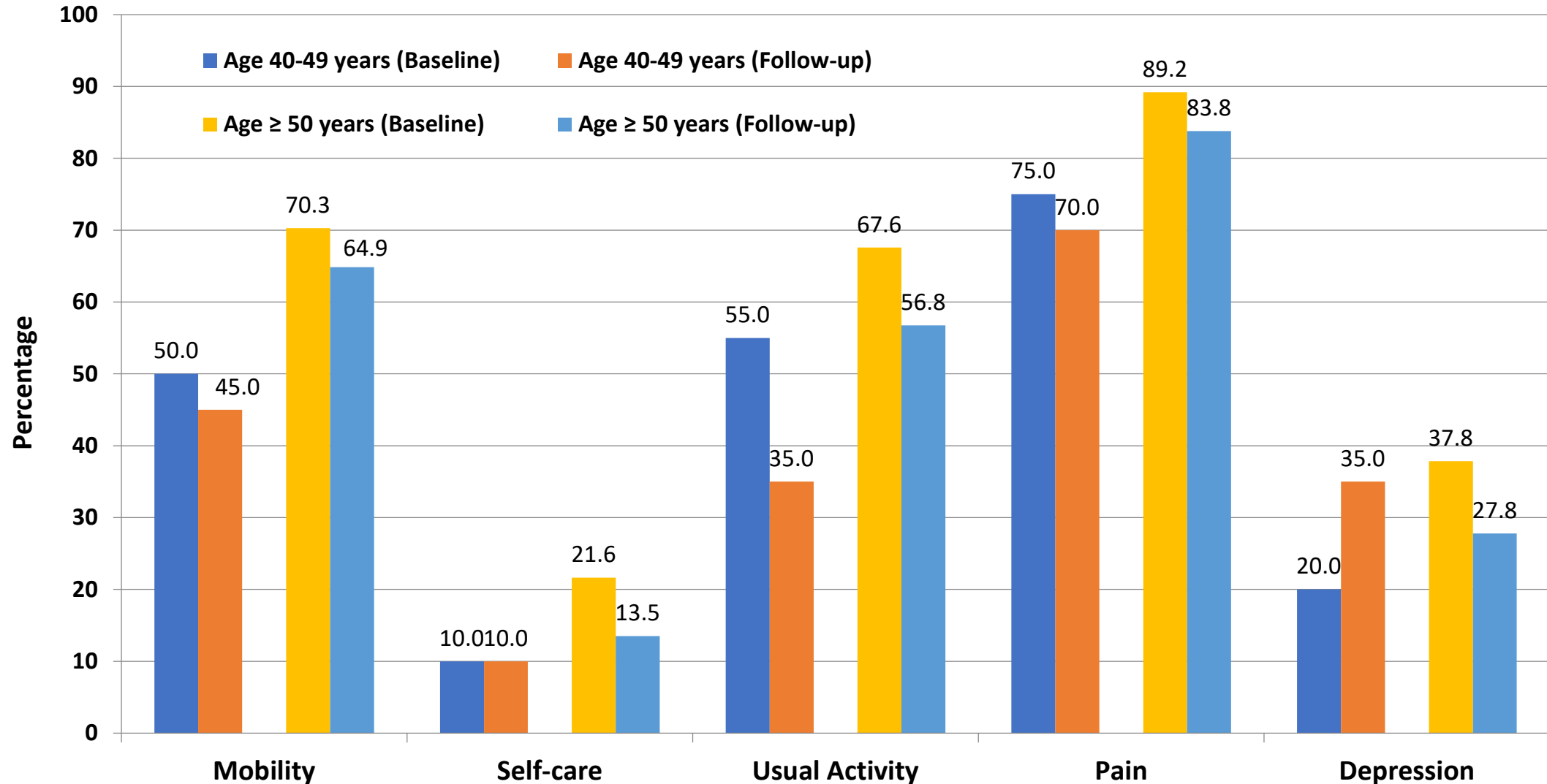
■ Worsening ■ Improvement/no change ■ Mixed results‡

Note: Study sample included the participants who completed EQ-5D-3L at baseline and follow up. Data are presented as mean (standard deviation) for continuous variables or number (column percentage) for categorical variables. * P values were calculated using Paired Student T-tests for continuous variables, and Chi-square tests for categorical variables to compare the differences between two age groups. †Score difference between baseline and follow-up. ‡Some profiles included both improved and worsening health states. Abbreviations: SD=standard deviation, VAS=visual analogue score.

Health State Change by Age Group



Proportion Reporting EQ-5D Problems



Variables Associated with Worsened Health

Variable	No worsening health	Worsening health	Total	P value
Experienced severe medical problems at baseline*				0.006
Yes	0 (0)	2 (100)	2 (3.6)	
No	44 (81.5)	10 (18.5)	54 (96.4)	
Newly diagnosed complications at baseline†				
Yes	7 (58.3)	5 (41.7)	12 (21.4)	0.05
No	37 (84.1)	7 (15.9)	44 (78.6)	
Joint bleeds at follow-up*				0.25
Yes	22 (75.9)	7 (24.1)	29 (53.7)	
No	22 (88.0)	3 (12.0)	25 (46.3)	

Note: Data are presented as number (row percentage). *P values were reported for Fisher's Exact tests. †P values were reported for Chi-square test.



Limitations

- Small sample size limits statistical power to detect between-group differences
- The study sample includes more persons with mild/moderate hemophilia in the 50+ years group than in the younger age group (40-49 years old)
- Individuals lost to follow-up further decreased the sample size



Conclusions

- In this sample, persons with hemophilia aged 50 years or older, a majority of whom had mild or moderate disease, displayed a higher rate of health state improvement within six months compared to the younger group, which included a larger proportion of those with severe hemophilia
- Severe complications, newly diagnosed complications, and joint bleeding contributed to worsening health states observed in this short-term observation



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Thank you!