

# THE TRAJECTORY OF HEALTH-RELATED QUALITY OF LIFE IN YOUTH AND MEN WITH HAEMOPHILIA A

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## INTRODUCTION

Health-related quality of life (HRQoL) is an important outcome in clinical trials.

Little is known about the natural history of HRQoL, over time, in youth and men with hemophilia A.

This abstract describes the longitudinal patterns of HRQoL among youth and young adults with severe haemophilia A (HA) over a 2 to 3-year period.

## METHODS

**Recruitment:** Males (13-29 years) with severe HA were recruited from 6 Canadian Treatment Centres. All participants were receiving routine care with regular prophylaxis.

**Observational Design:** Participants completed a comprehensive survey at 6 month intervals over 36 months. The first 24 months are presented here. HRQoL was measured using the generic SF-36 Physical Component Summary score (PCS).

**Clinical Data:** Information on annualized bleeding rates (ABR), target joints ( $\geq 3$  bleeds into the same joint in 3 months), joint health (Haemophilia Joint Health Score version 2.0; HJHS), and concomitant medical events were collected prospectively.

## RESULTS

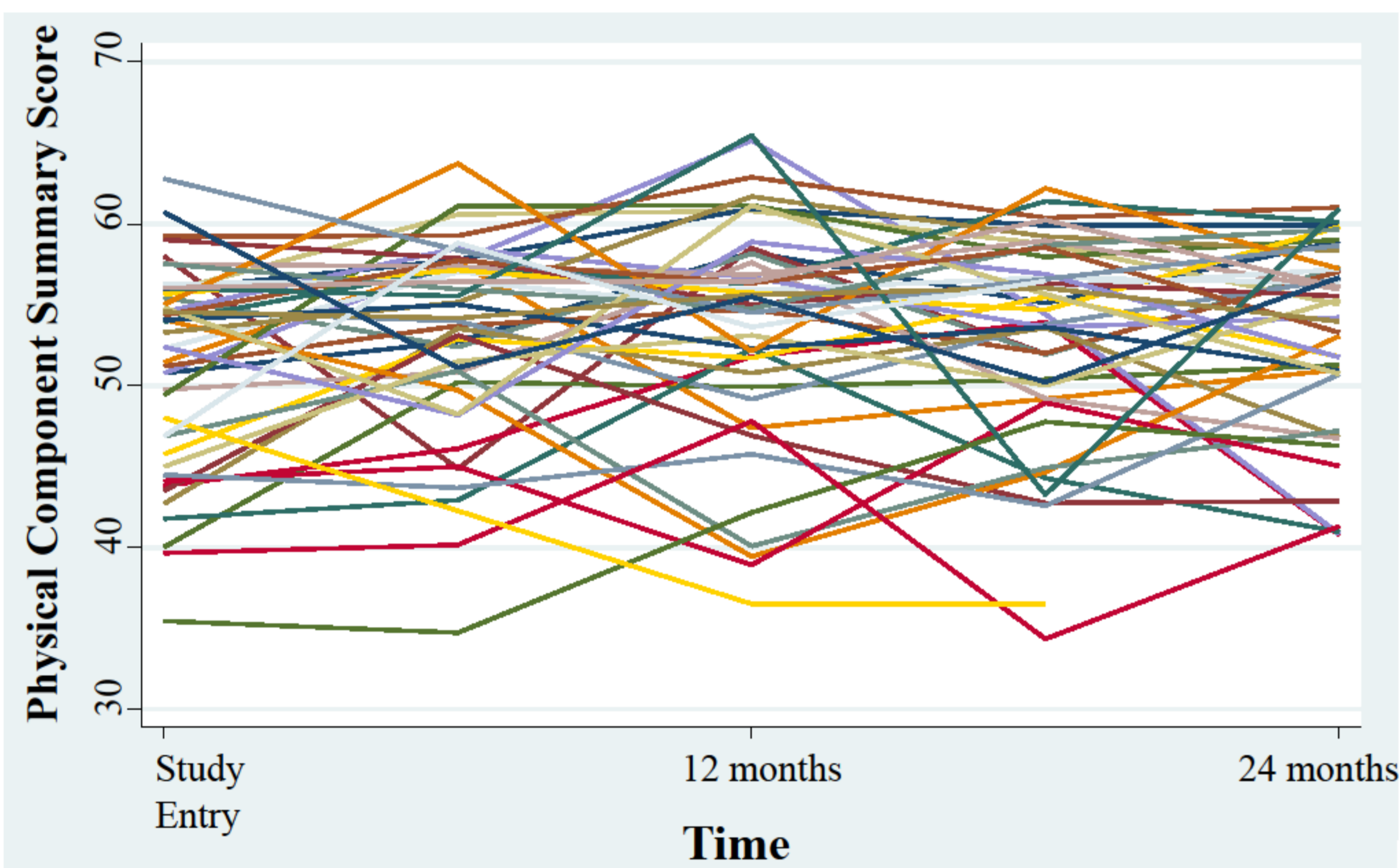
**Sample:** 46 participants completed the study

- 13 youth (mean age=15.7, range=13-17.9 years)
- 33 adults (mean age=23.6; range=18.4 -28.7 years)

All were on prophylaxis:

- 17% once per week
- 46% alternate day
- 37% daily

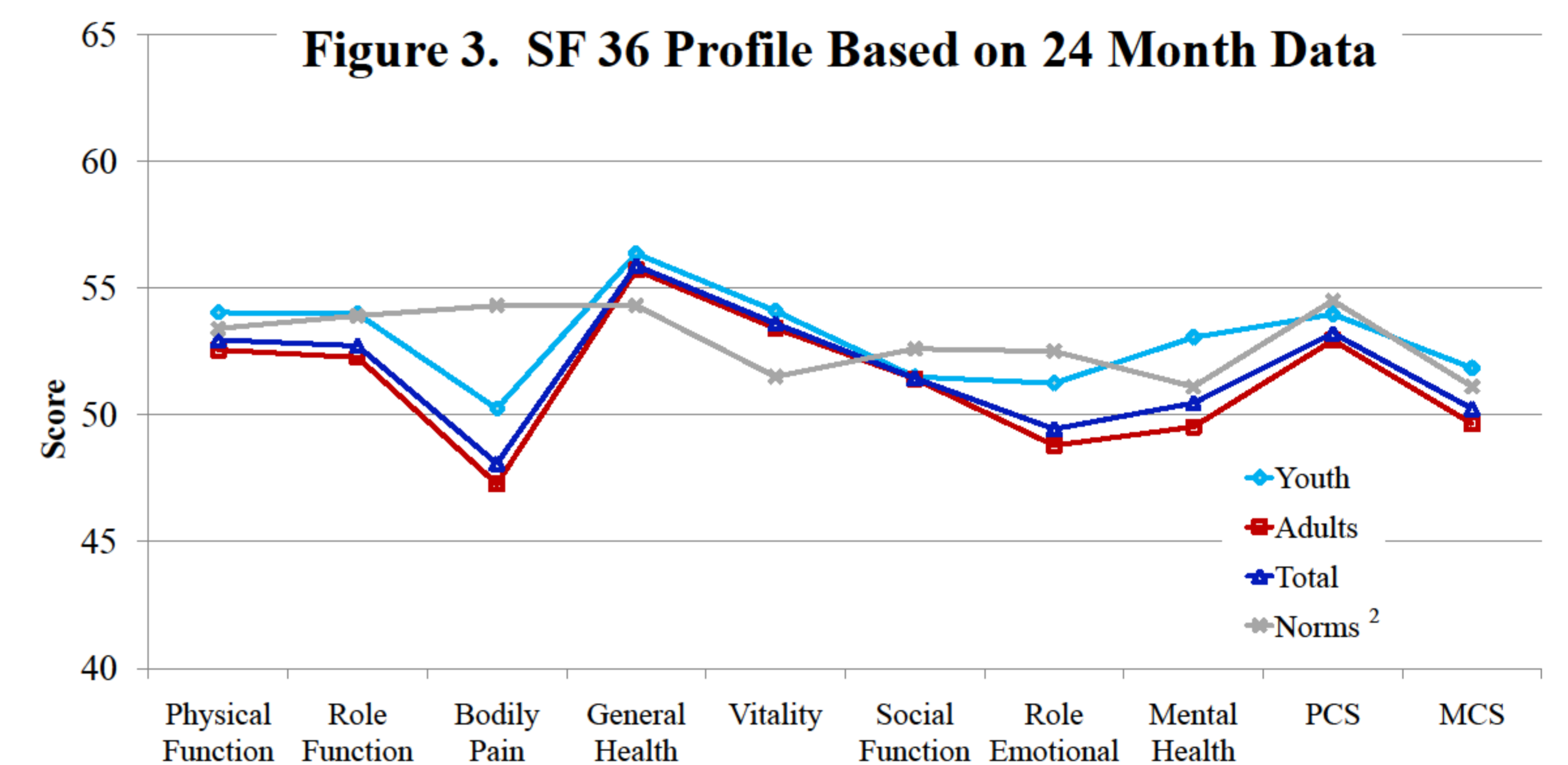
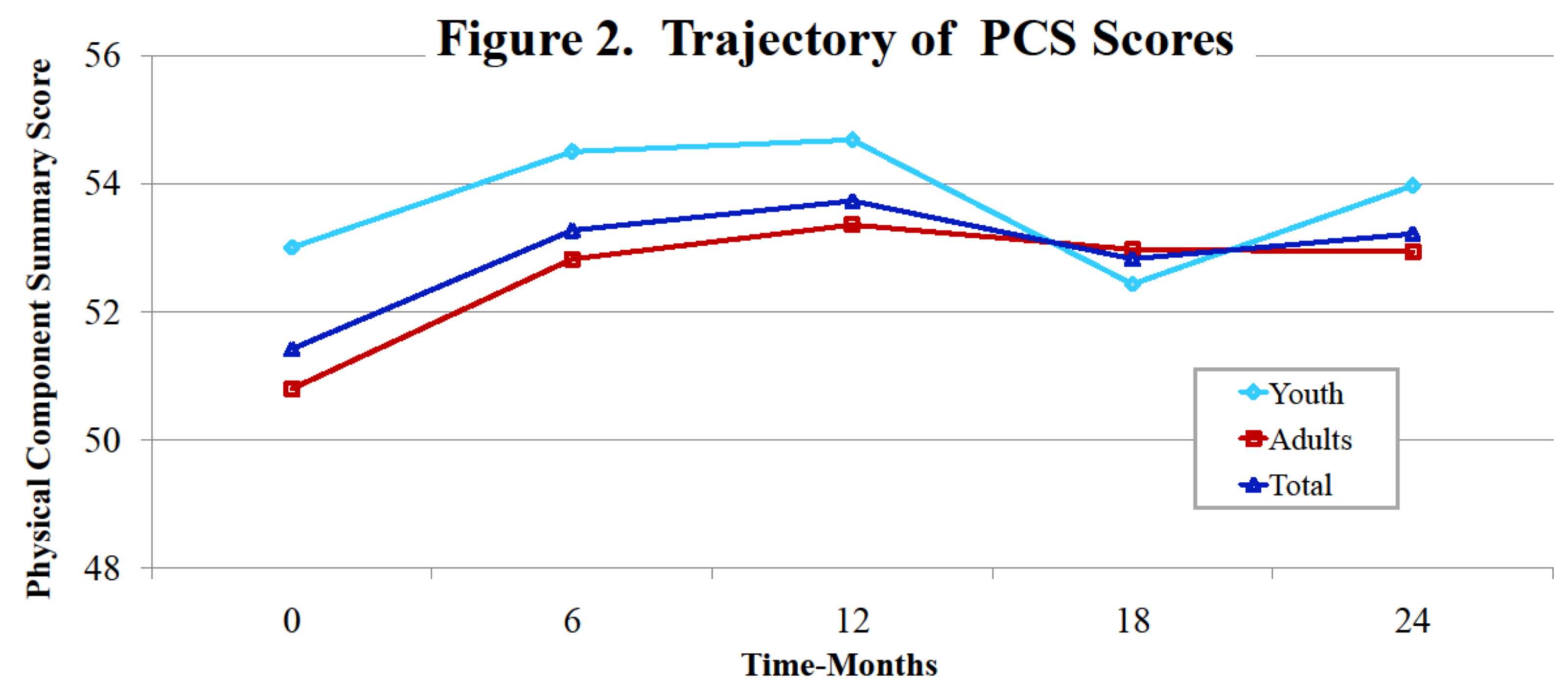
**Figure 1. Individual Variation in Trajectory of PCS Scores**



### What clinical factors impact HRQoL status scores?

- ❖ Regression of four key variables on HRQoL (as measured by the SF36 Physical Component Score) at 24 months showed that PCS scores were:
  - lower in older participants (age  $b=-0.06$ ,  $p=0.769$ );<sup>1</sup>
  - lower among those with a target joint at baseline ( $b=-0.90$ ,  $p=0.680$ );
  - lower in those with more bleeds during this period (total ABR  $b=-0.15$ ,  $p=0.347$ ); and
  - lower in those with worse joint health (HJHS  $b=-0.34$ ,  $p=0.001$ ).

Note the only statistically significant factor was joint health. A confirmatory analysis of the 12 month data yielded similar findings. The confluence of age and clinical measures explained 38% (24 months) of the variation.



### Do changes in bleeding frequency and joint health impact HRQoL change scores over one year?

- ❖ Regression of two key variables on Change in HRQoL (as measured by PCS at 24 months – PCS at 12 months) showed that change scores were not associated with:
  - a change in the bleeding rate ( $b=-0.17$ ,  $p=0.326$ ); or
  - a change in joint health (HJHS  $b=0.29$ ,  $p=0.239$ ).

Other factors, beyond clinical factors, must be explored to better understand individual changes over time.

## CONCLUSION

- There was substantial variation in HRQoL over time at the individual level, despite the fact that this was a non-interventional study. [Figure 1]
- Variation was less apparent at the group level. Youth had consistently higher HRQoL scores than adults (on average 1.5 to 2 points higher). The initial increase requires further exploration. [Figure 2]
- Our SF36 profiles were substantially more favorable than the haemophilia sample reported by Klamroth in 2011<sup>2</sup> and similar to the normative profile with the exception of bodily pain. [Figure 3]
- Age, target joint at baseline, bleeding (ABR) and joint health (HJHS) all contributed to HRQoL status. Joint health scores had the largest impact on HRQoL status. However, change in bleeding and joint health did not appear to predict changes in HRQoL over a one-year period.
- These results aid in our understanding of HRQoL among youth and men with hemophilia on prophylaxis. However, other factors must be explored in future research to better understand individual changes in HRQoL over one year.

## REFERENCES

1. Where b represents an unstandardized regression coefficient.
2. Klamroth, R., et al. (2011). "The relative burden of haemophilia A and the impact of target joint development on health-related quality of life: results from the ADVATE Post-Authorization Safety Surveillance (PASS) study." *Haemophilia* 17(3): 412-421.

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