

The impact of Vanishing White Matter on non-affected family members

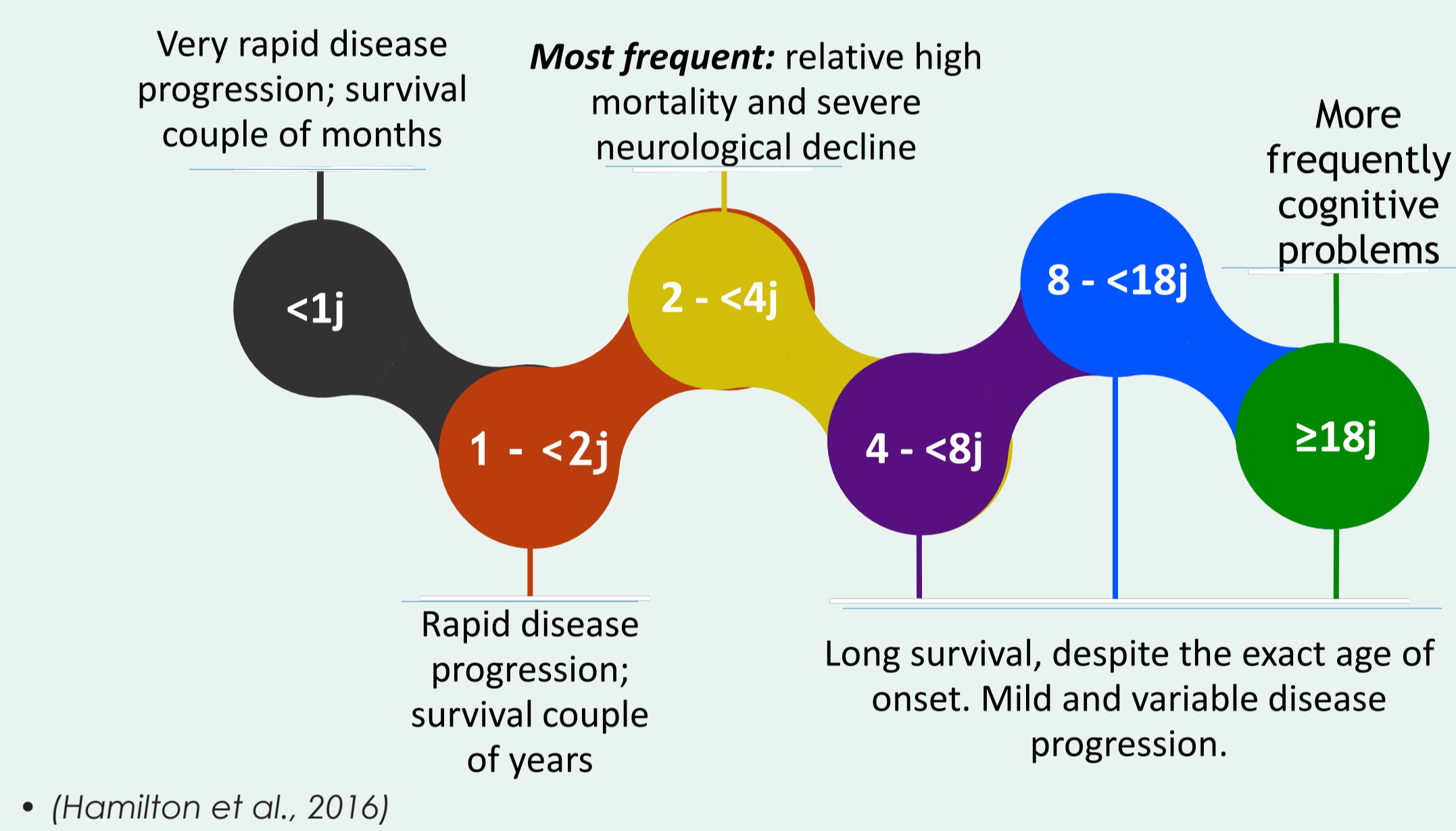
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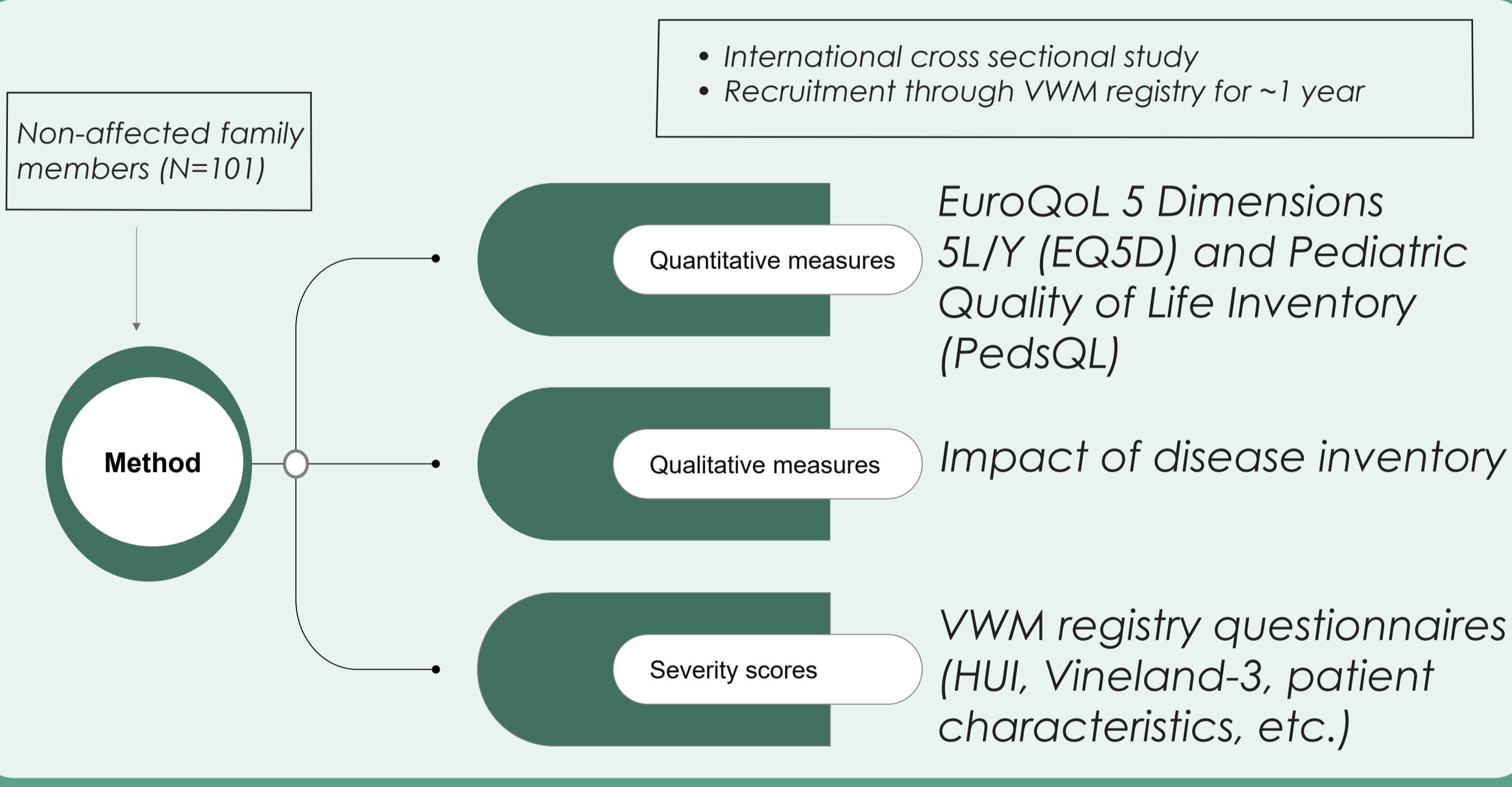
Background

Vanishing White Matter (VWM)

- Leukodystrophy with infantile to adult onset.¹
- Chronic progressive neurological deterioration and additionally stress-provoked episodes of rapid worsening.¹
- Variable disease progression and severity, correlated to age of onset.²
- Require different types of care throughout their lives.
- Impact on the different family members has not been investigated.



Method



Conclusion

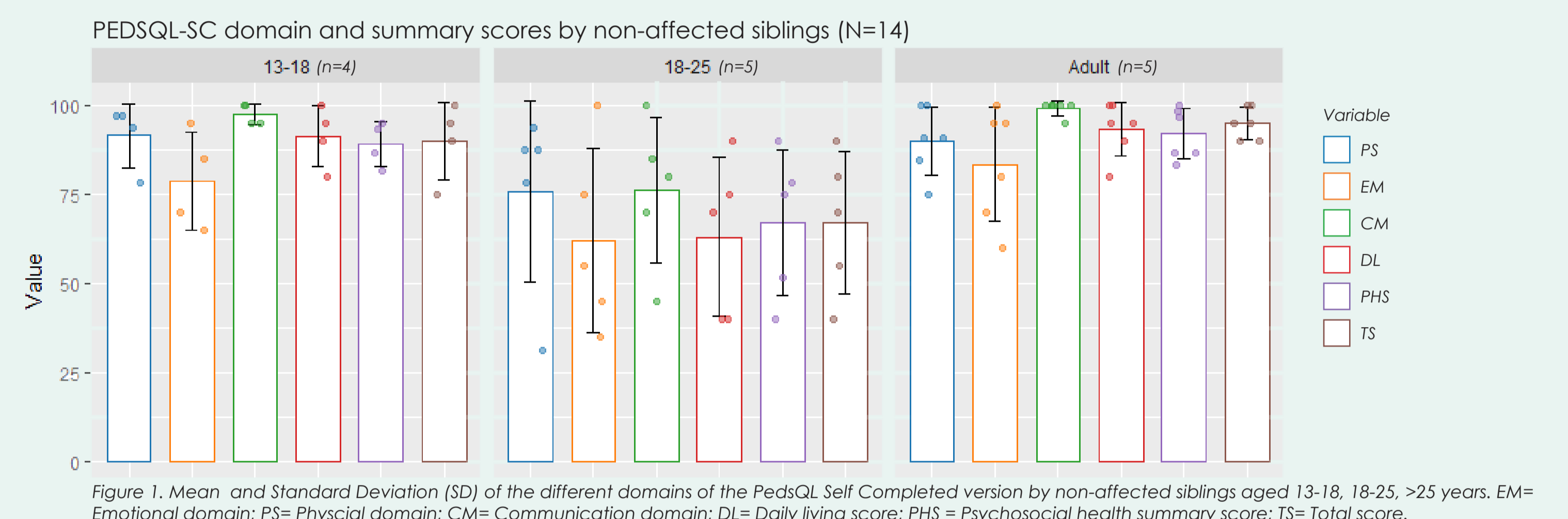
1. Non-affected family members highlight **significant impact** of VWM on **daily functioning** and **quality of life**.
2. **Qualitative research** will be of great value, **exploring the different needs** of family members even further.
3. **Raising awareness** on these different needs is a first step towards developing a more **tailored approach**.

References

- 1) Hamilton EMC, van der Lei HDW, Vermeulen G, et al.. Natural history of vanishing white matter. *Ann Neurol.* 2018;84(2):274-288
- 2) Van der Knaap MS, Barth PG, Gabreëls FJ, et al.. A new leukoencephalopathy with vanishing white matter. *Neurology.* 1997;48(4):845-855

Results & Discussion

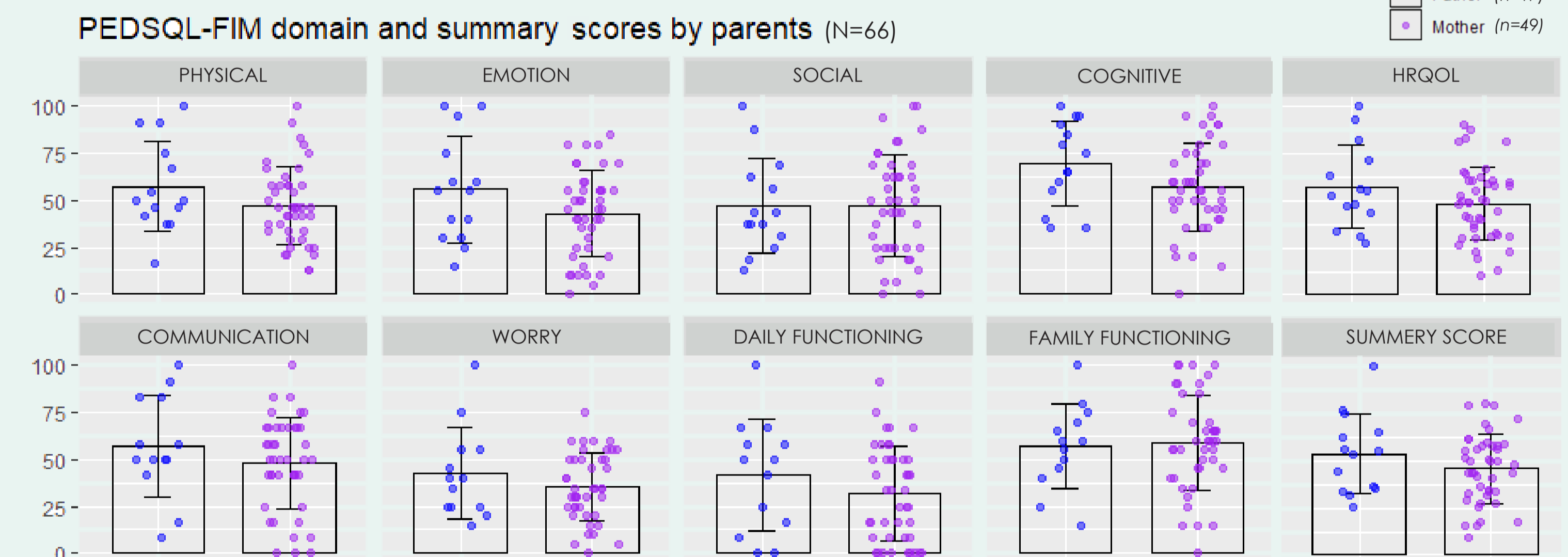
PedsQL- Self Completed



Discussion 1:

- Siblings aged 18-25 scored worse on all domains of the PedsQL-SC than other age categories.
- All siblings scored the worst on the emotional domain of the PedsQL-SC.

PEDSCL - Family Impact Module



Discussion 2:

- Fathers and mothers do not significantly differ on their domain and total scores.
- Both parents scored the worst on the daily functioning domain.

EQ5D-5L

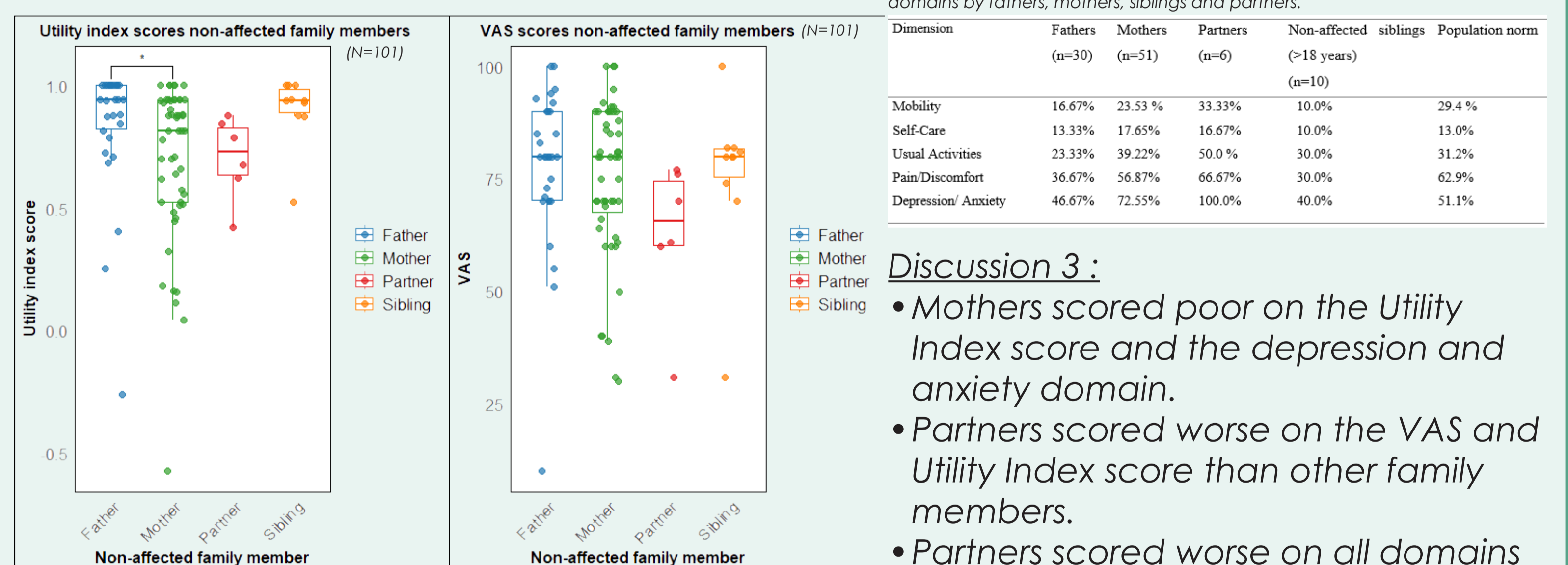


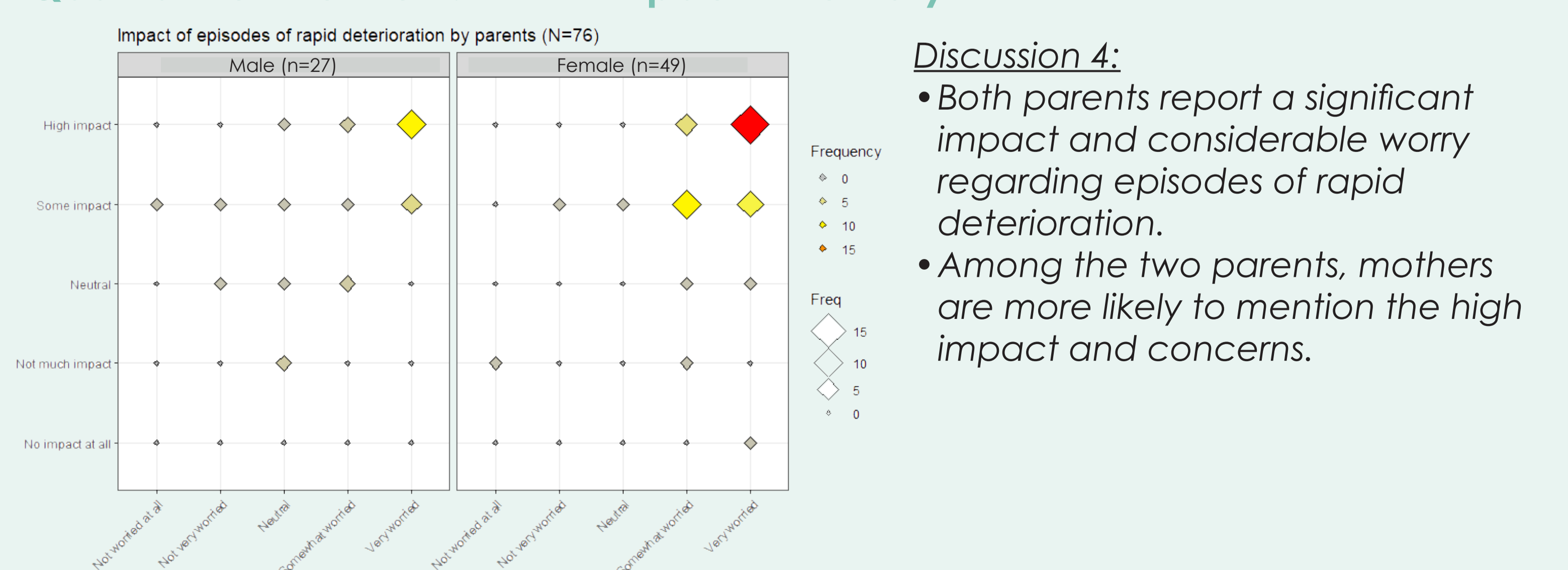
Table 1. The different percentages of reporting any problems within the EQ5D domains by fathers, mothers, siblings and partners.

Dimension	Fathers (n=30)	Mothers (n=51)	Partners (n=6)	Non-affected siblings (>18 years) (n=10)	Population norm
Mobility	16.67%	23.53%	33.33%	10.0%	29.4%
Self-Care	13.33%	17.65%	16.67%	10.0%	13.0%
Usual Activities	23.33%	39.22%	50.0%	30.0%	31.2%
Pain/Discomfort	36.67%	56.87%	66.67%	30.0%	62.9%
Depression/Anxiety	46.67%	72.55%	100.0%	40.0%	51.1%

Discussion 3:

- Mothers scored poor on the Utility Index score and the depression and anxiety domain.
- Partners scored worse on the VAS and Utility Index score than other family members.
- Partners scored worse on all domains than to the normal population.

Qualitative interview: VWM Impact inventory



Discussion 4:

- Both parents report a significant impact and considerable worry regarding episodes of rapid deterioration.
- Among the two parents, mothers are more likely to mention the high impact and concerns.