

Factors Associated with Decreased Quality of Life Scores in Adults with MEN1

Sneha Goswami BA, Benjamin Peipert BA, Susan Yount PhD, Cord Sturgeon MD

HealthMeasures User Conference
September 27, 2017
Presented by: Sneha Goswami

No financial disclosures



Multiple Endocrine Neoplasia type 1 (MEN1)

Background

- MEN1 is a genetic disease with **highly variable clinical manifestations**:¹
 - Primary hyperparathyroidism (PHPT; 95%)
 - Pancreatic neuroendocrine tumors (pNET; 30-80%)
 - Pituitary adenoma (15-90%)
- Recent studies indicate that **PHPT**² and **neuroendocrine tumors**³ are associated with decreased health-related quality of life (HRQOL).
- Very little is known about **HRQOL in MEN1** and the factors associated with poor HRQOL.
 - Only 2 prior studies total n = 79^{4,5}

1. Thakker RV et al. *J Clin Endocrinol Metab.* 2012;97(9):2990-3011.

2. Pasiaka JL et al. *World J Surg.* 2002;26(8):942-949.

3. Beaumont JL et al. *Pancreas.* 2012;41(3):461-466.

4. You YN et al. *Surgery.* 2007;142(6):829-836;

4. Berglund G et al. *Fam Cancer.* 2003;2(1):27-33.

Factors Associated with Decreased HRQOL in MEN1

Hypotheses

1. Individuals with MEN1 will report worse HRQOL than the general US population.
2. The following factors will be associated with worse HRQOL:
 - **Clinical factors**
 - Presence of pNETs or PHPT
 - Persistent hyperparathyroidism following surgery
 - Post-operative hypocalcemia
 - Number of pancreatic surgeries
 - Iatrogenic diabetes
 - **Treatment-related factors**
 - Frequency of doctor appointments
 - Travel distance for doctor appointments

Factors Associated with Decreased HRQOL in MEN1

Methods

- **Study population:** Adults (≥ 18 years) diagnosed with MEN1 were recruited online through an MEN1 support group (AMENSupport).
- **Online Survey:** two-part questionnaire
 - MEN1-specific questionnaire
 - HRQOL assessment: NIH Patient-Reported Outcomes Measurement Information System (PROMIS-29[®])
- **Statistical analysis**
 - One-sample T test was used to compare PROMIS T-scores of MEN1 patients to US normative data.
 - Mann-Whitney U test was used for subgroup analysis of categorical variables.
 - Bonferroni-Holms was used to correct for multiple comparisons.

HRQOL Assessment

NIH Patient-Reported Outcomes Measurement Information System
(PROMIS-29[®])



Mental

Physical

Social

7 PROMIS-29[®] Domains

Anxiety

Depression

Fatigue

Pain Impact

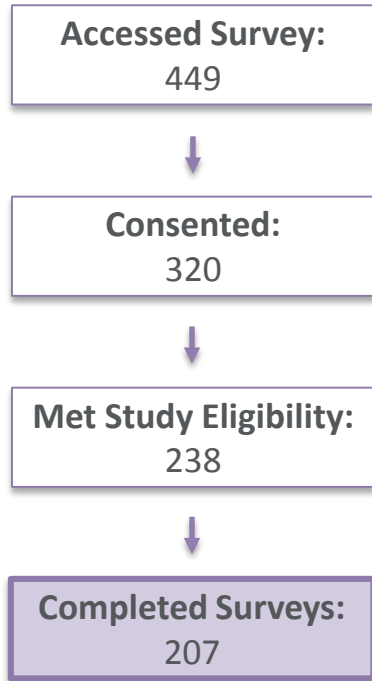
Sleep Disturbance

Physical Function

Social Function

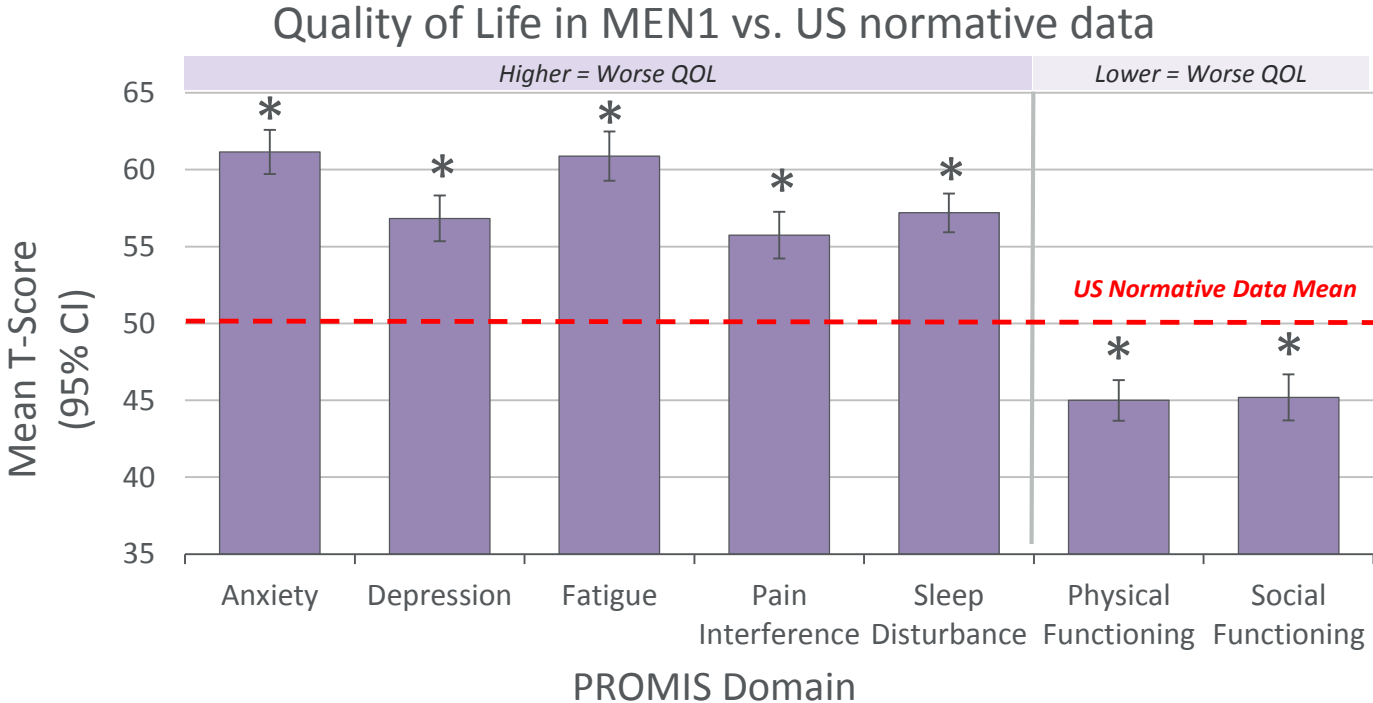
Results

Our Sample



Participant Demographics	Number (%/±SD)
Female	156 (76%)
Caucasian (Race)	200 (97%)
Country/Region of Origin	US: 153 (74%) Europe: 24 (11.6%) Canada: 18 (8.7%)
Mean Age	45 years (±12)
Clinical Characteristics	Number (%/±SD)
Mean Age at MEN-1 Diagnosis	34 years (±14)
Primary hyperparathyroidism	188 (91%)
History of pNET	154 (74%)

Result 1: MEN1 patients report worse QOL scores in all 7 PROMIS-29[®] domains than US normative data

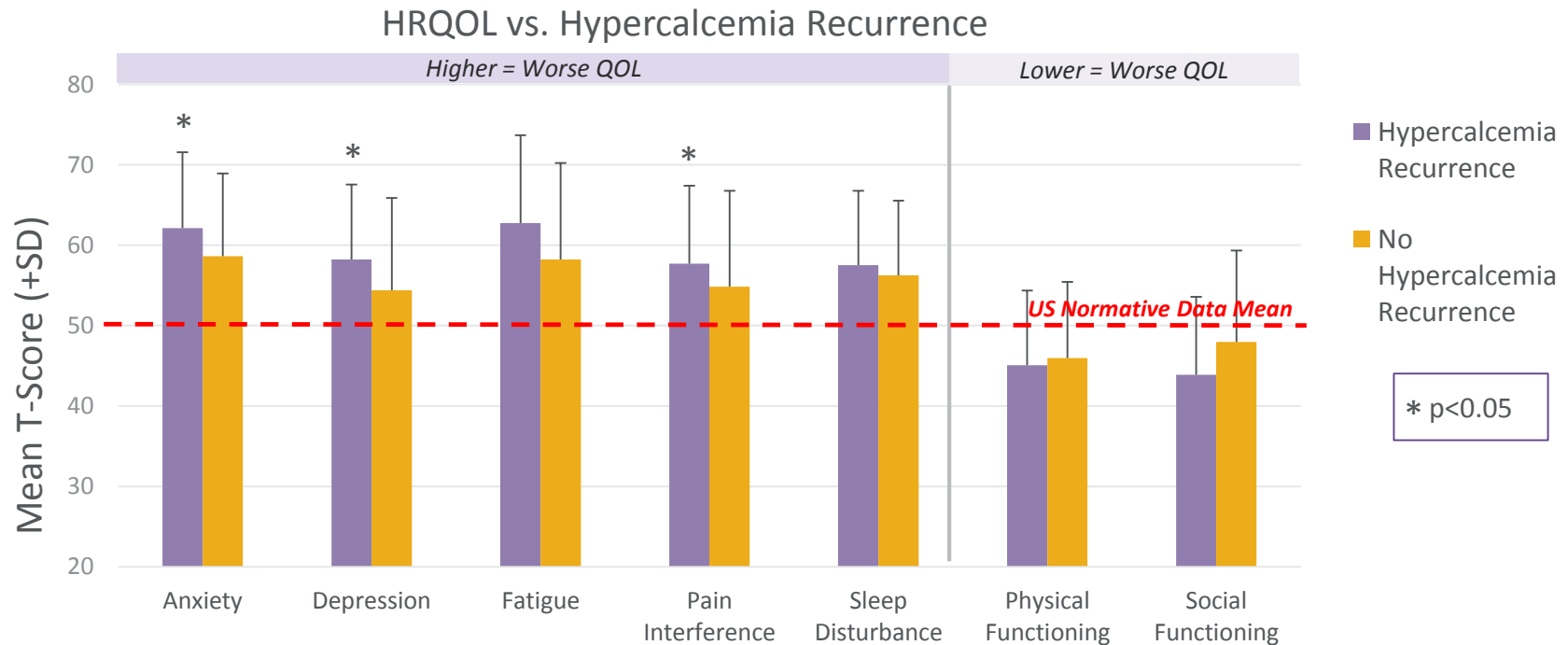


*p<0.05
across all
PROMIS
domains

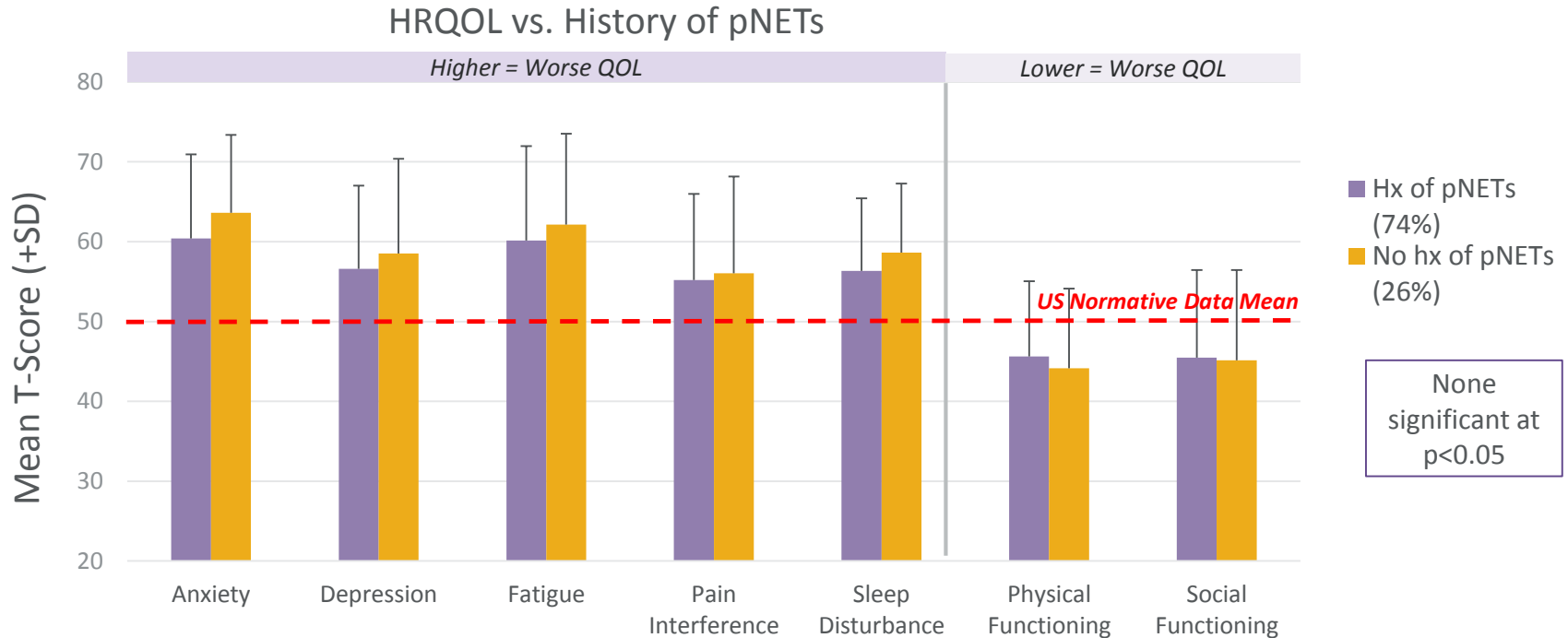
Results

Factors associated with decreased HRQOL scores in MEN1 patients

Result 2: Hypercalcemia recurrence is associated with worse HRQOL in 3/7 PROMIS-29[®] Domains

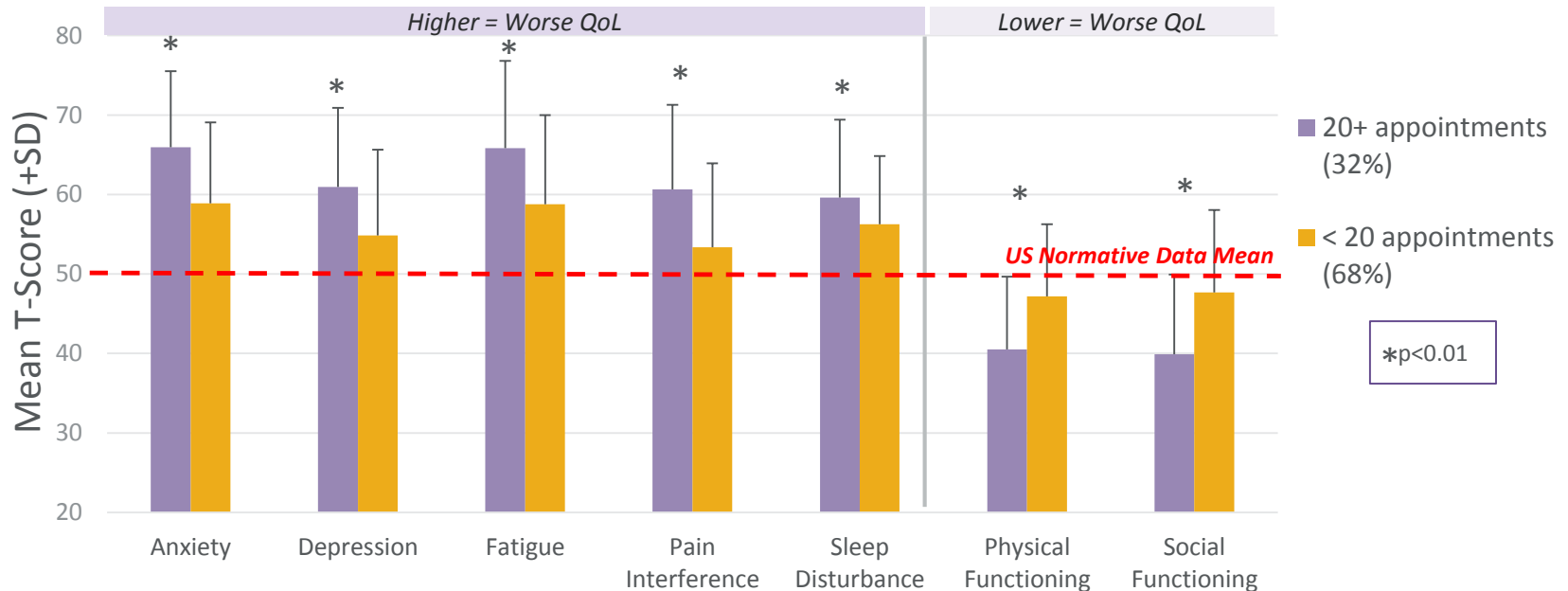


Result 3: History of pNET is not associated with HRQOL in any PROMIS-29[®] domain



Result 4: Increased frequency of doctor appointments (≥ 20 /year) is associated with worse HRQOL in all 7 PROMIS-29[®] Domains

HRQOL vs. Frequency of Doctor Appointments



Result 6: Factors that have no statistically significant association with HRQOL

PROMIS-29 Domains	Mean T-score			Mean T-score			Mean T-score		
	Pancreatic Surgery	Pancreatic Surgery (-)	P-value	Diabetes	Diabetes (-)	P-value	Sex (M)	Sex (F)	P-value
Anxiety	61.2	60.8	0.78	63.2	60.6	0.76	60.3	61.4	0.58
Depression	56.8	56.9	0.89	59.7	59.2	0.93	56.8	56.8	0.96
Fatigue	61.0	60.7	0.91	65.1	63.9	0.76	58.6	61.5	0.17
Physical Function	45.7	44.8	0.59	43.4	37.0	0.09	44.2	45.3	0.05
Pain	55.3	55.9	0.78	59.8	63.7	0.42	56.8	55.3	0.53
Sleep	57.2	57.2	0.95	58.8	57.5	0.89	55.2	57.7	0.04
Social Roles	45.5	45.1	0.89	43.4	43.5	0.81	44.6	45.5	0.58

*Unadjusted p-values displayed. No p-values significant after Bonferroni-Holms correction.

Factors Associated with Decreased HRQOL in MEN1

Conclusions

- Individuals with MEN1 report **worse HRQOL** compared to the US population across **all 7 PROMIS-29 domains**.
- **Recurrent hypercalcemia** following parathyroid surgery is strongly associated with decreased HRQOL.
- Having **≥20 doctor appointments/year** is associated with decreased HRQOL.
- MEN1 patients with a history of **pNET** report similar HRQOL as those without pNET.

Factors Associated with Decreased HRQOL in MEN1

Study Limitations

- Selection bias is inherent in this study due to the recruitment method.
 - Males and minorities were underrepresented.
- Several tumor types (including anterior pituitary) were intentionally omitted in efforts to minimize the length of the survey.
- Patient privacy was protected by making the survey anonymous. Consequently, patient responses could not be linked to any clinical dataset.

Acknowledgments



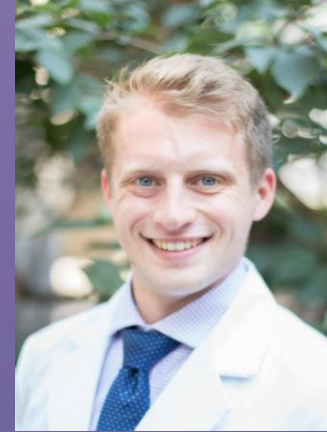
Cord Sturgeon, MD



Susan Yount, PhD



Irene Helenowski, PhD



Benjamin Peipert,
Medical Student

Northwestern University
Feinberg School of Medicine
Department of Surgery

