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

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Presented by: Sneha Goswami
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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 PHPT² and neuroendocrine tumors³ 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 = 79⁴,⁵

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®)

7 PROMIS-29® Domains

- Anxiety
- Depression
- Fatigue
- Pain Impact
- Sleep Disturbance
- Physical Function
- Social Function
Results
### Participant Demographics

<table>
<thead>
<tr>
<th></th>
<th>Number (%/±SD)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Female</td>
<td>156 (76%)</td>
</tr>
<tr>
<td>Caucasian (Race)</td>
<td>200 (97%)</td>
</tr>
<tr>
<td>Country/Region of Origin</td>
<td></td>
</tr>
<tr>
<td>US</td>
<td>153 (74%)</td>
</tr>
<tr>
<td>Europe</td>
<td>24 (11.6%)</td>
</tr>
<tr>
<td>Canada</td>
<td>18 (8.7%)</td>
</tr>
<tr>
<td>Mean Age</td>
<td>45 years (±12)</td>
</tr>
</tbody>
</table>

### Clinical Characteristics

<table>
<thead>
<tr>
<th></th>
<th>Number (%/±SD)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mean Age at MEN-1 Diagnosis</td>
<td>34 years (±14)</td>
</tr>
<tr>
<td>Primary hyperparathyroidism</td>
<td>188 (91%)</td>
</tr>
<tr>
<td>History of pNET</td>
<td>154 (74%)</td>
</tr>
</tbody>
</table>
Result 1: MEN1 patients report worse QOL scores in all 7 PROMIS-29® domains than US normative data

### Quality of Life in MEN1 vs. US normative data

<table>
<thead>
<tr>
<th>PROMIS Domain</th>
<th>US Normative Data Mean</th>
<th>Higher = Worse QOL</th>
<th>Lower = Worse QOL</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anxiety</td>
<td>55</td>
<td>*</td>
<td></td>
</tr>
<tr>
<td>Depression</td>
<td>45</td>
<td>*</td>
<td></td>
</tr>
<tr>
<td>Fatigue</td>
<td>50</td>
<td>*</td>
<td></td>
</tr>
<tr>
<td>Pain</td>
<td>45</td>
<td>*</td>
<td></td>
</tr>
<tr>
<td>Sleep</td>
<td>55</td>
<td>*</td>
<td></td>
</tr>
<tr>
<td>Physical Functioning</td>
<td>40</td>
<td>*</td>
<td></td>
</tr>
<tr>
<td>Social Functioning</td>
<td>40</td>
<td>*</td>
<td></td>
</tr>
</tbody>
</table>

Higher = Worse QOL
Lower = Worse QOL

* *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

HRQOL vs. History of pNETs

Higher = Worse QOL
Lower = Worse QOL

Mean T-Score (+SD)

Anxiety | Depression | Fatigue | Pain Interference | Sleep Disturbance | Physical Functioning | Social Functioning

Hx of pNETs (74%)
No hx of pNETs (26%)

None significant at p<0.05
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

Higher = Worse QoL  
Lower = Worse QoL

<table>
<thead>
<tr>
<th>Domain</th>
<th>Mean T-Score (+SD)</th>
<th>20+ appointments (32%)</th>
<th>&lt; 20 appointments (68%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anxiety</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Depression</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Fatigue</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Pain Interference</td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>Sleep Disturbance</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Physical Functioning</td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>Social Functioning</td>
<td></td>
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</tbody>
</table>

* p<0.01
Result 6: Factors that have no statistically significant association with HRQOL

<table>
<thead>
<tr>
<th>PROMIS-29 Domains</th>
<th>Mean T-score</th>
<th>Mean T-score</th>
<th>Mean T-score</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Pancreatic Surgery</td>
<td>Pancreatic Surgery (-)</td>
<td>P-value</td>
</tr>
<tr>
<td>Anxiety</td>
<td>61.2</td>
<td>60.8</td>
<td>0.78</td>
</tr>
<tr>
<td>Depression</td>
<td>56.8</td>
<td>56.9</td>
<td>0.89</td>
</tr>
<tr>
<td>Fatigue</td>
<td>61.0</td>
<td>60.7</td>
<td>0.91</td>
</tr>
<tr>
<td>Physical Function</td>
<td>45.7</td>
<td>44.8</td>
<td>0.59</td>
</tr>
<tr>
<td>Pain</td>
<td>55.3</td>
<td>55.9</td>
<td>0.78</td>
</tr>
<tr>
<td>Sleep</td>
<td>57.2</td>
<td>57.2</td>
<td>0.95</td>
</tr>
<tr>
<td>Social Roles</td>
<td>45.5</td>
<td>45.1</td>
<td>0.89</td>
</tr>
</tbody>
</table>

*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

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