Symptom Clusters for revising scale membership for the analysis of Patient Reported Outcome Measures

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Topics of this talk

• Introduce the resources
  – RT01
  – UCLA-PCI

• Role of Symptom Clusters
  – Revising scale membership
  – Meaningful way of summarising PROMs
  – Extracting important information for the analysis and interpretation

• Sensitivity of PROMs
  – Ceiling effect
  – Missing data
  – Trends and implications
The issue (value of PROs vs complexity of PROs)

• **Value of proms**
  – Patient perspective
  – Systematic collection of information over time
  – Increasing routine use of PROMs in clinical setting

• **Complexity of PROMs**
  – Number of questions
  – Different scales, dimensions, domains, scoring levels
  – Relevance of PROMs and missing data

• **Summarising of PROMs data**
  – Required to extract important information within PROMs
  – Tool specific validated guidance on scoring, coding and summing-up
  – Averaging items within domains or subscales (SF-36, UCLA-PCI, EPIC)
  – Sum of scores, maximum or minimum score (IPSS, ICIQ-UI-SF)
  – Health status converted into a single index value (EQ-5D)
Resources used to illustrate this study

MRC RT01 dataset (secondary analysis)

- Large, multicentre UK RCT coordinated for the Medical Research Council (MRC)
- 843 patients with localised prostate cancer
- Randomised to standard (64 Gy) or escalated (74 Gy) dose of conformal radiotherapy with neoadjuvant androgen suppression
- PROMs data collected longitudinally with the UCLA-PCI
- 10 time points (pre-hormone therapy, pre-radiotherapy, week 10, months 6, 12 and 18, years 2, 3, 4 and 5)

Resources used to illustrate this study

UCLA-PCI

URINARY FUNCTION
This section is about your urinary habits. Please consider ONLY THE LAST 4 WEEKS.

12. Over the past 4 weeks, how often have you leaked urine?
   - Every day .................................. 1
   - About once a week ....................... 2 (Circle one number)
   - Less than once a week ................. 3
   - Not at all .................................. 4

13. Which of the following best describes your urinary control during the last 4 weeks?
   - No control whatsoever .............. 1
   - Frequent dribbling ..................... 2 (Circle one number)
   - Occasional dribbling ................. 3
   - Total control ............................. 4

14. How many pads or adult diapers per day did you usually use to control leakage during the last 4 weeks?
   - 3 or more pads per day .............. 1
   - 1-2 pads per day ....................... 2 (Circle one number)
   - No pads .................................. 3

15. How big a problem, if any, has each of the following been for you? (Circle one number on each line)
   - Dripping urine or wetting your pants ............ 1 2 3 4 5
   - Urine leakage interfering with your sexual activity ............ 1 2 3 4 5

16. Overall, how big a problem has your urinary function been for you during the last 4 weeks?
   - No problem .................................. 1
   - Very small problem ..................... 2
   - Small problem ............................ 3 (Circle one number)
   - Moderate problem ..................... 4
   - Big problem ............................... 5

Figure 1. Longitudinal profiles of the Urinary Domain of Health, UCLA-PCI. The time profiles of mean scores with the 95% confidence intervals.

## Correlation of items

<table>
<thead>
<tr>
<th></th>
<th>Urinary leak</th>
<th>Urinary control</th>
<th>Number of pads</th>
<th>Dripping/wetting</th>
<th>Urinary leak interf with sex</th>
<th>Urinary bother</th>
</tr>
</thead>
<tbody>
<tr>
<td>Urinary leak</td>
<td>1</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Urinary control</td>
<td>0.753</td>
<td>1</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Number of pads</td>
<td>0.214</td>
<td>0.202</td>
<td>1</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Dripping/wetting</td>
<td>0.698</td>
<td>0.694</td>
<td>0.209</td>
<td>1</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Urinary leak interf with sex</td>
<td>0.326</td>
<td>0.306</td>
<td>0.200</td>
<td>0.389</td>
<td>1</td>
<td></td>
</tr>
<tr>
<td>Urinary bother</td>
<td>0.505</td>
<td>0.549</td>
<td>0.168</td>
<td>0.527</td>
<td>0.271</td>
<td>1</td>
</tr>
</tbody>
</table>

### Table 1. Pearson correlation, Urinary Domain of the UCLA-PCI

Summarizing PROMs data

Concept / Context
- To extract meaningful and relevant information
- Grouping of PROMs items is study/sample specific
- Multiple-item scales should be evaluated

Generic approach
- Items grouped according to the predefined domains
- Items with different prevalence or trends are included
- Loss of sensitivity and specificity

Sample specific / Smart
- Increase in sensitivity and specificity
- Highly correlated items are grouped
- Items of similar prevalence and trends
Missing data – intermittent

<table>
<thead>
<tr>
<th>Time point</th>
<th>Urinary leak</th>
<th>Urinary control</th>
<th>Dripping /Wetting</th>
<th>Number of pads</th>
<th>Interference with sex</th>
<th>Urinary bother</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pre-hormone therapy (n = 578)</td>
<td>4 (0.7)</td>
<td>5 (0.9)</td>
<td>8 (1.4)</td>
<td>6 (1.0)</td>
<td>49 (8.5)</td>
<td>2 (0.3)</td>
</tr>
<tr>
<td>Pre-radiotherapy (n = 757)</td>
<td>10 (1.3)</td>
<td>11 (1.5)</td>
<td>18 (2.4)</td>
<td>13 (1.7)</td>
<td>93 (12.3)</td>
<td>9 (1.2)</td>
</tr>
<tr>
<td>Week 10 (n = 738)</td>
<td>16 (2.2)</td>
<td>16 (2.2)</td>
<td>19 (2.6)</td>
<td>22 (3.0)</td>
<td>131 (17.8)</td>
<td>14 (1.9)</td>
</tr>
<tr>
<td>Month 6 (n = 712)</td>
<td>26 (3.7)</td>
<td>25 (3.5)</td>
<td>31 (4.3)</td>
<td>33 (4.6)</td>
<td>107 (15.0)</td>
<td>24 (3.4)</td>
</tr>
<tr>
<td>Year 1 (n = 689)</td>
<td>15 (2.2)</td>
<td>14 (2.0)</td>
<td>17 (2.5)</td>
<td>21 (3.0)</td>
<td>77 (11.2)</td>
<td>12 (1.7)</td>
</tr>
<tr>
<td>Month 18 (n = 655)</td>
<td>17 (2.6)</td>
<td>16 (2.4)</td>
<td>18 (2.7)</td>
<td>17 (2.6)</td>
<td>103 (15.7)</td>
<td>17 (2.6)</td>
</tr>
<tr>
<td>Year 2 (n = 645)</td>
<td>31 (4.8)</td>
<td>34 (5.3)</td>
<td>37 (5.7)</td>
<td>33 (5.1)</td>
<td>98 (15.2)</td>
<td>31 (4.8)</td>
</tr>
<tr>
<td>Year 3 (n = 594)</td>
<td>23 (3.9)</td>
<td>25 (4.2)</td>
<td>32 (5.4)</td>
<td>28 (4.7)</td>
<td>84 (14.1)</td>
<td>25 (4.2)</td>
</tr>
<tr>
<td>Year 4 (n = 515)</td>
<td>27 (5.2)</td>
<td>27 (5.2)</td>
<td>30 (5.8)</td>
<td>31 (6.0)</td>
<td>77 (15.0)</td>
<td>27 (5.2)</td>
</tr>
<tr>
<td>Year 5 (n = 425)</td>
<td>20 (4.7)</td>
<td>23 (5.4)</td>
<td>22 (5.2)</td>
<td>21 (4.9)</td>
<td>69 (16.2)</td>
<td>20 (4.7)</td>
</tr>
</tbody>
</table>

Table 3. Missing data, Urinary Domain of the UCLA-PCI: n (%).

## Missing data – intermittent

<table>
<thead>
<tr>
<th>Rank</th>
<th>Item name</th>
<th>Missing (%)</th>
<th>Rank</th>
<th>Item name</th>
<th>Missing (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td><strong>Urine leak interfering with sex</strong></td>
<td>14.1</td>
<td>11</td>
<td>Quality of erections</td>
<td>4.9</td>
</tr>
<tr>
<td>2</td>
<td>Ability to reach orgasm</td>
<td>8.6</td>
<td>12</td>
<td>As healthy as anyone*</td>
<td>4.6</td>
</tr>
<tr>
<td>3</td>
<td>Ability to have erection</td>
<td>6.7</td>
<td>13</td>
<td>Vaginal/anal intercourse</td>
<td>4.0</td>
</tr>
<tr>
<td>4</td>
<td>Frequency of erections</td>
<td>6.6</td>
<td>14</td>
<td><strong>Dripping/wetting</strong></td>
<td>3.8</td>
</tr>
<tr>
<td>5</td>
<td>Health get worse*</td>
<td>6.2</td>
<td>15</td>
<td>Awakened with an erection</td>
<td>3.8</td>
</tr>
<tr>
<td>6</td>
<td>Sexual function bother</td>
<td>6.0</td>
<td>16</td>
<td><strong>Number of pads</strong></td>
<td>3.7</td>
</tr>
<tr>
<td>7</td>
<td>Health is excellent*</td>
<td>5.9</td>
<td>17</td>
<td><strong>Urinary control</strong></td>
<td>3.3</td>
</tr>
<tr>
<td>8</td>
<td>Get sick easier*</td>
<td>5.9</td>
<td>18</td>
<td>Urinary leak</td>
<td>3.1</td>
</tr>
<tr>
<td>9</td>
<td>Rate sexual function</td>
<td>5.9</td>
<td>19</td>
<td>Urinary function bother</td>
<td>3.0</td>
</tr>
<tr>
<td>10</td>
<td>Sexual desire</td>
<td>4.9</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Table 4. Missing data, first 19 items of UCLA-PCI (including SF-36*) ranked in order of higher % of intermittent missing data (MRC RT01 data)
Summary and future work

• Symptom grouping is population/trial specific
• Symptom scales should be revised prior to data analysis and interpretation
• Trends in missing data and ceiling effect should be investigated
• Prevention of missing data and ceiling effect includes
  – Easy and relevant PROMs questions
  – Patient feedback and involvement

• Next steps
  – Explore symptom grouping and scale membership in EPIC
Any questions ???