Doing More With Outcomes: Lessons from the Parkinson’s Outcomes Project

Peter Schmidt, Ph.D.
Science from Art

“Once a treatment enters the mainstream, science is left behind. The next questions — when to use it and on which patients — become matters of judgment, not measurement.”
“Incidentally, psychoanalysis is not a science; it is at best a medical process, and perhaps more like witch-doctoring....”
“If you are sick, I would advise that you go to the witch doctor because he is the man in the tribe who knows the most about the disease; on the other hand, his knowledge is not science.”

Richard Feynman
Nobel Prize, Physics, 1965
Success Here Isn’t Enough
Developing Improvement Science

- Cystic fibrosis

1964 CF median survival: 3 years
USA
Matthews Clinic
21 years
Improved risk with neurologist care
The Parkinson’s Outcomes Project

• 7+ years
• 14,000+ participants
• 20,000+ evaluations
• 20+ interventions
• 6 outcomes

• Goal: To find and understand the very best care so that we can teach others to provide it.
Age at Onset and Diagnosis

- Age at diagnosis
- Age at onset

Years: 30, 40, 50, 60, 70, 80, 90

692 YOPD
Across the Spectrum

713 newly diagnosed

Disease duration

427 with 20+ years of PD

Years

Number of Individuals

Recruitment
Most recent visit
NPF’s Parkinson’s Outcomes Project

Demographics

Social

Clinical evaluation

Interventions

Patient-reported outcomes
Very Simple Tests...

<table>
<thead>
<tr>
<th>Timed up &amp; go test (TUG)</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Without pushing off</td>
<td>5-30 in whole seconds; 30=unable to do; 999=not tested</td>
</tr>
<tr>
<td>If unable without pushing off, then pushing off from chair</td>
<td>5-30 in whole seconds; 30=unable to do; 999=not tested</td>
</tr>
<tr>
<td>If unable without assist device, then using cane or walker</td>
<td>5-30 in whole seconds; 30=unable to do; 999=not tested</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Cognition</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Immediate 5 word recall</td>
<td>0-5 words; 999=not tested</td>
</tr>
<tr>
<td>Verbal fluency (animal list)</td>
<td># of words; 999=not tested</td>
</tr>
<tr>
<td>Delayed 5 word recall</td>
<td>0-5 words; 999=not tested</td>
</tr>
</tbody>
</table>
Insight from Segments

You can’t “adjust” this away.
Approach to Measurement

- Clinical Value Compass (Nelson, et al., 1996)
Learning to Fight Parkinson’s
Understanding Outcomes
Are There Outliers?

Regression to the mean?

Baseline status (z-score)

Negative outliers

Overall 6 outcome score

2-year delta (z-score)
Are There Outliers?

Overall 6 outcome score

Subtype?

Baseline status (z-score)

2-year delta (z-score)
Comparing Centers

• Goals for case-mix comparisons:
  – Minimal adjustment: results $\sim$ reality
  – Map results to standardized domain so that we can look across outcomes
  – Comparisons inform further investigation, they are not answers
Case Mix: Sex and Comorbidities

• For each outcome, adjust individual results for sex and comorbidities as categorical variables.
## Center Summary Score

### Timed up-and-go results

<table>
<thead>
<tr>
<th>Age quintiles</th>
<th>Center I</th>
<th>Duration quintiles</th>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td></td>
<td></td>
<td>9.9</td>
<td>8.8</td>
<td>11.2</td>
<td>10.3</td>
<td>12.2</td>
</tr>
<tr>
<td>2</td>
<td></td>
<td></td>
<td>9.2</td>
<td>9.2</td>
<td>11.1</td>
<td>10.6</td>
<td>11.2</td>
</tr>
<tr>
<td>3</td>
<td></td>
<td></td>
<td>9.5</td>
<td>11.4</td>
<td>9.6</td>
<td>13.8</td>
<td>15.0</td>
</tr>
<tr>
<td>4</td>
<td></td>
<td></td>
<td>11.8</td>
<td>13.5</td>
<td>16.1</td>
<td>15.0</td>
<td>17.5</td>
</tr>
<tr>
<td>5</td>
<td></td>
<td></td>
<td>15.7</td>
<td>20.3</td>
<td>13.6</td>
<td>19.3</td>
<td>20.9</td>
</tr>
</tbody>
</table>

### Overall frequency by strata

<table>
<thead>
<tr>
<th>Total Cohort</th>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>4.6%</td>
<td>4.4%</td>
<td>4.5%</td>
<td>3.3%</td>
<td>3.1%</td>
</tr>
<tr>
<td>2</td>
<td>4.4%</td>
<td>3.5%</td>
<td>3.8%</td>
<td>4.3%</td>
<td>4.0%</td>
</tr>
<tr>
<td>3</td>
<td>3.8%</td>
<td>4.3%</td>
<td>3.7%</td>
<td>4.1%</td>
<td>4.3%</td>
</tr>
<tr>
<td>4</td>
<td>3.6%</td>
<td>4.0%</td>
<td>4.1%</td>
<td>3.9%</td>
<td>4.5%</td>
</tr>
<tr>
<td>5</td>
<td>3.6%</td>
<td>3.8%</td>
<td>4.0%</td>
<td>4.4%</td>
<td>4.2%</td>
</tr>
</tbody>
</table>

Population weighted average: 13.1 seconds
Comparing Centers

Each dot represents one center’s weighted average.

Distribution of total population results.

TUG score

0 5 10 15 20 25 30
Comparing Across Outcomes

Overall Results, 2009-2016

z-scores; higher is better

TUG
PDQ-39
Cognition
Hospitalization
Caregiver strain
Falls

Center E
Center J
Center G: Leader in Cognition

Cognitive score: higher is better
Center A: Leader in Falls

Cognitive score higher is better →

Falls

2012 2014 2016

Center A
Looking at All Outcomes

TUG
- TUG: 2012-2014
- TUG: 2009-2012
- Center Q TREND

PDQ-39
- PDQ-39: 2012-2014
- Center M TREND

Caregiver strain
- Caregiver strain: 2014-2016
- Caregiver strain: 2012-2014
- Caregiver strain: 2009-2012
- Center E TREND

Hospitalization
- Hospitalization: 2012-2014
- Hospitalization: 2009-2012
- Center H TREND
Exercise: Changing the Disease Course

SAN DIEGO -- Parkinson's patients who exercise regularly have slower declines in quality of life, researchers reported here. In an analysis of data from the National Parkinson Foundation database, patients who reported exercising at least 2.5 hours...
Next Steps

Baseline status (z-score)

Overall 6 outcome score

Negative outliers

2-year delta (z-score)
Variance: Mobility

Baseline status (z-score)

2-year delta (z-score)

TUG score
Variance: Mobility at Center 5

TUG score

2D SD metric: 1.9
Variance: Mobility at Center 9

Baseline status (z-score)

2-year delta (z-score)

TUG score

2D SD metric: 0.97
Where Would You Run Your Motor Trial?

**Center 5**
Highly predictable longitudinal outcomes

**Center 9**
Highly variable longitudinal outcomes
Precision Care

• Improving outcomes while improving science
• Alternate/supplement to biomarkers?

Accuracy

Precision
Thank You

“Mathematics is not a science from our point of view…”