From experience to quantification to co-production: reflections on patient reported outcomes in an online patient community

Paul Wicks, PhD
VP of Innovation
Amyotrophic Lateral Sclerosis (ALS) / Motor Neurone Disease (MND)
Amyotrophic Lateral Sclerosis
2007, iFirst Article, 1–7

Neuronal loss associated with cognitive performance in amyotrophic lateral sclerosis: An \(^{11}\text{C}\)-flumazenil PET study
Mid-2000’s Internet (Pre Social Media)

• Specific Websites
  – Bulletin boards / chatrooms
  – Moderated, more user-driven
  – Informal links to organizations

• Users
  – Diverse mix of users not just techies
  – Some monitoring but no interaction w/HCPs
  – Self-taught medical / scientific knowledge

• Technology
  – Dialup modems (56k & ISDN)
  – Decreasing use of fax, “snail mail”
  – Unlimited access net plans (AOL)
  – Dot-com boom
Emerging Patient-Driven Health Care Models: An Examination of Health Social Networks, Consumer Personalized Medicine and Quantified Self-Tracking

Melanie Swan *
Step 1: Create/update and share your health profile

Step 2: Find support from others like you and compare experiences

Step 3: Learn from aggregated community Treatment and Symptom Reports

Step 4: Take profile to your doctor to have an improved treatment conversation

Step 5: Play an integral part in your own health care
Who has ALS at PatientsLikeMe?

- **5,610** patients have this condition
- **50** New patients joined this month
- **5,557** say ALS is their primary condition

**What do patients take** to treat ALS and its symptoms?

<table>
<thead>
<tr>
<th>Treatment Name</th>
<th>Perceived Effectiveness</th>
<th>Overall Rating of Side Effects</th>
<th># of Evaluations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Riluzole</td>
<td></td>
<td></td>
<td>284</td>
</tr>
<tr>
<td>Lithium Carbonate</td>
<td></td>
<td></td>
<td>86</td>
</tr>
<tr>
<td>CoQ10 (CoEnzyme Q10)</td>
<td></td>
<td></td>
<td>53</td>
</tr>
<tr>
<td>Baclofen</td>
<td></td>
<td></td>
<td>36</td>
</tr>
<tr>
<td>Vitamin E</td>
<td></td>
<td></td>
<td>35</td>
</tr>
<tr>
<td>ALS Phase 2 Study NP001</td>
<td></td>
<td></td>
<td>28</td>
</tr>
<tr>
<td>ALS Phase 3 Study</td>
<td></td>
<td></td>
<td>26</td>
</tr>
<tr>
<td>Dexamphetamine (KNS-760704)</td>
<td></td>
<td></td>
<td>26</td>
</tr>
<tr>
<td>Sodium chlorite</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
What is Riluzole?

Riluzole is the first drug to be approved for amyotrophic lateral sclerosis (ALS). Riluzole, available as Rilutek®, does not cure ALS or improve symptoms, but may extend survival or the time to tracheostomy.

Reported Purpose & Perceived Effectiveness

<table>
<thead>
<tr>
<th>Purpose</th>
<th># of patients</th>
<th># of patients with evaluations</th>
<th>Perceived Effectiveness</th>
</tr>
</thead>
<tbody>
<tr>
<td>ALS (Amyotrophic Lateral Sclerosis)</td>
<td>1,476</td>
<td>299</td>
<td></td>
</tr>
<tr>
<td>PMA (Progressive Muscular Atrophy)</td>
<td>29</td>
<td>13</td>
<td></td>
</tr>
<tr>
<td>PLS (Primary Lateral Sclerosis)</td>
<td>25</td>
<td>7</td>
<td></td>
</tr>
<tr>
<td>General health</td>
<td>14</td>
<td>5</td>
<td></td>
</tr>
<tr>
<td>Stiffness/Spasticity</td>
<td>8</td>
<td>0</td>
<td></td>
</tr>
<tr>
<td>Fasciculations</td>
<td>7</td>
<td>2</td>
<td></td>
</tr>
</tbody>
</table>

See all 17 reasons taken ▼

Mouse over the table for more information

Reported Side Effects

<table>
<thead>
<tr>
<th>Side effects as an overall problem</th>
<th>Commonly reported side effects, conditions, and hospitalizations associated with Riluzole</th>
</tr>
</thead>
<tbody>
<tr>
<td>Severe</td>
<td>Fatigue 30, Nausea 18, Tiredness 9, Stomach pain 9, Elevated liver enzymes 8, Abdominal pain 8</td>
</tr>
<tr>
<td>Moderate</td>
<td></td>
</tr>
<tr>
<td>Mild</td>
<td></td>
</tr>
<tr>
<td>None</td>
<td></td>
</tr>
</tbody>
</table>

See all 74 reported side effects ▼
5 Phases of PRO development

**Qualitative phase**

- Concept Elicitation
  - To obtain input from patients
  - Opportunity to examine patient experience at a large scale
  - Item generation process
  - Based on open-ended questions

- Feedback
  - Equivalent to cognitive debriefing
    - To evaluate clarity, relevance, and adequacy of response options for each item
    - Item review based on patient feedback
    - 4 feedback questions are displayed below each evaluated item

**Quantitative/psychometric phase**

- Test
  - Psychometric evaluation of the new instrument (validity and reliability)
    - Flexible study design
    - Ongoing patient input available through item-level and post-survey feedback

- Retest

- Follow up
  - To evaluate the ability to detect change
ALS patient noticed ALSFRS-R wasn't sensitive enough to capture function in advanced ALS, “floor effect” of measure

200+ patients participated in study to construct and pilot a new, more sensitive instrument

3 new items were selected to be included in the new ALSFRS-EX measure

Being used by the VA biobank and academic studies
Challenges in PRO Development Today

- Slow (2-4 years) & expensive to develop ($725k-$2.1m)
- Only available in few diseases
- Typically license fee for use
- Many lack patient input in design
- Outdated e.g. home shopping, internet, smartphones


Open Research Exchange (ORE)

• A PRO builder toolset modeled on open source software
• Includes versioning, branching, contributions, feedback
• **Social Architecture** for collaboration, credentialing, credit
• **Automated psychometric statistics** for PRO improvement
• **Database of instruments** and supporting data
• **Scientific support** from experts at PatientsLikeMe & SAB
Patient-Relevant Concepts in Chronic Lymphocytic Leukemia (Janssen)

- 50 adults with CLL recruited via PatientsLikeMe
- 78% reported at least one symptom
- 369 descriptions of CLL symptoms including fatigue (40%), tiredness (38%), night sweats (38%), swollen lymph nodes (32%)
- Supplemental telephone interviews included for a subset
- Concept saturation was achieved using the web-only technique

Ovarian Cancer – “In your own words” (AstraZeneca)

- 30 adults with Ovarian cancer recruited via PatientsLikeMe
- Symptoms leading to diagnosis, patient journey
- Symptom fluctuations, worst symptom over course
- Impact of treatment(s) and description of follow-up care
Patient-Level Data Display
# Real-time Psychometric Stats

**View results for**: Study 1 - Test of Psoriasis Survey

**N = 357 of 400 invited: Multiple Sclerosis, No Diabetes, Ages 18-65, 207 M / 193 F, USA, MSRS-R 7.0-10.0, Dx Date 3+ yrs**

## Group Descriptive Results

**Q1: STIFF1**  
**Domain: Stiffness**  
**Avg. time: 17 s**

**How long have you experienced joint stiffness?**

<table>
<thead>
<tr>
<th>Response option</th>
<th>Code</th>
<th>Freq.</th>
<th>%</th>
<th>Cum. freq.</th>
<th>Cum. %</th>
<th>Avg. time</th>
</tr>
</thead>
<tbody>
<tr>
<td>Less than 1 week</td>
<td>0</td>
<td>228</td>
<td>15.49%</td>
<td>228</td>
<td>15.49%</td>
<td>12 s</td>
</tr>
<tr>
<td>Less than one month</td>
<td>1</td>
<td>339</td>
<td>23.02%</td>
<td>765</td>
<td>53.41%</td>
<td>19 s</td>
</tr>
<tr>
<td>Less than one year</td>
<td>2</td>
<td>371</td>
<td>26.07%</td>
<td>1126</td>
<td>75.24%</td>
<td>15 s</td>
</tr>
<tr>
<td>Less than 5 years</td>
<td>3</td>
<td>254</td>
<td>17.26%</td>
<td>1446</td>
<td>96.74%</td>
<td>15 s</td>
</tr>
<tr>
<td>More than 5 years</td>
<td>4</td>
<td>254</td>
<td>17.26%</td>
<td>1700</td>
<td>95.25%</td>
<td>16 s</td>
</tr>
<tr>
<td>I prefer to skip</td>
<td>-97</td>
<td>10</td>
<td>2.31%</td>
<td>1710</td>
<td>97.31%</td>
<td>5 s</td>
</tr>
<tr>
<td>I don't know</td>
<td>-98</td>
<td>5</td>
<td>1.65%</td>
<td>1715</td>
<td>100.00%</td>
<td>12 s</td>
</tr>
<tr>
<td>Does not apply</td>
<td>-99</td>
<td>0</td>
<td>0.00%</td>
<td>0</td>
<td>100.00%</td>
<td>---</td>
</tr>
<tr>
<td>Forced skip</td>
<td>-100</td>
<td>0</td>
<td>0.00%</td>
<td>0</td>
<td>100.00%</td>
<td>---</td>
</tr>
<tr>
<td>Did not complete</td>
<td>-999</td>
<td>0</td>
<td>0.00%</td>
<td>0</td>
<td>100.00%</td>
<td>---</td>
</tr>
</tbody>
</table>

## Quantitative Feedback Responses

**Does it apply?**
- Very well: 5 (27.7)
- Reasonably well: 8 (44.4)
- A little bit: 3 (16.7)
- Not at all: 2 (11.1)

**Easy to understand?**
- Very easy: 5 (27.7)
- Easy: 8 (44.4)
- Somewhat easy: 3 (16.7)
- Not easy at all: 2 (11.1)

**Do responses fit?**
- Very well: 3 (27.7)
- Reasonably well: 9 (44.4)
- A little bit: 4 (16.7)
- Not at all: 3 (11.1)

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**Q2: SPAS1**  
**Domain: Spasticity**  
**Avg. time: 17 s**

**How many times have you experienced severe joint spasticity in the last month?**

![Histogram showing frequency distribution of spasticity severity](https://via.placeholder.com/150)

**Min:** 0  
**Max:** 28  
**Mean:** 11.5  
**Median:** 11  
**Mode:** 11  
**SD:** 2.76

## Missing Responses

<table>
<thead>
<tr>
<th>Code</th>
<th>Freq.</th>
<th>%</th>
<th>Avg. time</th>
</tr>
</thead>
<tbody>
<tr>
<td>-97</td>
<td>10</td>
<td>2.31%</td>
<td>5 s</td>
</tr>
<tr>
<td>-98</td>
<td>5</td>
<td>1.65%</td>
<td>12 s</td>
</tr>
<tr>
<td>-99</td>
<td>0</td>
<td>0.00%</td>
<td>---</td>
</tr>
<tr>
<td>-100</td>
<td>0</td>
<td>0.00%</td>
<td>---</td>
</tr>
<tr>
<td>-999</td>
<td>0</td>
<td>0.00%</td>
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Arnstein (1969) Ladder of Citizen Participation

8. Citizen Control
7. Delegated Power
6. Partnership
5. Placation
4. Consultation
3. Informing
2. Therapy
1. Manipulation

Tokenism

Citizen Power

BMJ

“Gimme My Damn Data”
Medicine 2.0
September 18, 2009

NICE National Institute for Health and Care Excellence

Patients Included

pcori
Let the patient revolution begin!

Estimates of effect size for selected ALS treatments. Each line represents the probability distribution of the effect size; a high, narrow peak indicates that the effect size is more precisely estimated (generally because of larger sample size). More effective treatments will be centered toward the left.

Observations

Clinical Trials

Subjects no more: what happens when trial participants realize they hold the power?

Open Access

Patients will hold us all accountable in new and necessary ways

Paul Wicks, vice president of innovation, Timothy Vaughan, director of data science, James Heywood, co-founder and chairman

PatientsLikeMe, Cambridge, MA 02141, USA
From experience to quantification to co-production: reflections on patient reported outcomes in an online patient community

Paul Wicks, PhD
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