Contents

- Background
- Capabilities
- Research
- Business model
- Helping your patients
- Discussion
Stephen Heywood – Builder / carpenter, dx’d with ALS @ 29 yrs. Survived 8 years.

Jamie Heywood – Mechanical engineer, founded ALS Therapy Development Foundation (ALSTDF, now ALSTDI) to find a cure for Stephen

Ben Heywood – Film producer in Hollywood

Jeff Cole – Friend from college, also MIT
Taking the fight against ALS public

- Stephen became major face in US ALS coverage
- Relationship between Stephen and Jamie documented in book by Pulitzer-prize winning book
- 5-year documentary film about Stephen and ALSTDF; “So Much So Fast”
- Died in October 2006
First-generation web-based networking

- Anonymous (non validated)
- Often inaccurate or unattributed medical information
- Provides support and empathy for small groups
- Difficult to connect to those with the information to help
First-generation social networks

- Some validation through association with illness
- “Light” shared information on current treatments
- Improved support and richer social connections
- No timeline or illness history
“Given my status, what is the best outcome I can expect to achieve and how do I get there?”

Making an emphasis on sharing information could help patients make their decisions.

The type of relationships identified by online dating services could be transformed to help someone find a “patient like me” to offer their knowledge, experience, and support.
Our core values

<table>
<thead>
<tr>
<th>Honor Patients’ Trust</th>
<th>Openness</th>
</tr>
</thead>
<tbody>
<tr>
<td>Our patients trust us with their most valued health information. We honor that trust, and we are dedicated to advancing the knowledge in the disease with the information they share.</td>
<td>Per our Openness Philosophy, we believe that sharing health information is good. Why? Because sharing will drive massive change in healthcare.</td>
</tr>
</tbody>
</table>

**PATIENTS FIRST**

<table>
<thead>
<tr>
<th>Transparency</th>
<th>Create WOW!</th>
</tr>
</thead>
<tbody>
<tr>
<td>No surprises. Our members shouldn't be surprised by anything we do. Our goal is to disclose what we do with members' information, how we make money, as well as all of our partnerships on the site.</td>
<td>When people see our site, we want them to think, “Wow!” Achieving our vision takes flawless execution and a deep understanding of patient needs.</td>
</tr>
</tbody>
</table>
How does PatientsLikeMe work?

SHARE
Patients’ sharing detailed health data is what makes our communities special. Patients learn from each other through sharing of traditionally private data.

FIND
Patients’ find other patients like them to learn what options are available for treatment. Patients then share information with their health care team.

LEARN
Patients learn about treatment options and what to expect from each in our Treatment and Symptom databases.
Finding a “patient like me” is important!
<table>
<thead>
<tr>
<th>Condition</th>
<th>First Patient</th>
<th>Registered Patients</th>
<th>Est. US Prev. (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Amyotrophic Lateral Sclerosis</td>
<td>Nov 2005</td>
<td>3,480</td>
<td>30,000 (12%)</td>
</tr>
<tr>
<td>Multiple Sclerosis</td>
<td>Mar 2007</td>
<td>11,900</td>
<td>500,000 (2%)</td>
</tr>
<tr>
<td>Parkinson’s Disease</td>
<td>Mar 2007</td>
<td>3,500</td>
<td>500,000 (0.7%)</td>
</tr>
<tr>
<td>HIV/AIDS</td>
<td>Sep 2007</td>
<td>2,200</td>
<td>1,000,000 (0.2%)</td>
</tr>
<tr>
<td>Mood Disorders</td>
<td>Feb 2008</td>
<td>9,700</td>
<td>20,000,000 (0.05%)</td>
</tr>
<tr>
<td>Progressive Supranuclear Palsy</td>
<td>Sep 2008</td>
<td>53</td>
<td>10,000 (0.5%)</td>
</tr>
<tr>
<td>Multiple System Atrophy</td>
<td>Sep 2008</td>
<td>188</td>
<td>25,000 (0.8%)</td>
</tr>
<tr>
<td>Devics NMO</td>
<td>Sep 2008</td>
<td>112</td>
<td>15,000 (0.7%)</td>
</tr>
<tr>
<td>Fibromyalgia</td>
<td>Nov 2008</td>
<td>3,200</td>
<td>7,000,000 (0.05%)</td>
</tr>
</tbody>
</table>
Contents

- Background
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“Given my status, what is the best outcome I can expect to achieve, and how do I get there?”
“Given my status, what is the best outcome I can expect to achieve, and how do I get there?”
“Given my status…”

- Demographics
- Disease history
- Genetics
- Primary disease measure
  - Pathology
  - Function
- Secondary disease measures
  - Symptoms
  - Wellbeing / HR-QOL
“Given my status, what is the best outcome I can expect to achieve, and how do I get there?”
“...what is the best outcome I can expect to achieve...”

- Patient Search (outliers)
- Contextual data (%iles)
“...what is the best outcome I can expect to achieve...”

**ALS Condition**

**Female, 45 years**
Mount Vernon, WA

**Diagnosis Summary**
Onset: Legs
First symptom: 01/08
Diagnosis: 09/08

**FRS: 39**
(latest: 02/03/09)
Progression rate percentile
- 5-10th (rapid)
- 10-25th
- 25-75th (average)
- 75-90th
- 90-95th (slow)

**Onset**
Feb 08
May 08
Aug 08
Nov 08
Feb 09

**Female, 43 years**
Malmö, Sweden

**Diagnosis Summary**
Onset: Legs
First symptom: 01/92
Diagnosis: 08/98

**FRS: 30**
(latest: 09/06/08)
Progression rate percentile
- 5-10th (rapid)
- 10-25th
- 25-75th (average)
- 75-90th
- 90-95th (slow)

**Onset**
Jan 93
Jan 95
Jan 97
Jan 99
Jan 01
Jan 03
Jan 05
Jan 07
Jan 09

**A4V mutation**

**D90A mutation**
“...what is the best outcome I can expect to achieve...”
“...what is the best outcome I can expect to achieve...”

Anxiety vs MS severity
“Given my status, what is the best outcome I can expect to achieve, and how do I get there?”
...and how do I get there?

- Treatment reports
- Forum posts
- Analytics

Treatments patients are currently taking for slow my MS progress:

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Count</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Glatiramer acetate</td>
<td>1218</td>
<td>100%</td>
</tr>
<tr>
<td>Interferon beta-1a SubQ injection</td>
<td>779</td>
<td>64%</td>
</tr>
<tr>
<td>Interferon beta 1a IM injection</td>
<td>664</td>
<td>55%</td>
</tr>
<tr>
<td>Natalizumab</td>
<td>386</td>
<td>32%</td>
</tr>
<tr>
<td>Interferon beta-1b SubQ Injection</td>
<td>368</td>
<td>30%</td>
</tr>
</tbody>
</table>

**Efficacy** (Effect based on reason taken):

- Major: 17% (36)
- Moderate: 14% (30)
- Slight: 9% (20)
- None: 8% (17)
- Can't tell: 52% (112)

**Side-effects** (as an overall problem):

- Severe: 8% (19)
- Moderate: 13% (30)
- Mild: 50% (111)
- None: 29% (67)

Summary results are based on 234 individual responses. For more information, see overall results for Adherence, Burden and Cost.

Individual Patient Reported Evaluations (2 of 234):

**Efficacy**
- Can't tell

**Side-effects**
- Mild

**Advice/Tips**
Doing the injections manually (rather than using the autoject) results in less site reactions. Read the included information, and make sure you are aware of the symptoms of the "reaction" that some experience immediately after injecting. It can be really scary when it happens if you don't know what it is. It happened to me once, after being on Copaxone for over 1.5 years.

See full evaluation 6 helpful marks
Treatment Reports

**Baclofen**

**Brand Names:** Lioresal

*See also:* Baclofen TTP (Intrathecal Pump)

**What is Baclofen?** Baclofen is a muscle relaxant and antispastic agent used in the treatment of muscle spasms, cramping, and tightness. It is available in tablet, syrup form, or can be transfused by surgical insertion of a pump in the spinal cord. [Read more...](#)

See more information, including instructions, precautions, side effects, and interactions.

**Reasons Taken**

297 patients in the community are taking or have taken Baclofen:

<table>
<thead>
<tr>
<th>Reason</th>
<th>Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Stiffness/spasticity</td>
<td>195</td>
</tr>
<tr>
<td>Other</td>
<td>33</td>
</tr>
<tr>
<td>Fasciculations</td>
<td>20</td>
</tr>
<tr>
<td>Disease-related symptoms</td>
<td>15</td>
</tr>
<tr>
<td>Cramps</td>
<td>7</td>
</tr>
<tr>
<td>Pain</td>
<td>5</td>
</tr>
<tr>
<td>Don't know</td>
<td>2</td>
</tr>
<tr>
<td>Slow my ALS progress</td>
<td>2</td>
</tr>
<tr>
<td>Mobility</td>
<td>3</td>
</tr>
<tr>
<td>General health</td>
<td>3</td>
</tr>
<tr>
<td>Anxiety</td>
<td>1</td>
</tr>
<tr>
<td>Fatigue</td>
<td>1</td>
</tr>
<tr>
<td>Spasticity</td>
<td>1</td>
</tr>
<tr>
<td>Laryngospasm</td>
<td>1</td>
</tr>
</tbody>
</table>

**Dosages**

Based on the patients currently taking Baclofen.

Top 10 (Show all)

<table>
<thead>
<tr>
<th>Dosage</th>
<th>Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>25 mg</td>
<td>22</td>
</tr>
<tr>
<td>10 mg</td>
<td>6</td>
</tr>
<tr>
<td>20 mg</td>
<td>12</td>
</tr>
<tr>
<td>30 mg</td>
<td>36</td>
</tr>
<tr>
<td>40 mg</td>
<td>31</td>
</tr>
<tr>
<td>50 mg</td>
<td>29</td>
</tr>
<tr>
<td>60 mg</td>
<td>16</td>
</tr>
<tr>
<td>70 mg</td>
<td>7</td>
</tr>
<tr>
<td>100 mg</td>
<td>2</td>
</tr>
<tr>
<td>120 mg</td>
<td>0</td>
</tr>
</tbody>
</table>

**Patients**

- **Taking Baclofen**
  - bukaro: 40 mg daily since Jan 05, 2006
  - beltaneman: 10 mg daily since Aug 10, 2007
  - donny/fis: 60 mg daily since Sep 05, 2003

- **Forum**

  **What are people saying about Baclofen?**

  **Most Recent**

  *Is baclofen right for me?*
  
  We recently went to seem a movement/joint specialist regarding spasticity. At present Dad takes Baclofen to ease stiffness at night to relax him while he sleeps. The problem with using these drugs during the day is that they may ...
  
  *Oct 26, 2008*

  *Is baclofen right for me?*
  
  ...Common oral medications for spasticity Baclofen (Lioresal) 1 Diazepam (Valium) Dantrolene (Dantrium) Tizanidine (Zanaflex) ... is warranted. We usually start with Lioresal (Baclofen) and caution the
Adverse Event Reporting

Report an Adverse Event

If you experienced a serious or unexpected problem associated with Avonex, it may be of interest to the U.S. Food and Drug Administration (FDA). To improve patient safety, the FDA monitors serious or unexpected problems — which they call “adverse events” — with products they regulate. We’d like to ask you a few questions to help determine if your experience should be reported to the FDA.

Question 1

In what context were you taking Avonex?

- As prescribed by my health care provider, or as part of my regular treatment regimen
- As a vaccine
- As part of a clinical trial or research study

[Continue]  [Cancel, go to my Treatment History]

How are events reported?
Unexpected and severe side effects are reported through MedWatch, the FDA Safety Information and Adverse Event Reporting Program. Vaccines and investigational treatments have a different reporting process.

About MedWatch
MedWatch is the FDA Safety Information and Adverse Event Reporting Program. It allows healthcare professionals and patients to voluntarily report serious problems that they suspect are associated with FDA-regulated treatments and medical devices.

Pilot launched Mid-February in MS

13 events submitted to FDA (20% of reports started)

Starting to code with MEDRA
Mapping: Find a Patient Near You
Contents

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- Discussion
Our Dedicated R&D team commands scientific expertise in many areas

**World Class Institutions**
- Harvard Medical School
- Yale
- MIT & MIT Media Lab
- King’s College London
- Sloan – Kettering Cancer Center
- Rutgers

**Recognized Expertise**
- Animal models of drug discovery
- Psychological consequences of movement disorders
- Interactive web design
- Genetic discovery
- Predictive modeling

**Diverse & Complementary**
- Drug discovery
- Psychology
- Sociology
- Genetics
- Nursing

**Widely Published**
- 60+ publications in peer-reviewed journals including: New England Journal of Medicine, Neurology, Proceedings of the National Academy of Sciences, Archives of Internal Medicine, Medical Care, Medical Decision Making, AIDS...

**Thought leaders**
- TEDMED
- Director’s Lecture, NIH
- BOT @ AMA
- Milken Global Conference
- MGH Grand Rounds

**Integrated Responsibilities**
- Community design
- Survey design & research
- PRO development
- Academic collaborations
- Health data integrity
Published Research Findings: Measuring function in advanced ALS: Validation of ALS FRS-EX extension items

- 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 standard ALSFRS measure
- Published, in use in ALS research around the world

Measuring function in advanced ALS: validation of ALSFRS-EX extension items

P. Wicks\textsuperscript{a}, M. P. Massaglia\textsuperscript{a}, C. Wolf\textsuperscript{b} and J. Heywood\textsuperscript{a}

\textsuperscript{a}PatientsLikeMe Inc., Research & Development, Cambridge, MA, USA; and \textsuperscript{b}Person living with ALS, patient member of PatientsLikeMe.com

Keywords: ALS, ALSFRS-R, clinical rating scale, floor effect

Background: With the aid of assistive technology, some patients with amyotrophic lateral sclerosis (ALS) are able to live for several years past the lowest measurable level of function on the Amyotrophic Lateral Sclerosis Functional Rating Scale – Revised (ALSFRS-R), a widely used end-point in ALS assessment. There is a research need to monitor patient function at the end of life, particularly in the face of severe impairment or ‘locked in syndrome’. Methods: We used an online community for people with ALS (PALS) (PatientsLikeMe) to construct and pilot a number of new items to add to the
Published Research Findings: ALS patients request more information about cognitive symptoms

90% of the time
ALS patients are told about possible physical symptoms

10% of the time
ALS patients are told about possible cognitive symptoms

Forum post: “How many of you have noticed a change in your personality? How many of you have become more difficult, stubborn?”

Survey on disease knowledge sent to 250 ALS patients, 90 caregivers

Despite widely known cognitive dysfunction in ALS, patients were not being warned by their doctors

Published in European Journal of Neurology
13% of Parkinson’s patients report pathological gambling compulsions

3% of ALS patients report pathological gambling compulsions

Introduction:
Pathological gambling (PG) was first described in Parkinson’s disease in 2000 and has been estimated to affect between 3-7% of patients. Current opinion suggests that these behaviours may be triggered by dopaminergic medication (usually dopamine agonist drugs) although younger age, increased impulsivity and higher novelty seeking traits appear pertinent risk factors in this population. ALS is a neurodegenerative disorder traditionally held to affect only motor functions; however it is now clear that a small proportion of ALS (~5-10%) patients experience fluid personality changes consistent with fronto-temporal dementia whilst a larger proportion (33-50%) experience milder cognitive change of a predominantly executive nature.

Whilst there have been no reports in the literature of pathological gambling amongst ALS patients, it has been noted anecdotally in clinical experience. Patients with ALS-FTD have been described as engaging in repetitive and compulsive behaviours including compulsive gambling and other perseverative behaviours, and FTD in isolation has been reported as comorbid with PD. Previous reports have

Forum post: “I am spending a lot of money that I should not spend. I wake up thinking about the lottery… It all started after starting to take Requip and Stalevo. Help me before I spend all of our little savings.”

400+ patients from ALS & PD recruited for study of pathological gambling

Publication accepted in Movement Disorders
Peer-reviewed Publications

- Pathological Gambling amongst Parkinson's Disease and ALS patients in an online community, *Movement Disorders*. 2009; (In press)


- PatientsLikeMe: Consumer Health Vocabulary as a Folksonomy *AMIA Proceedings*;2008;682-6


- Social uses of personal health information within PatientsLikeMe, an online patient community: what can happen when patients have access to one another’s data, *J Med Internet Res*. 2008;10(3):e15

Lithium delays progression of amyotrophic lateral sclerosis

Francesco Formai*, Patrizia Longone†, Luisa Cafaro‡, Olga Kastziuchenka*, Michela Ferrucci*, Maria Laura Manca§, Gloria Lazzeri*, Alida Spalloni†, Natasia Bellio†, Paola Lenzi*, Nicola Modugno*, Gabriele Siciliano*, Ciro Isidori†, Luigi Murri§, Stefano Ruggieri‡, and Antonio Paparelli*

*Department of Human Morphology and Anatomy, Istituto Neurologico Mediterraneo, Istituto Santa Lucia Foundation, 00179 Rome, Italy
†Istituto di Fisiologia e Neurobiologia, Istituto di Patologia Neurologica, Istituto Santa Lucia Foundation, 00179 Rome, Italy
‡Istituto di Fisiologia e Neurobiologia, Istituto di Patologia Neurologica, Istituto Santa Lucia Foundation, 00179 Rome, Italy
§Istituto di Fisiologia e Neurobiologia, Istituto di Patologia Neurologica, Istituto Santa Lucia Foundation, 00179 Rome, Italy

ALSFRS-R (raw data)
Timeline from first discussion

- **1st Post - link to Italian article w/ Google translation**
- **Lithium tool goes live**
- **Patient requests additional functionality**
- **PNAS Article Published**

Timeline from first discussion

- November 2007: First post
- December 2007: Link to Italian article with Google translation
- January 2008: Lithium tool goes live
- February 2008: Patient requests additional functionality
- March 2008: PNAS Article Published
- April 2008: Timeline from first discussion

Timeline from first discussion

- November 2007
- December 2007
- January 2008
- February 2008
- March 2008
- April 2008
- May 2008
- June 2008
- July 2008
- August 2008
- September 2008
- October 2008

Timeline from first discussion

- Posts
- Patients
A patient-lead trial of lithium

<table>
<thead>
<tr>
<th>Patient</th>
<th>Profile Star</th>
<th>Onset Date</th>
<th>Diagnosis Date</th>
<th>Type</th>
<th>Sex</th>
<th>Age</th>
<th># of FRS scores</th>
<th>Onset to Start</th>
<th>Start to Today</th>
<th># of data points in months before and after Lithium start</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hanns</td>
<td>✅</td>
<td>✅</td>
<td>✅</td>
<td></td>
<td></td>
<td></td>
<td>8</td>
<td>11</td>
<td>0</td>
<td>0 2 2 1 1 1 1 2</td>
</tr>
<tr>
<td>humberto-from-brazil</td>
<td>✅</td>
<td>✅</td>
<td>✅</td>
<td></td>
<td></td>
<td></td>
<td>4</td>
<td>11</td>
<td>0</td>
<td>1 1 1 1 1 1 1 1</td>
</tr>
</tbody>
</table>

**Side Effects**
- Muscle weakness, stiffness, twitching, or tightness
- Constipation
- Slurred speech
- Excessive saliva
- Weight gain or loss

**Blood Level**
- Lithium: 0.2 mmol/L

**Lithicarb**
- 150 mg/day

**FRS**
- 17

**FVC**
- 40

**On Rilutek**

**humberto-from-brazil**
- Male, 42 years
- Onset: Arms

**Started Lithium**

- Jan 07
- Jan 08
Our findings…

Months on Lithium

Euordis 2009

N=134 Lithium (start)
N=134 Matched Controls
Probability models for key disease events
Contents

- Background
- Capabilities
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- Business model
- Discussion
Business model – How we make money

- For-profit company allows faster growth, greater focus and sustainability
- Completely transparent to all users
- Access products
  - Clinical trial access
  - Market research
  - Forum keyword monitoring
- Data products
  - Clinical research
  - Longitudinal partnerships
  - PatientsLikeMe University
- Community development partners - pharma / insurers / med device manufacturers -
- Typically multi-million $ engagements
Community development planning

- Developing a new community
  - Define, Research, Innovate, Visualize, Execute (DRIVE)
  - ~3 months lead time to soft launch
  - Choosing/developing patient-reported outcome measures
  - Developing visualization
  - Engaging thought leaders & patients
  - Accomplishing client goals

- Patient organization provides design input, patient referrals; gets improved service for patients, data as needed

- Pharma provides funding; gets PR, direct line to patient voice, data for marketing / EBM groups, competitive advantage
John37's Profile

John37
Male, 28 years
Cambridge, MA

Diagnosis Summary
Familial: No
Diagnosis: 09/04
Genetics: COL1A1 GS86V
Bone Fractures: 25-50

About Me
Lorem ipsum dolor sit amet, consectetur adipiscing elit, sed do eiusmod tempor incididunt ut labore et dolore magna aliqua. Ut enim ad minim veniam, quis nostrud exercitation ullamco laboris nisi ut aliquip ex ea commodo consequat. Duis aute irure dolor in reprehenderit in voluptate velit esse cillum dolore eu fugiat nulla pariatur. Excepteur sint occaecat cupidatat non proident, sunt in culpa qui officia deserunt mollit anim id est laborum.

Bone Mineral Density
(DEXA test)
(latest: 9/01/09)

IO Condition

Breaks
(latest: 9/01/09)

Function
(latest: 9/01/09)

Spinal Curvature
Moderate

Teeth

Hearing: No Symptoms

Breathing: Mild

Bruising/Bleeding: Severe

My latest Instant Pain is Severe

Feel lousy today. Pain meds are doing nothing, and I can barely walk

OI: Type III Height: 3'4''

This member is at 3 stars data quality.
Lessons & pitfalls for Health 2.0

- “If you build it, they will come”?  
  - Not true. A lot of possible communication channels for patients; Facebook, Yahoo Groups, Ning, blogs, etc.

- Online ≠ Free.  
  - Forum moderation, advertisement, new content, insurance / legal, maintenance / security, upgrades, hosting

- Online champions are hugely influential across multiple channels  
  - Devic’s NMO community because of 1 patient  
  - BobbyB in ALS

- “Official” information is best?  
  - Dynamic, warts n’ all data > static, approved, unrepresentative data

- Online data collection is being taken more seriously  
  - Fewer barriers to publication than anticipated
The future we’d like to see…

www.patientslikeme.com

pwicks@patientslikeme.com