

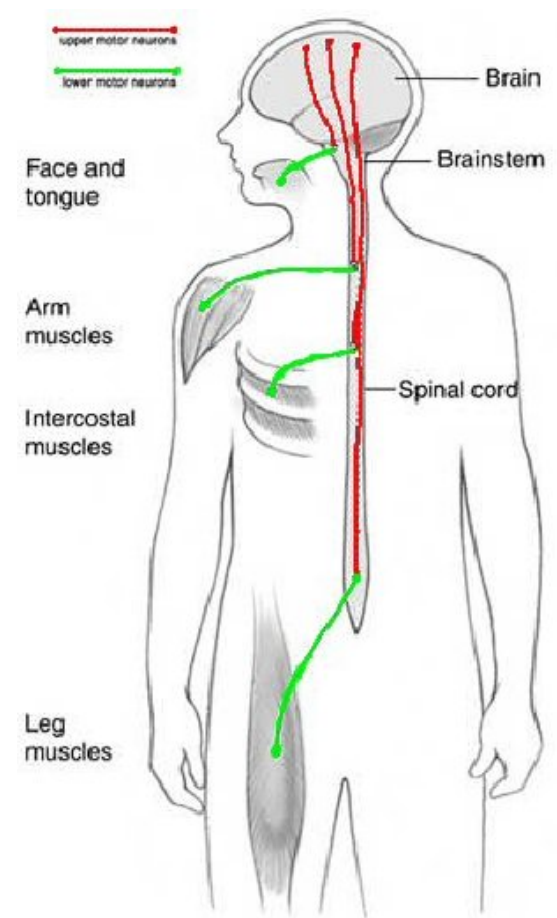
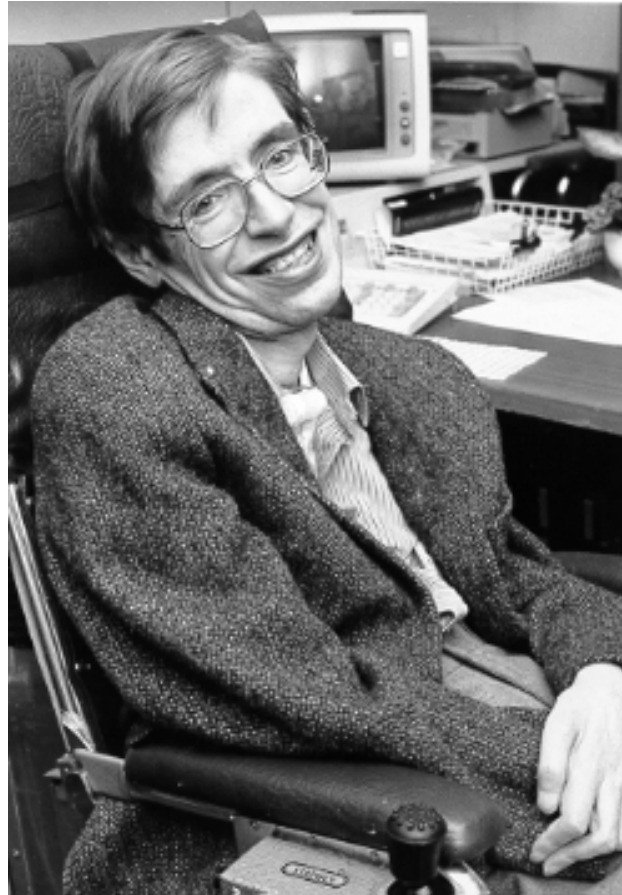
patientslikeme®

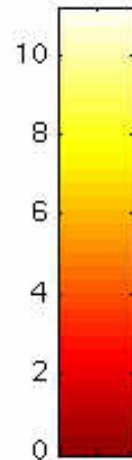
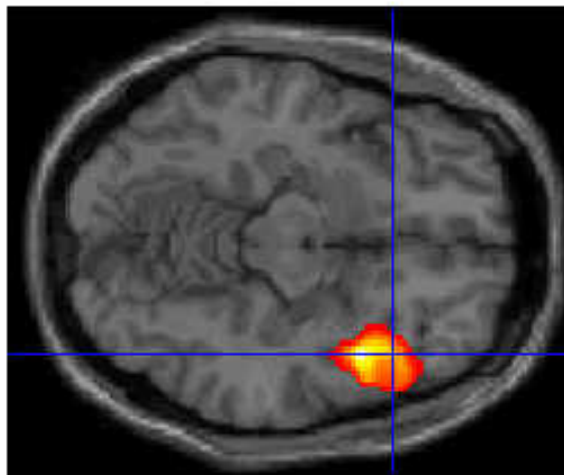
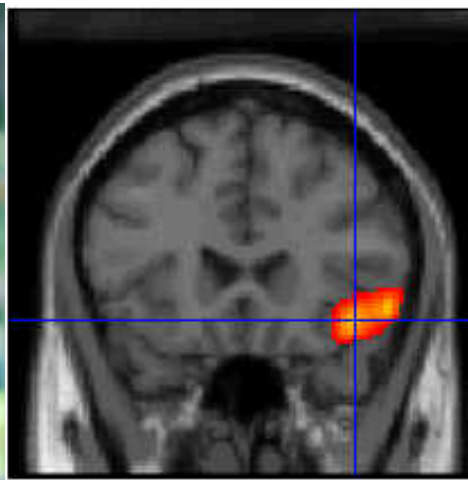


From experience to quantification to co-production: reflections on patient reported outcomes in an online patient community

Paul Wicks, PhD
VP of Innovation

Amyotrophic Lateral Sclerosis (ALS) / Motor Neurone Disease (MND)





CANT	Cont
CENT	Cent
CAN E	Came

Amyotrophic Lateral Sclerosis

2007, iFirst Article, 1-7

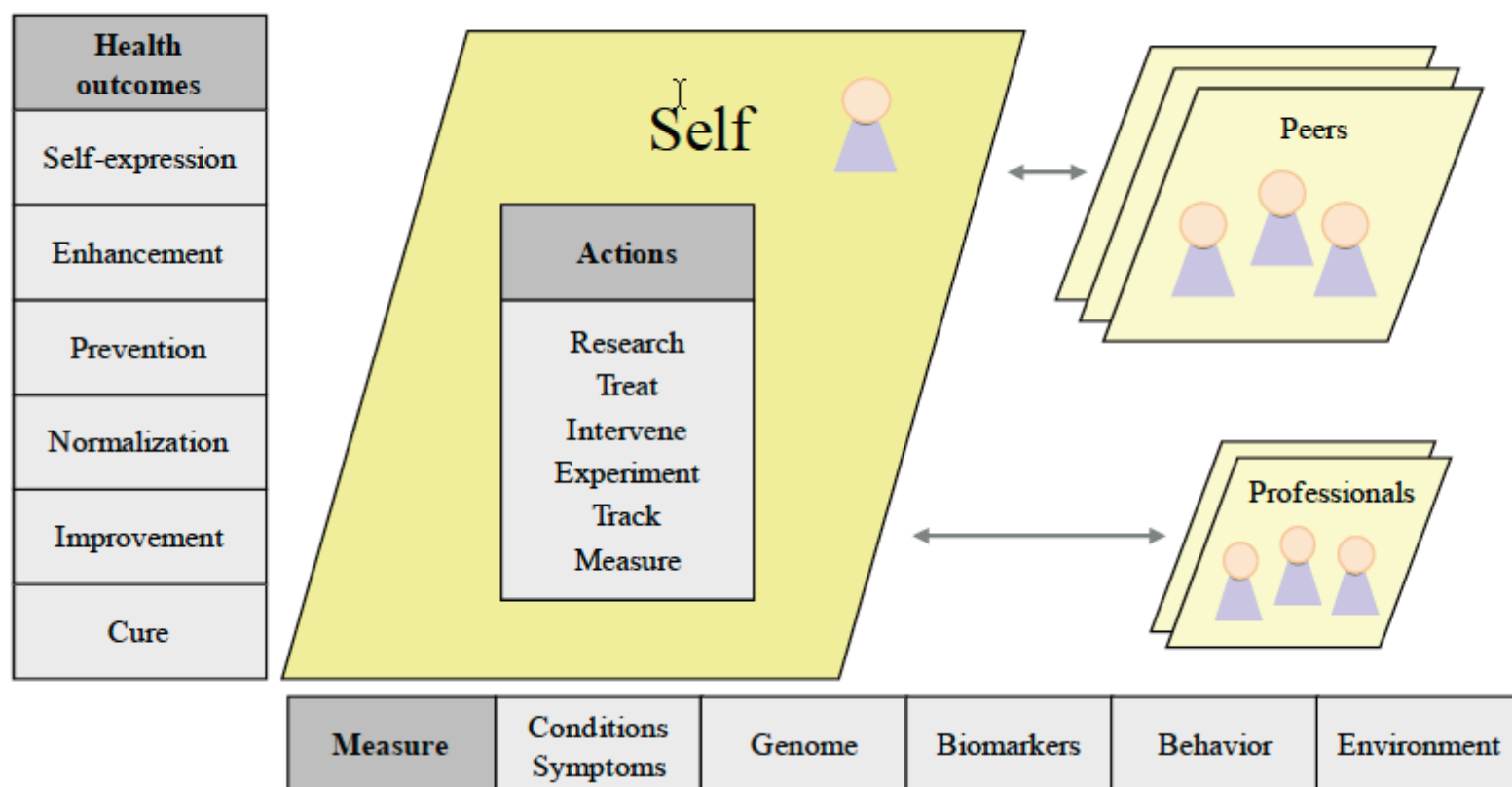
Neuronal loss associated with cognitive performance in amyotrophic lateral sclerosis: An (^{11}C)-flumazenil PET study

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



Figure 1. A new model of health and health care.

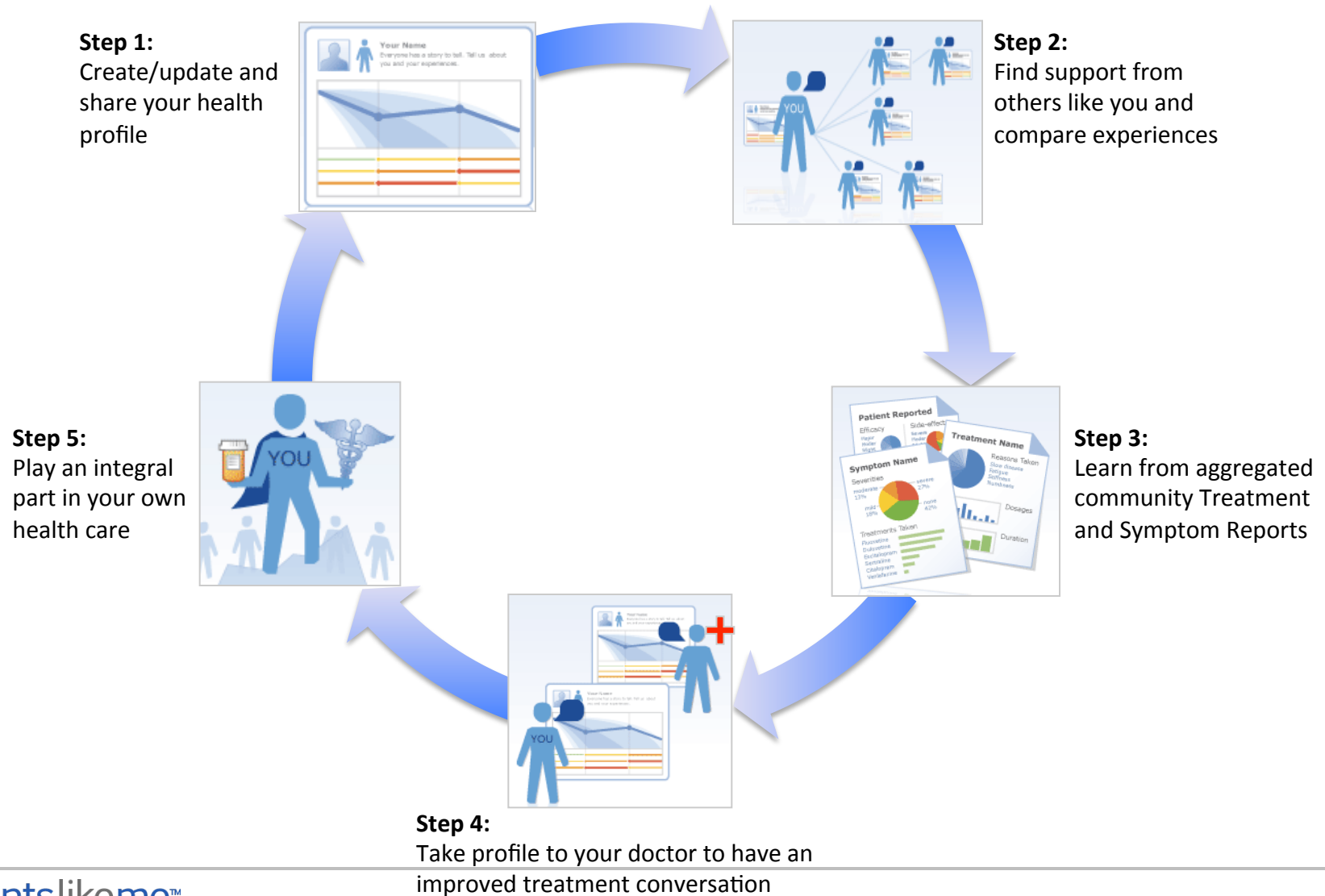


Int. J. Environ. Res. Public Health **2009**, *6*, 492-525

Emerging Patient-Driven Health Care Models: An Examination of Health Social Networks, Consumer Personalized Medicine and Quantified Self-Tracking

Melanie Swan *

PatientsLikeMe





Male, 50 years
London, United Kingdom

Interests: [Advocacy](#), [Parenting](#) and [1 more](#) ▼

Display charts from

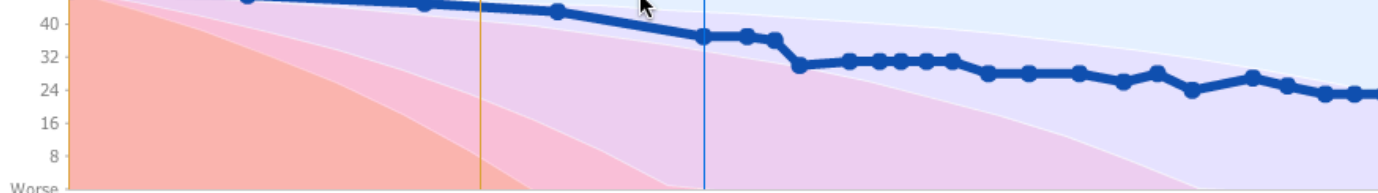
PLM 1 mo 6 mo

1 yr 2 yr **All**

Nov 20, 2012

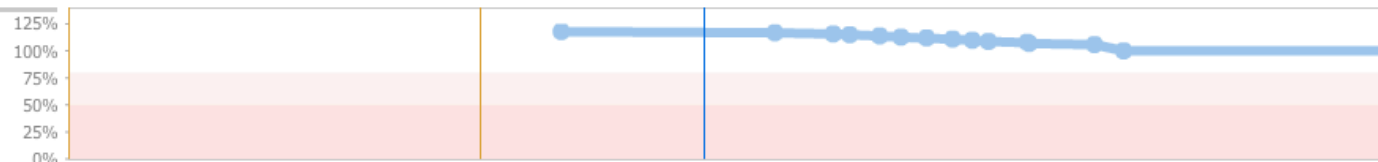
ALS First Sympt...	ALS Diagno...	Joined PLM
Dec 1, 2007	Jun 16, 2009	Apr 19, 2010

- >90th (slow)
- 75-90th
- 25-75th (average)
- 10-25th
- <10th (rapid)





Last update:
Nov 9, 2012

Warning
Severe



Last update:
Nov 16, 2012

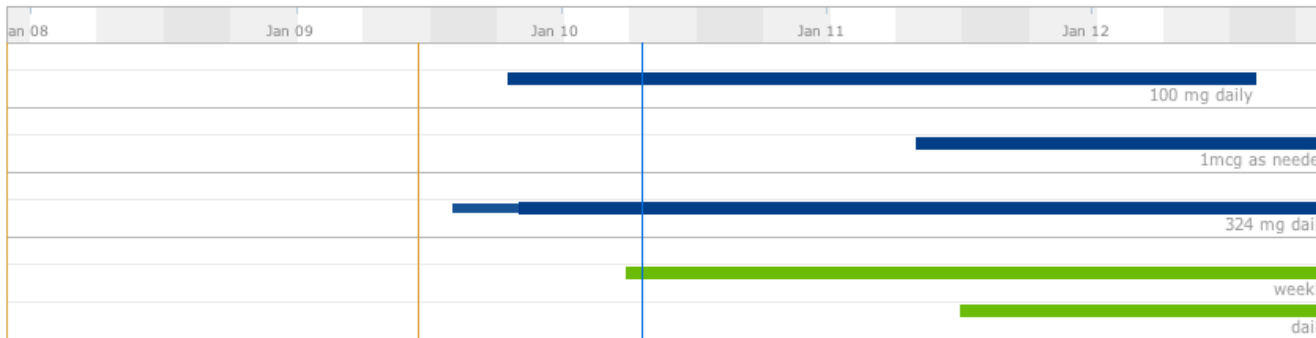
Type of treatment

- Prescription Drug
- Over the Counter Drug
- Supplement
- Lifestyle Modification
- Equipment
- Other
-  Side Effect
-  Evaluation

 Side Effect

e Evaluation

Roll over bars for more information



▼ ALS (Amyotrophic Lateral Sclerosis)

 Rilutek

▼ Anxious mood

 Lorazepam

▼ Cramps

e Quinine

▼ Fatigue

e Rolling Walker

e Wheelchair (manual)

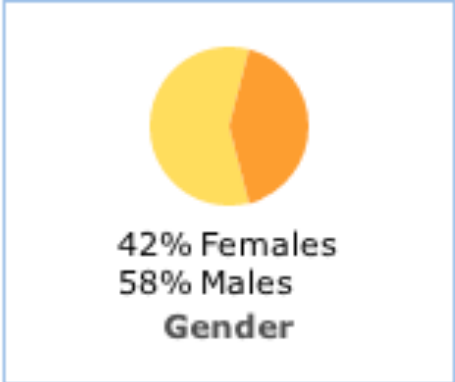
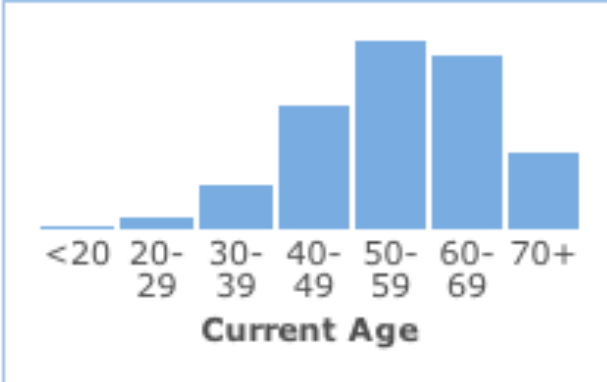


Who has ALS at PatientsLikeMe?

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?

Treatment name	Perceived effectiveness	Overall rating of side effects	# of Evaluations
Riluzole	<div><div></div></div>	<div><div></div></div>	284
Lithium Carbonate	<div><div></div></div>	<div><div></div></div>	86
CoQ10 (CoEnzyme Q10)	<div><div></div></div>	<div><div></div></div>	53
Baclofen	<div><div></div></div>	<div><div></div></div>	36
Vitamin E	<div><div></div></div>	<div><div></div></div>	35
ALS Phase 2 Study NP001	<div><div></div></div>	<div><div></div></div>	28
ALS Phase 3 Study Dexpramipexole (KNS-760704)	<div><div></div></div>	<div><div></div></div>	26
Sodium chlorite	<div><div></div></div>	<div><div></div></div>	26

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

Purpose	# of patients	# of patients with evaluations	Perceived Effectiveness				
			Major	Moderate	Slight	None	Can't tell
ALS (Amyotrophic Lateral Sclerosis)	1,476	299					
PMA (Progressive Muscular Atrophy)	29	13					
PLS (Primary Lateral Sclerosis)	25	7					
General health	14	5					
Stiffness/Spasticity	8	0					
Fasciculations	7	2					

See all 17 reasons taken ▼

See all 1,184 patients currently taking Riluzole

Mouse over the table for more information

Reported Side Effects

Side effects as an overall problem

Severe	25	
Moderate	49	
Mild	66	
None	191	





Commonly reported side effects, conditions, and hospitalizations associated with Riluzole

Fatigue	30
Nausea	18
Tiredness	9
Stomach pain	9
Elevated liver enzymes	8
Abdominal pain	8

See all 74 reported side effects ▼

Patient Evaluations

See all 331 patient evaluations



g

3

i

ALS

nacona
100 mg daily
since Jun 17,
2011

Perceived effectiveness  **Side Effects** 

for ALS
(Amyotrophic
Lateral
Sclerosis)

See full evaluation



1

i

ALS

thomcuddy
100 mg daily
since Aug 24,
2008

Perceived effectiveness  **Side Effects** 

for ALS
(Amyotrophic
Lateral
Sclerosis)

Advice/Tips

We can never know if Rilutek does any of us any good. If it doesn't seem to be doing any harm, I believe it is better to take it than not to. See full evaluation



i

ALS

ALS Boy
100 mg daily
since Oct 20,
2008

5 Phases of PRO development

Qualitative phase

Quantitative/psychometric phase

Concept Elicitation

Feedback

Test

Retest

Follow up

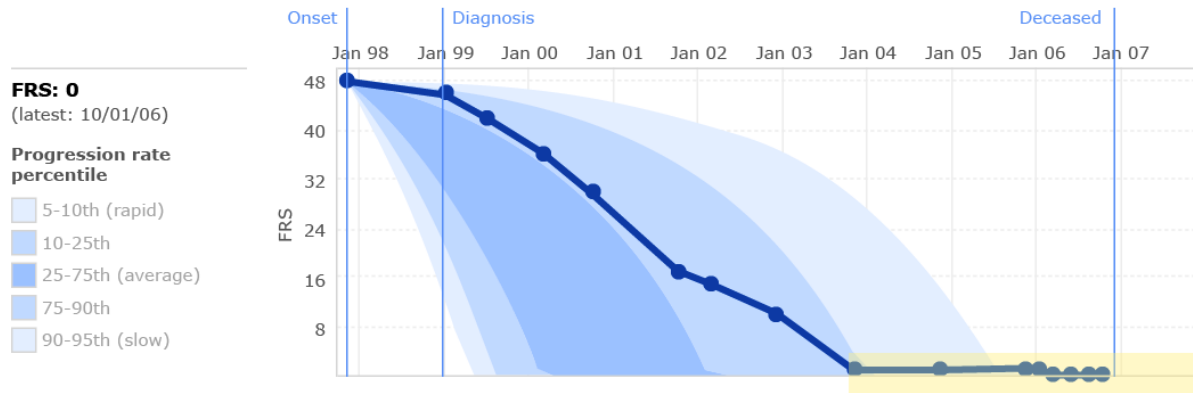
- To obtain input from patients
- Opportunity to examine patient experience at a large scale
- Item generation process
- **Based on open-ended questions**

- 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**

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

- To evaluate the ability to detect change

Measuring Advanced ALS



- 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 ¹¹

European Journal of Neurology 2009, 16: 353–359

doi:10.1111/j.1468-1331.2008.02434.x

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

P. Wicks^a, M. P. Massagli^a, C. Wolf^b and J. Heywood^a

^aPatientsLikeMe Inc., Research & Development, Cambridge, MA, USA; and ^bPerson living with ALS, patient member of Patients-LikeMe.com

Keywords:

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

Received 19 August 2008
Accepted 18 November 2008

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

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

Rosen R. Development of Patient Reported Outcome (PRO) Measures: How Feasible is a PRO for Asymptomatic PCa Treatment? FDA Public Workshop Clinical Trial Design Issues Drug and Device Development for Localized Prostate Cancer, 2013.

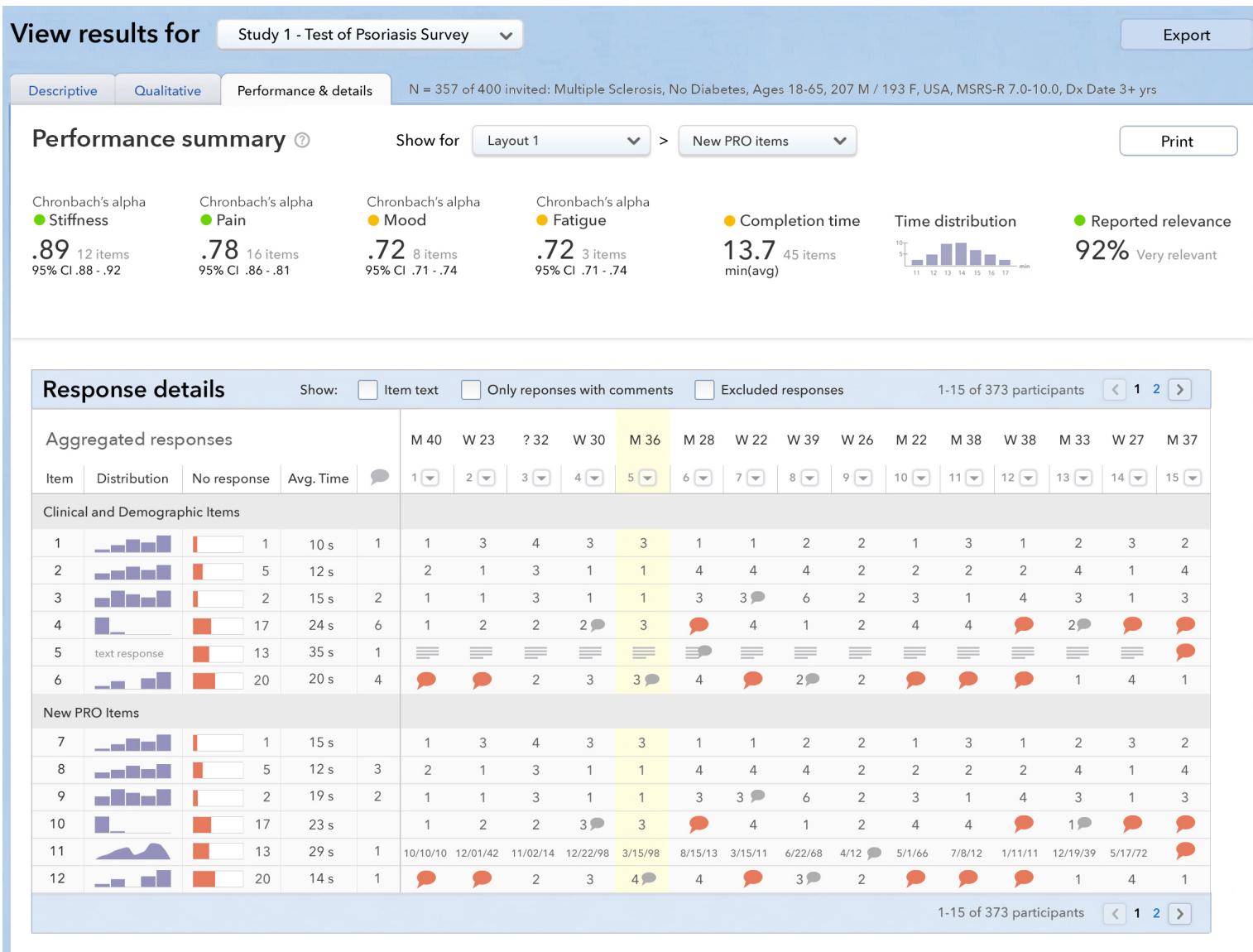
Hayes R. Patient-Reported Outcome (PRO) Instruments as Drug Development Tools (Session III). Consensus Science New Tools and Tactics for Next-Gen Drug Development., Washington, DC: 2011.

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

Export

Descriptive

Qualitative

Performance & details

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

Show for

Layout 1

New PRO items

All Domains

Print

Q1: STIFF1

Domain: Stiffness

Avg. time: 17 s

How long have you experienced joint stiffness?

Response option	Code	Freq.	%		Cum. freq.	Cum. %	Avg. time
Less than 1 week	0	228	15.49%	<div><div></div></div>	228	15.49%	12 s
Less than one month	1	339	23.02%	<div><div></div></div>	795	38.51%	19 s
Less than one year	2	397	26.97%	<div><div></div></div>	1192	65.48%	15 s
Less than 5 years	3	254	17.26%	<div><div></div></div>	1446	82.74%	15 s
More than 5 years	4	254	17.26%	<div><div></div></div>	1700	95.25%	16 s
I prefer to skip	-97	10	2.31%	<div><div></div></div>	1710	97.31%	5 s
I don't know	-98	5	1.65%	<div><div></div></div>	1715	100.00%	12s
Does not apply	-99	0	0.00%	<div><div></div></div>	0	100.00%	---
Forced skip	-100	0	0.00%	<div><div></div></div>	0	100.00%	---
Did not complete	-999	0	0.00%	<div><div></div></div>	0	100.00%	---

Quantitative Feedback Responses

Does it apply?	Freq.	%
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?	Freq.	%
Very easy	5	27.7
Easy	8	44.4
Somewhat easy	3	16.7
Not easy at all	2	11.1

Do responses fit?	Freq.	%
Very well	3	27.7
Reasonably well	9	44.4
A little bit	4	16.7
Not at all	3	11.1

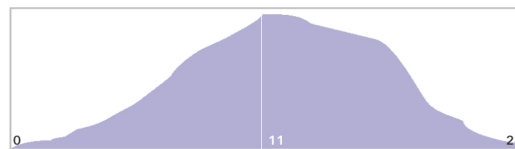
Q2: SPAS1

Domain: Spasticity

Avg. time: 17 s

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

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



Missing Responses	Code	Freq.	%	Avg. time
I prefer to skip	-97	10	2.31%	5 s
I don't know	-98	5	1.65%	12s
Does not apply	-99	0	0.00%	---
Forced skip	-100	0	0.00%	---
Did not complete	-999	0	0.00%	---

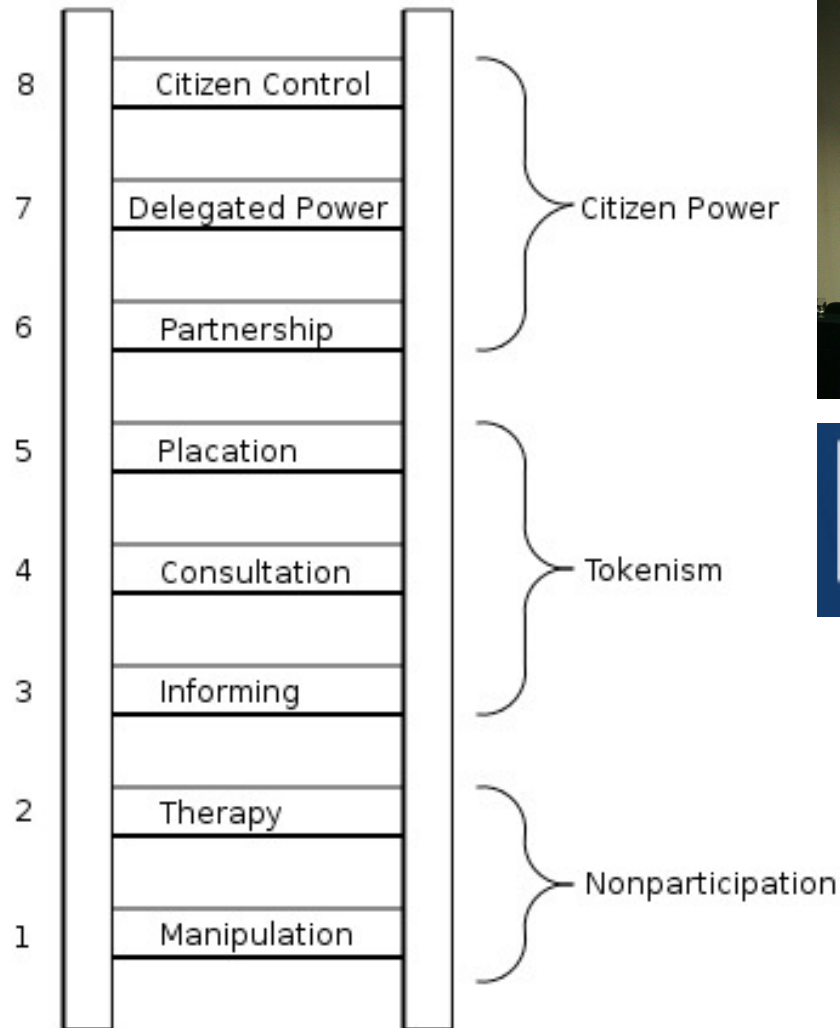
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Not easy at all	2	11.1

Do responses fit?	Freq.	%
Very well	3	27.7
Reasonably well	9	44.4
A little bit	4	16.7
Not at all	3	11.1

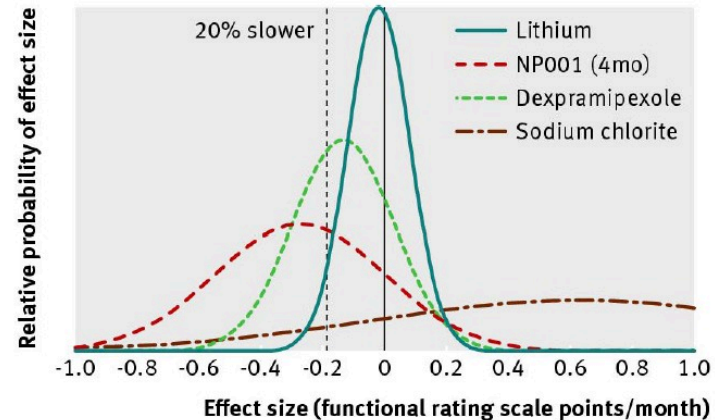
Arnstein (1969) Ladder of Citizen Participation



NICE National Institute for Health and Care Excellence



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 left¹⁴

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 *cofounder and chairman*

PatientsLikeMe, Cambridge, MA 02141, USA

patientslikeme®



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

Paul Wicks, PhD
VP of Innovation