Neuroscience Trials of the Future: A Workshop

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About PatientsLikeMe

- Founded in 2004 as a direct response to family's experience with chronic disease
- Built as an open, patient facing research based community
- Launched as ALS community in 2005 and opened to any condition in 2011
- Deep patient data and experience in 30-40 chronic life-changing conditions
- It is free to join and there's no advertising

Over a decade of patient-generated data…

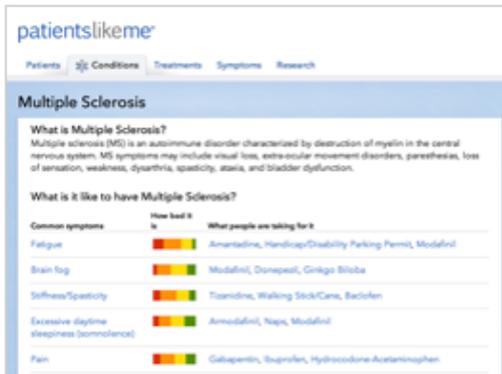| Patients	Data	Insights
• 390,000+ patients • 2,500+ conditions	• 30+ million structured data points • 3+ million free-text posts • 15+ PROMs	• 65+ publications, most peer reviewed • Patient-generated taxonomy • Safety monitoring platform • Open Research Exchange (ORE)

How Members Engage on PatientsLikeMe
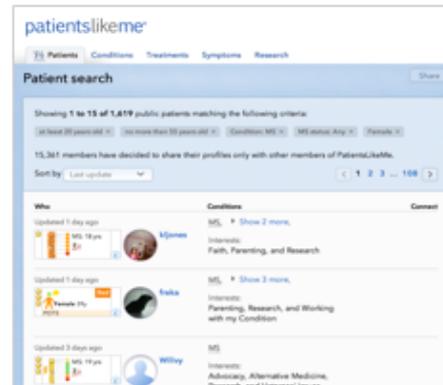
Learn
about living with & treating life-changing conditions

- Answer the questions, "Is this *normal?*" and "What are my *choices?*"
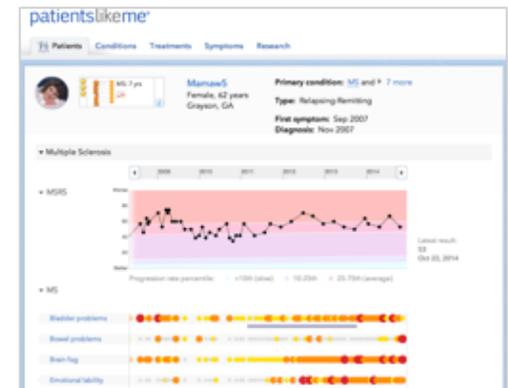- Learn from aggregated patient treatment and symptom data

Connect
with others sharing those experiences

- Find patients to connect with based on age, gender, disease, treatment, symptoms, interests
- Get day-to-day support from others to live better, together

Track
their history and progress over time

- Document symptom changes, treatments starts and stops, triggers of exacerbations, etc.
- Generate real-world outcomes data for use by researchers

Data Domains

| Basic Information (age, sex, etc.) |
| Diseases (early signs, diagnosis status, etc.) |
| General & Specific Symptoms (onset, severity status, etc.) |
| Treatments & Side Effects (Rx, OTC, Supp., non-drug, etc.) |
| Quality of Life & Behavior Status (all patients, some disease specific) |
| Outcome Measures of Disease (disease dependent) |
| Patient-generated narrative text, wearable and sensor data |

Engagement

Data Integrity

Standards *

Evidence

Knowledge

Empowerment

* Patient data mapped to ICD-10, ICF, SNOMED, MedDRA, RxNorm

Multiple Sclerosis Patient Profile

Conditions on PLM

Neurological and brain
- Multiple Sclerosis (48,187)
- Parkinson's Disease (11,940)
- Epilepsy (9,944)
- Migraine (8,365)
- ALS (Amyotrophic Lateral Sclerosis) (8,141)

Muscle, bone, and joint
- Fibromyalgia (62,220)
- Rheumatoid Arthritis (RA) (9,207)
- Systemic Lupus Erythematosus (18,124)
- Osteoarthritis (5,261)
- Degenerative Disc Disease (3,496)

Mental health
- Major Depressive Disorder (21,511)
- Generalized Anxiety Disorder (18,755)
- Post-traumatic stress disorder (14,735)
- Panic Disorder (10,112)
- Social Anxiety Disorder (6,022)

Metabolism and nutrition
- Diabetes Type 2 (18,156)
- Diabetes Type 1 (2,473)
- Obesity (2,099)
- High Cholesterol (Hypercholesterolemia) (1,921)
- Vitamin D Deficiency (1,681)

Gastrointestinal
- IBS (Irritable Bowel Syndrome) (4,872)
- GERD (Gastroesophageal reflux disease) (4,215)
- Crohn's Disease (4,023)
- Ulcerative colitis (1,234)
- Celiac Disease (828)

Respiratory
- Asthma (5,855)
- Idiopathic Pulmonary Fibrosis (5,457)
- COPD (Chronic Obstructive Pulmonary Disease) (2,349)
- Sleep Apnea Disorder (1,909)
- Cystic Fibrosis (1,237)

Oncology
- Lung Cancer (4,020)
- Multiple Myeloma (2,580)
- Breast Cancer (1,673)
- Prostate Cancer (827)
- Colon Cancer (428)

Cross-disease symptoms
- Anxious mood (115,512)
- Depressed Modd (116,211)
- Fatigue (117,668)
- Pain (114,463)
- Insomnia (104,507)

Patient-Centered Data Perspective

- Research in service of patients/platform
- Data collection vs. data donation
- "Your data makes a difference"
- Our data outlives us
- Each of us is an n=1 study
- The language of patients
- Research as a journey

Letitia

Data Privacy

"Before I write my name on the board, I'll need to know how you're planning to use that data."

Privacy Policy

- No guarantees on privacy
- Shared and restricted data
- Transparency about the use of data
- Members may choose to opt out
- Certain studies may require IRB/informed consent
- Data sharing agreements
- Qualitative data challenges
- Seek permission to use member profiles in published material

Patient Views on Privacy - Trait or State?

- Individual characteristics
  - Demographics
  - Motivations
  - Tolerance for ambiguity and risk
  - Disease factors (e.g., type, stage of progression, stigma)
  - Affinity for technology
- Conditional factors:
  - Type of data
  - Recipient of data
  - Who, when, and how of data sharing

PLM: participation in two funded NIH grants developing patient privacy measures

Social Media Use During Clinical Trials

Unblinding

…can you describe your pills in more detail? Like a more complete description of what they look like, how they react when they get wet, what their texture is, how long do they last in the mouth after being swished around, and a more complete description of the taste…

Nonadherence to condition

…If you do end up participating, you may want to keep in mind the possibility of dropping out of the trial early on if it appears you are not responding – and if it appears that you are unlikely to be on the [study drug]…

Medical Advice

I'm convinced I'm the resident expert on the subject at this point. So yeah, I'm gonna go out on a limb and assert you should stop the [study drug]! And no I'm not a doctor, but I'm STILL going to recommend it.

From: Glickman SW, Galhenage S, McNair L, Barber Z, Patel K, Schulman KA, McHutchison JG. The potential influence of Internet-based social networking on the conduct of clinical research studies. Journal of Empirical Research on Human Research Ethics. 2012 Feb;7(1):71-80.

We are human beings and we will talk; patients are not going to change, so the researchers must.
- trial participant

From: Lipset, CH. (2014). Engage with research participants about social media. Nature Medicine 20, 231.

RADCOMP

RADCOMP: comparative effectiveness study of proton and photon radiation treatment for breast cancer (PCORI)

- Multi-center trial
- Primary outcomes
  - Effectiveness in reducing major cardiovascular events
- Secondary Outcomes
  - Disease control
  - Quality of life
  - Radiation dose and cardiac toxicity
  - Long-term survival

PatientsLikeMe is supporting patient engagement and retention by providing a trial specific organization page portal for the trial (RadComp.org)

- Information about the trial and trial locations
- Links to trial sites, trial logins for patients and investigators (with partner RTOG [Radiation Therapy Oncology Group])
- Access to all PatientsLikeMe functionality for tracking and connecting to other breast cancer patients