Crowdsourcing patient generated data in a technology enabled community

Environmental Measurement Symposium
August 10, 2016

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VP Advocacy, Policy & Patient Safety
@SallyOkun

patientslikeme®

Patient stories – perspective matters

About PatientsLikeMe

- Overview of patient experience
- Patient informed journey framework and principles
- Data informatics and conventions
- Patient vocabulary

Emerging science of patient generated data

- Sampling of research publications
- FDA Research Collaboration Agreement
- Patient-informed clinical trials

What's next?

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> ...patients should help decide which research is conducted, help to plan the research and interpret the data, and hear the results before anybody else.

~Heather Goodare, Richard Smith~

The rights of patients in research. *BMJ* 1995;310:1277

The missing voice…it seems so obvious now

"The missing ingredient in the development of new therapies is the voice of the patient."

The missing patient voice in value-based care

The Missing Voice of Patients in Drug-Safety Reporting

How Listening to the Voices of Patients Can Improve Quality of Life

The patient voice in research—evolution of a role

Engaging the Patient Voice in the Delivery of Accountable Care in Medicaid

Increasing the Fidelity of Patient Voices and Their Impact on Health System Performance

Hearing the Patient's Voice? Factors Affecting the Use of Patient Survey Data in Quality Improvement

Perspective matters…

behind every bit of data is a patient's story

About PatientsLikeMe

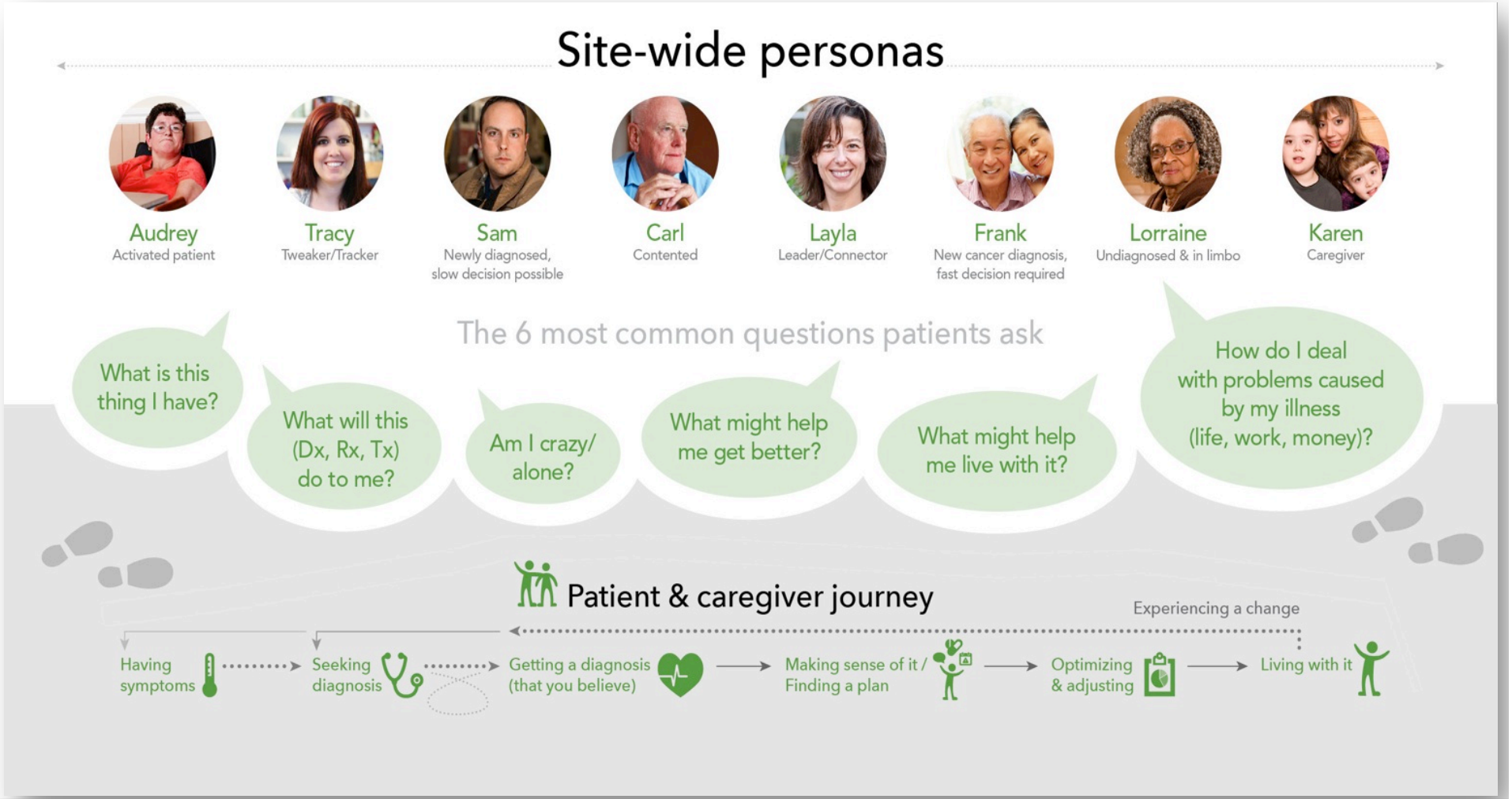
Our mission is to improve the lives of patients through new knowledge derived from shared real-world experiences and outcomes

- Founded in 2004 as direct response to Stephen Heywood's diagnosis of ALS
- Online, open, patient-facing community for people living with illness
- Deep patient data and experience in ~40 life-changing conditions
- Free for patients with no advertising
- Research-based infrastructure designed to create value for patient data
- Open-access research publications

Patients	Data	Insights
• 400,000+ patients • 2,500+ conditions	• 30+ million structured data points • 3+ million free-text posts • 15+ PROMs	• 70+ publications peer reviewed • Patient-generated taxonomy • Safety monitoring platform • Research collaboration with FDA

Diverse personas, similar experiences…

Ethnography approach to patient & caregiver journey reveals common events, feelings and questions



Patient voice informatics and data conventions:

Emerging science of patient reported and generated data

- **Basic Information** (age, sex, etc.)
- **Diseases, Conditions** (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

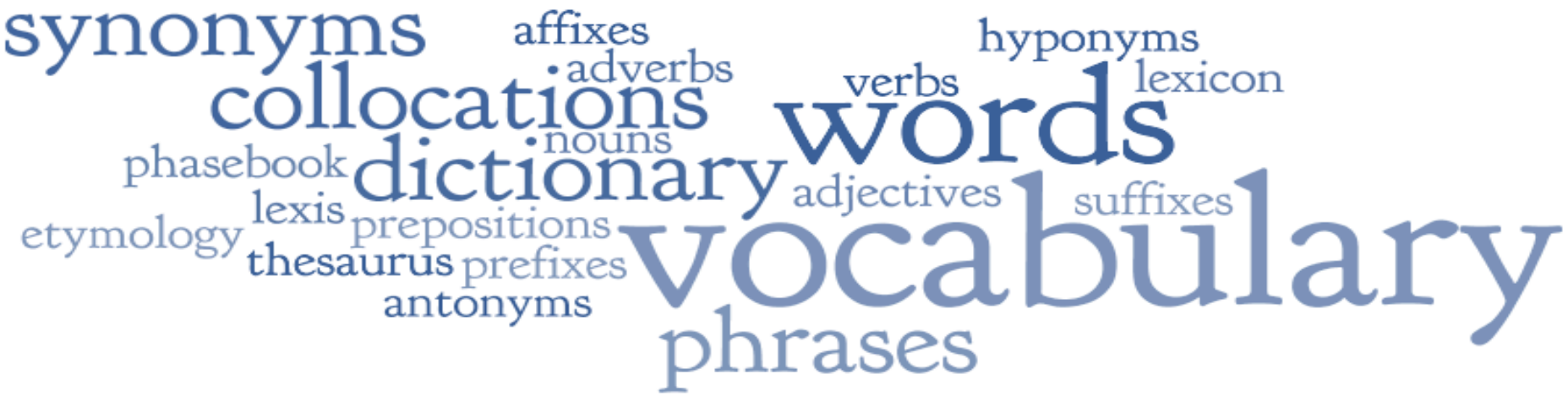
Standards

Evidence

Knowledge

Empowerment

patientslikeme® Data mapped to ICD10, ICF, SNOMED, MedDRA, RxTerms


synonyms
affixes
adverbs
hyponyms
verbs
lexicon
collocations
words
nouns
phasebook
dictionary
adjectives
suffixes
lexis
prepositions
etymology
vocabulary
thesaurus
prefixes
antonyms
phrases


Language of Health
Gait Disturbance
Patient Voice Side Effects
Conditions
Patient Voice Symptoms
Walking into walls
Walking slower
Staggering walk
Right leg dragging
Left leg drag
Solu-Medrol Infusion
Sinemet 25/100
Stumbling walk
Limping in left leg
Shuffle foot when tired
Right foot dragging
Toe dragging
Diabetes Type 1
Gait problems
Problems with stairs
Stumbling
Diabetes Type 2
Diabetic Neuropathy
Major Depressive Disorder
Migraine
Marfan Syndrome
Lyme Disease
Myasthenia Gravis
Bipolar II Disorder
Parkinson's Disease
Multiple Sclerosis
Chronic Fatigue Syndrome
Fibromyalgia
Epilepsy
Walking into walls
Wobbly walk
Shuffling gait
Difficulty walking less than 25m
Intubation
Walk like drunk
Limping in left leg
Uncoordinated left leg
Hip swings heavier right leg to walk
Muscular Dystrophy
Wide stance walking
Difficulty climbing stairs
Leg dragging
Hepatitis C
Herniated Disease
Left leg drag
Limping
Hereditary Spastic Paraplegia
Kidney Transplant
Bump into things-walk like a drunk
Trigeminal Neuralgia
Legg Calve Perthes Disease
Hip Dysplasia
Peripheral Neuropathy
Sickle Cell Disease
Asthma
Hypermobility Syndrome
Hip Dysplasia
Diabetes Type 1
Kidney Transplant
Hypothyroidism
Hypertrophic Obstructive Cardiomyopathy
Failed Back Syndrome
Marfan Syndrome
Chronic Neuralgia
Bipolar I Disorder
Cystic Fibrosis
Dystonia
Cerebral Cavernous Malformation
PMA (Progressive Muscular Atrophy)
Peripheral Arterial Disease
Diabetes Mellitus
Fibromyalgia
PLS (Primary Lateral Sclerosis)
Tinnitus
Parkinson's Disease
Hearing Loss
Peripheral Neuropathy
Chronic Pain Syndrome
ADHD (Attention Deficit/Hyperactivity Disorder)
Psoriatic Arthritis
Walking into walls
Levetiracetam
Ampyra
Effexor
Baclofen
Stumbling
Oxcarbazepine
Interferon beta
Carbidopa-Levodopa 25-100
Vitamin B12 Deficiency
Bump into things-walk like a drunk
Phenytoin
Unsteady gait
Clonazepam
Seroquel
Lyrica
Difficulty walking
Lisinopril
Parkinson's Disease
Cyclothymia
Bupropion
Methotrexate
Cystic Fibrosis
Hydrocodone-Acetaminophen
Surgery
Carbamazepine
Pregabalin
Dysthymia
Gabapentin
PLS (Primary Lateral Sclerosis)
Lamictal
Topiramate
MS (Multiple Sclerosis)
Neurontin
Zonegran
Dystonia
ALS (Amyotrophic Lateral Sclerosis)
Physical Workout Sessions with Davis Johnstone
Alprazolam XR
Pride Jazzy Power Chair
Major Depressive Disorder
Cytoxan
Barrett's Esophagus
Parkinson's Disease
IBS (Irritable Bowel Syndrome)
Panic Disorder
Amantadine
Hashimoto's Thyroiditis
Neuromyelitis Optica
Chronic Fatigue Syndrome
Geodon
Chiari Malformation
Lamotrigine
CRPS II (Complex regional pain syndrome type II)
Bipolar I Disorder
Spinal Cord Injury
TBI (Traumatic Brain Injury)
Hypermobility Syndrome
Difficulty walking
Neurosarcoidosis
Hypothyroidism
Bipolar I Disorder
Major Depressive Disorder
Orthostatic Hypotension
Chronic Neuralgia
Glioblastoma
Polycythemia Vera
Lyme Disease
Celiac Disease
Neurogenic Bladder
Dysthymia
Herniated Disc
Spinal Stenosis
Radiculopathy
Hereditary Spastic Paraplegia
Neurosarcoidosis
Myasthenia Gravis
Degenerative Disc Disease
Trigeminal Neuralgia
Chronic Fatigue Syndrome
Autoimmune Disease Undefined
Episodic Ataxia
Systemic Lupus Erythematosus
Neuromyelitis Optica
PSP (Progressive Supranuclear Palsy)
ALS (Amyotrophic Lateral Sclerosis)
RA (Rheumatoid Arthritis)
Walking slower
Myasthenia Gravis
Bipolar II Disorder
Parkinson's Disease
Migraine
Multiple Chemical Sensitivity
Major Depressive Disorder
Epilepsy
Lyme Disease
Fibromyalgia
Sjogren's Syndrome
Myelomalacia
POTS (Postural Orthostatic Tachycardia Syndrome)
Diabetes Type 2
Autoimmune Disease Undefined
Chronic Fatigue Syndrome
MS (Multiple Sclerosis)
Fibromyalgia
Chronic Fatigue Syndrome
Migraine
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Systemic Lupus Erythematosus
Hypermobility Syndrome
Bipolar I Disorder


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GLASBERGEN
"You have to learn about thousands of diseases, but
I only have to focus on fixing what's wrong with ME!
Now which one of us do you think is the expert?"

“I am not a zero” – Fixing floor effects in an ALS PRO

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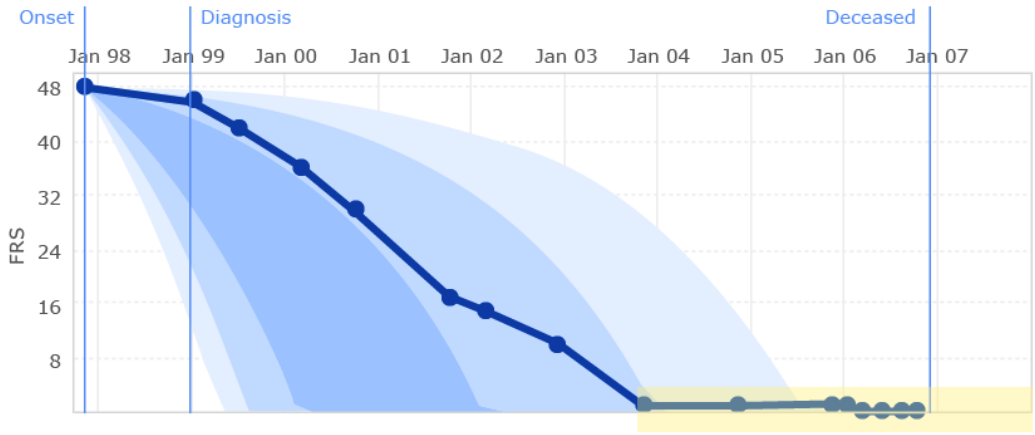


European Journal of Neurology 2009, **16:** 353–359 doi:10.1111/j.1468-1331.2008.02434.x

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]

[a]*PatientsLikeMe Inc., Research & Development, Cambridge, MA, USA; and* [b]*Person living with ALS, patient member of PatientsLikeMe.com*

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

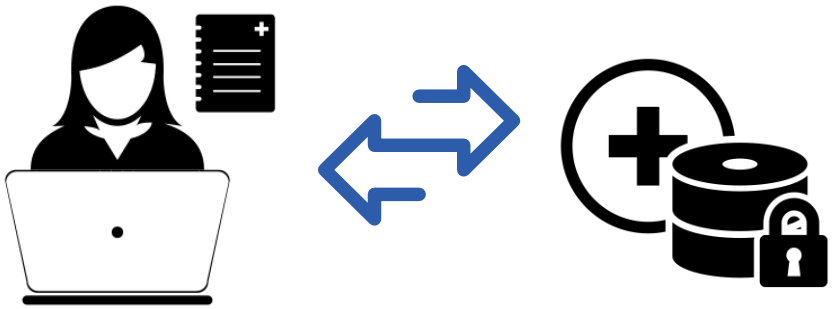
Received 19 August 2008

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

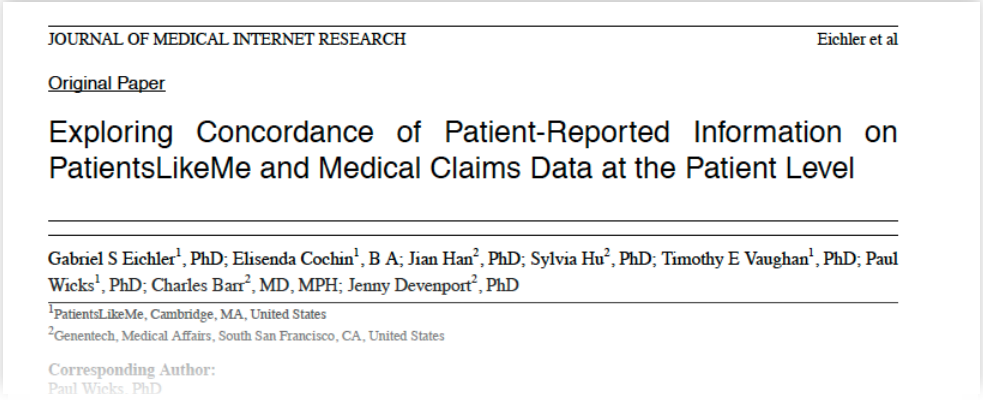
- An ALS patient, Cathy Wolf, noticed that our ALSFRS-R wasn‘t sensitive enough to capture function in advanced ALS and that she had been a “zero” for some years due to the “floor effect” of measure
- Cathy participated as an author of the study despite being quadriplegic, through assistive communication technology
- 200+ patients participated in study to construct and pilot a new, more sensitive instrument including patients much too sick to be studied in normal clinic setting
- 3 new items in new ALSFRS-EX, published in EurJNeurol
- Currently used by VA Brain Bank and a number of academic studies

Concordance between patient reported data and claims data

Genentech
A Member of the Roche Group



- 94% Match rate for patient ID
- 92% Match rate for diagnoses
- 74-100% Match rate for treatments



JOURNAL OF MEDICAL INTERNET RESEARCH Eichler et al

Original Paper

Exploring Concordance of Patient-Reported Information on PatientsLikeMe and Medical Claims Data at the Patient Level

Gabriel S Eichler[1], PhD; Elisenda Cochin[1], B A; Jian Han[2], PhD; Sylvia Hu[2], PhD; Timothy E Vaughan[1], PhD; Paul Wicks[1], PhD; Charles Barr[2], MD, MPH; Jenny Devenport[2], PhD

[1]PatientsLikeMe, Cambridge, MA, United States

[2]Genentech, Medical Affairs, South San Francisco, CA, United States

Corresponding Author:
Paul Wicks, PhD

- Researchers often question whether patients using PLM accurately report their diagnosis and treatment information
- We used de-identified linking methods to assess data concordance in 603 patients with MS or Parkinson's disease
- Where data did not match, was often due to sparse data in claims (e.g. Medicare, patient-reported data preceded claims data window, changed last name)
- Impact: This data increases our confidence that patients are who they say they are and can report information accurately

Patients prompt study of lithium in ALS progression

_computational BIOLOGY ANALYSIS

Accelerated clinical discovery using self-reported patient data collected online and a patient-matching algorithm

Paul Wicks, Timothy E Vaughan, Michael P Massagli & James Heywood

Mathematical description of algorithm. We developed an algorithm to minimize the area between the FRS progression curves of patients and controls over the entire course of the disease (before lithium start). The area is illustrated in **Figure 1c**.

1. Define t_0 as the lithium start date of the patient who took lithium.
2. Determine the patient's FRS at twice-monthly intervals t_i before their study start date (linearly interpolating if necessary), back to time of onset. These are FRS_i.
3. For each control, define their t_0 as one of their reported FRS dates, and determine FRS_i as above.
4. For each patient-control pair, calculate the area:

$$\text{i.Area} = \sum_i abs(FRS_i^{treated} - FRS_i^{control}) \times (t_i - t_{i-1})$$

5. For each patient, choose the control that minimizes this area.

Published alongside matching algorithm and full de-identified dataset for replication

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Wicks P, Vaughan TE, Massagli MP, Heywood J (2011) Accelerated clinical discovery via open data platform and patient-matching algorithm, Nature Biotechnology, 29(5):411-414

Efficacy of Off-Label Drug Use

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21% of all prescriptions are for off-label purposes

73% of these lack scientific evidence for their use

JOURNAL OF MEDICAL INTERNET RESEARCH Frost et al

Original Paper

Patient-reported Outcomes as a Source of Evidence in Off-Label Prescribing: Analysis of Data From PatientsLikeMe

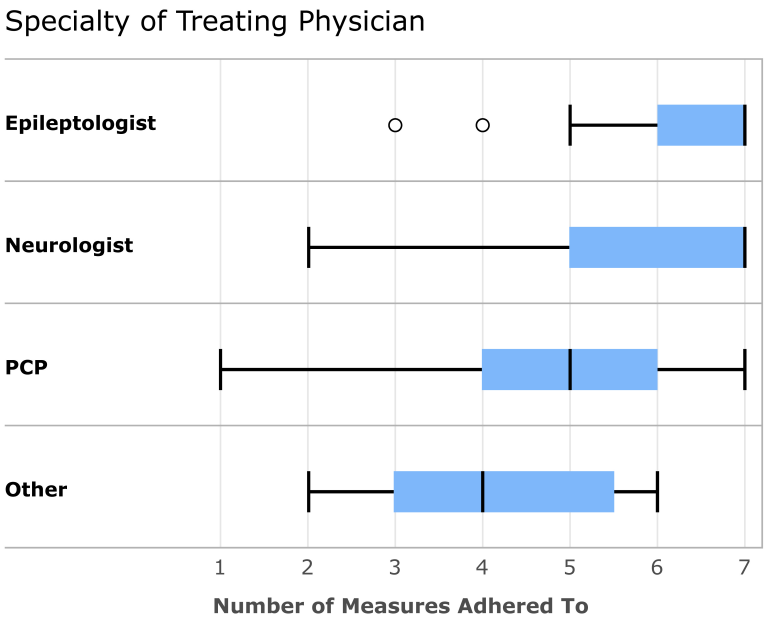
Jeana Frost[1], PhD; Sally Okun[2], RN; Timothy Vaughan[2], PhD; James Heywood[2], BS; Paul Wicks[2], PhD

[1]VU Amsterdam, KankerNL, Amsterdam, Netherlands
[2]PatientsLikeMe Inc., Cambridge, MA, United States

Corresponding Author:
Jeana Frost, PhD

- Many drugs cause N/V but unclear how tolerable different patient groups might find these side effects
- Different “shapes” of nausea per disease and drug e.g. duration, severity, timing, amelioration
- Implications for clinical trials of products with N/V side effects
- Data shared with R&D group

Patients informing quality of care in epilepsy



AMERICAN ACADEMY OF NEUROLOGY®

- Adapted AAN's epilepsy quality measures to self-report instrument
- Found significant differences between treating physician types
- Identified gaps around side effect management, surgery referral, reproductive issues in women
- Fed into National Quality Forum, lead to changes in neurology training and CPD

The 46 questions in this survey have been broken down into the following sections.

1. **Demographics**
   Who is in our response panel, where do they live, and what conditions do they have?

2. **Agency**
   How much about trial awareness and selection is driven by the patient vs. other factors?

3. **Trial awareness**
   How and why do patients become aware of trials?

4. **Evaluation factors**
   How do patients decide to learn more and develop interest in joining a particular trial?

5. **Decision to enroll**
   What are the factors that motivate a patient to decide to enroll?

6. **Decision to withdraw**
   How frequently do patients consider withdrawing, and for what reasons?

7. **Previous participation**
   What kinds of trials have our panel experienced?

8. **Trial satisfaction and impressions**
   What do patients think about the trials they have participated in?

FDA and PatientsLikeMe Collaboration

Research Collaboration Agreement (RCA)	
Goals	To analyze and evaluate [patient-generated] **data** from a novel source for use by the FDA in support of its mission to protect the public health by assuring the safety, efficacy and security of medical products and devices.
Objectives	PatientsLikeMe and the FDA will systematically explore the potential of patient-generated data to inform regulatory review activities related to risk assessment and risk management.
FDA Team	Regulatory Science Staff (RSS) within the Office of Surveillance and Epidemiology (OSE) of the Center for Drug Evaluation and Research (CDER)

Progress	• Weekly Core Team teleconferences
	• PLM onsite visit to FDA in July; FDA onsite visit to PLM in September
	• PLM Data Science Workshop held at FDA in October
	• Data identification and transfer processes initiated
	• Research priorities identified relevant to four main program areas within OSE:
	  • Pharmacovigilance
	  • Pharmacoepidemiology
	  • Medication Error Prevention and Analysis
	  • Drug Product Risk Management

FDA and PatientsLikeMe Collaboration, cont.

Research Priorities and Planning

Early Projects	**Data Characterization Projects** ✓ MedDRA coding validation study ✓ PLM ICSR quality study from reports submitted from MedWatch pilot ✓ Drug treatment coding validation study ✓ PLM patient population generalizability study • Data density and site engagement of PLM population • Treatment indication data assessment • Patient Counts for PLM populations
Emerging Project Concepts	• Real-world treatment observation • Drug safety communication • Exploration of PLM side effect / tolerability information • Detection of medical errors • Exploration of signal from patient-generated data • Evaluation of REMS
Publication Ideas	• Perspective on FDA / PLM Collaboration • History of PLM's patient-generated data

FDA Patient-Generated Health Data Exploration

- Initial exploratory research of PatientsLikeMe PGHD is promising
- Structured PGHD may have potential to be a rich source of patient insights, experiences and preferences
- Additional exploration is needed to fully develop analytic methods
- Need to understand potential to regulatory decision-making process.

MedDRA Coding Validation Study: Overview

Objective: To examine PLM data to gain understanding of:

- PLM coding of user voice entries to MedDRA.
- Whether PLM MedDRA coding is equivalent to or different from FDA MedDRA coding.
- How patient vocabulary compares to MedDRA, and understanding the nuances.

Process:

- 3350 deidentified PLM records with MedDRA coding provided to FDA reviewers
- Review based on International Conference on Harmonization MedDRA Term Selection principles

Initial Findings:

- Over 95% of the PLM verbatim data for adverse events, symptoms and conditions are coded as FAERS data
- Under 5% identified differences in the coding approach due to different data models / purposes

Individual Case Safety Report Comparison Study

Objective: To examine PatientsLikeMe to determine whether:

- PLM reports contain the minimum 4 data elements necessary to be reportable FAERS ICSRs.
- PLM adverse event ICSRs are comparable to FAERS manufacturer or directly reported ICSRs
- PLM ICSRs contain sufficient level of detail for FDA to apply established pharmacovigilance principles in the review and assessment of causality

Process:

- 33 de-identified PLM ICSRs submitted to FAERS in 2008-2009 reviewed for completeness and quality

Initial Findings:

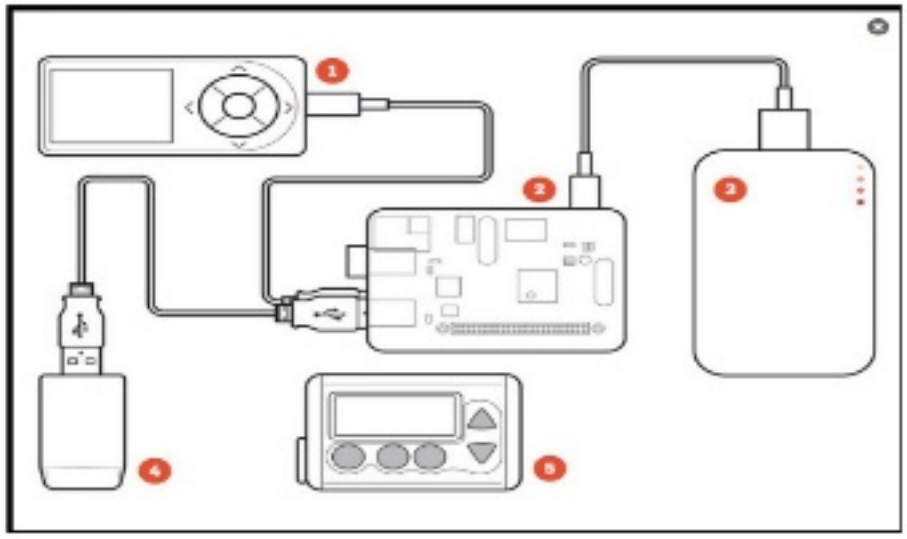
- 2/3 report drug discontinuation or dose adjustment due to ADE
- 3/4 reported on labeled ADEs yet several not recognized by MD
- 1/4 reported on unlabeled ADEs
- Good quality and data in sufficient detail to support FDA's pharmacovigilance work

Hacking Diabetes

#WeAreNotWaiting

We took the "louder alarm system"

...and turned it into an artificial pancreas that auto-adjusts my insulin levels as needed.



-- @DanaMLewis

HHS IDEA LAB

#HackRedTape

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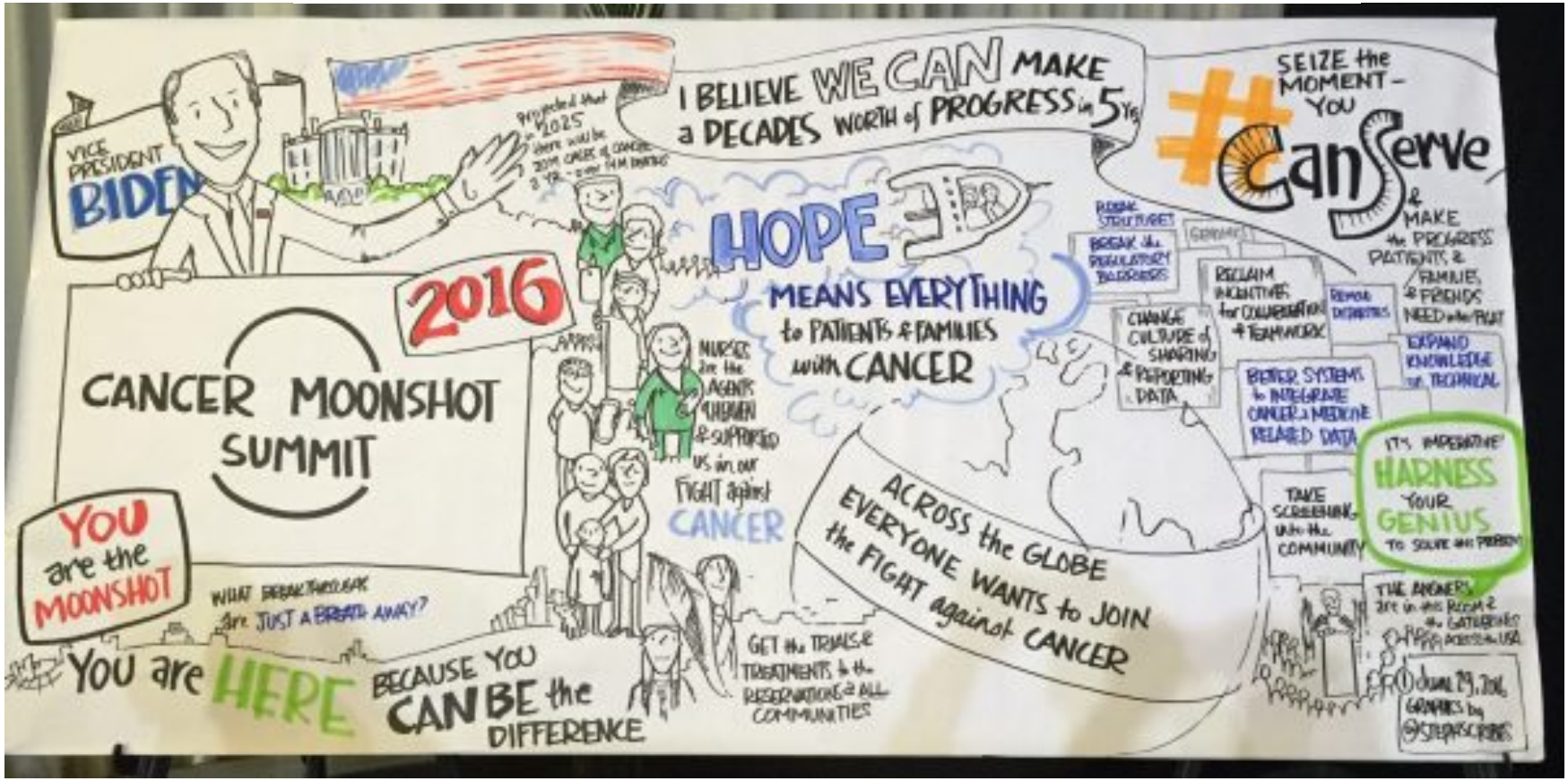
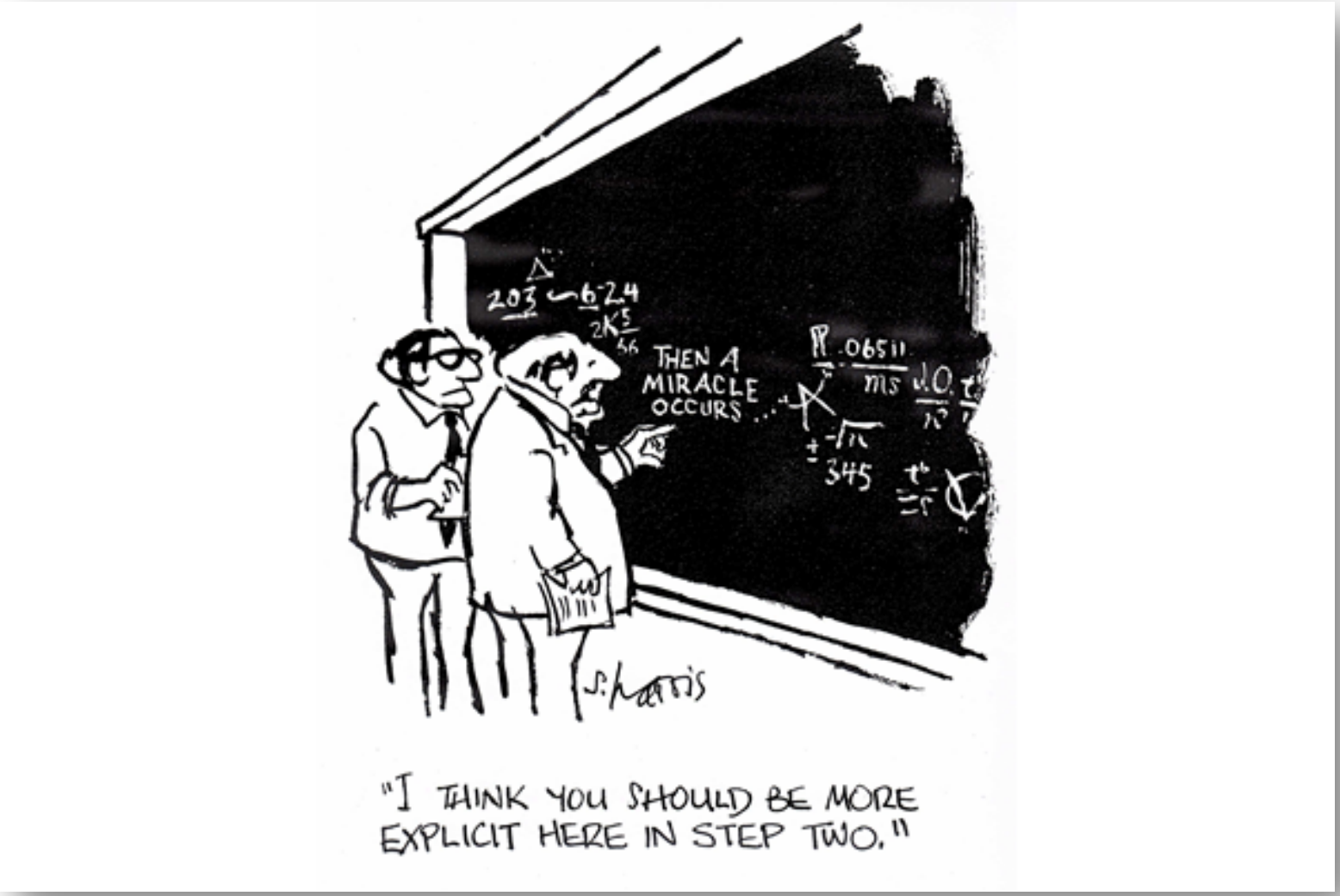


What's next?



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Moving person generated data into evidence…

- Identify and elevate best practices for patient-centric data collection models
- Study patient data characteristics to better understand how it compares to other sources
- Publish in literature to advance knowledge base
- Convert data into actionable information
- Make measure results / scores easy to understand and helpful to all stakeholders
- Connect patient outcomes to care planning and self management

Imagine...

...a connected and data driven world in which the lived experiences of each of us shapes a continually learning system that supports our collective health and well-being...

that's what's next!