



# Research manuscripts bibliography

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# 2024

## Survey of patient experience and management of vasomotor symptoms due to menopause from the PatientsLikeMe community

**Theme:** Menopause, Vasomotor symptoms, women's health, peri-menopause **Collaborator:** Astellas Inc.

This study aimed to describe menopause and treatment experiences of women with vasomotor symptoms due to menopause in the United States. A cross-sectional survey was administered to women 40-65 years of age recruited from PatientsLikeMe, a dedicated online platform for patients. The primary endpoint was women's experiences with menopause and VMS (e.g., night sweats, hot flashes, sleep problems), including impact on HRQoL, as reported in the cross-sectional survey.

Shepherd JA, Shiozawa A, Schild AL, Singh D, Mancuso SA. Survey of patient experience and management of vasomotor symptoms due to menopause from the PatientsLikeMe community. Menopause 31(11):p 979-986, November 2024. | DOI: 10.1097/GME.0000000000002427

## Retrospective text and qualitative analyses of patient experience and management of vasomotor symptoms due to menopause: voices from the PatientsLikeMe community

**Theme:** Menopause, Vasomotor symptoms, women's health, peri-menopause **Collaborator:** Astellas Inc.

Mixed-methods research included women aged 40 to 65 years in the PatientsLikeMe community who were recruited using convenience sampling. Text from online posts by members was analyzed retrospectively using natural language processing. Relevant data, including numbers and percentages of women and frequencies of mentions, were summarized descriptively. Qualitative semi structured interviews were conducted; data, notes, and recordings were transcribed and deidentified, and thematic analyses were performed.

Shepherd JA, Shiozawa A, Schild AL, Singh D, Mancuso SA. Retrospective text and qualitative analyses of patient experience and management of vasomotor symptoms due to menopause: voices from the PatientsLikeMe community. Menopause. 2024 May 28:10-97.

## A Descriptive Study of the Caregiver's Experience of Managing Care for Children and Adolescents With Attention-Deficit/Hyperactivity Disorder During the COVID-19 Pandemic: The UNCOVER Study

**Theme:** ADHD, COVID-19

**Collaborator:** Takeda Pharmaceuticals USA

This study surveyed caregivers of children and adolescents with attention-deficit/hyperactivity disorder (ADHD) to understand the impact of the COVID-19 pandemic on aspects of their disorder, quality of life, and treatment experience. The UNCOVER study (Understanding the patient experience during the COVID-19 pandemic, effects on real world quality of life, symptom management and clinical outcomes in patients treated and untreated for ADHD) surveyed caregivers in the US who are members of PLM and managing care for children and adolescents to better understand the impact of the COVID-19 pandemic on aspects of their disorder and quality of life.

Raveendran S, Burke MC, Klahn K, et al. A descriptive study of the caregiver's experience of managing care for children and adolescents with attention-deficit/hyperactivity disorder during the COVID-19 pandemic: the UNCOVER Study. Prim Care Companion CNS Disord. 2024;26(1):23m03587

## 2023

### [The Experience of Adults With Attention-Deficit/Hyperactivity Disorder in 2021 During the COVID-19 Pandemic: The UNCOVER Study](#)

**Theme:** ADHD, COVID-19

**Collaborator:** Takeda Pharmaceuticals USA

This study surveyed adults with attention-deficit/hyperactivity disorder (ADHD) to understand the impact of the COVID-19 pandemic on aspects of their disorder, quality of life, and treatment experience. The UNCOVER study (Understanding the patient experience during the COVID-19 pandemic, effects on real world quality of life, symptom management and clinical outcomes in patients treated and untreated for ADHD) surveyed adult patients with ADHD in the US who are members of PLM to better understand the impact of the COVID-19 pandemic on aspects of their disorder and quality of life.

Raveendran S, Burke MC, Klahn K, et al. The experience of adults with attention-deficit/hyperactivity disorder in 2021 during the COVID-19 pandemic: the UNCOVER Study. Prim Care Companion CNS Disord. 2023;25(4):22m03474

### [Design of a real-world, prospective, longitudinal, observational study to compare vortioxetine with other standard of care antidepressant treatments in patients with major depressive disorder: a PatientsLikeMe survey](#)

Major depressive disorder (MDD) is a recurrent psychiatric condition that presents challenges in responding to treatment and achieving long-term remission. To improve outcomes, a shared decision-making treatment approach with patient and healthcare practitioner (HCP) engagement is vital. PatientsLikeMe (PLM), a peer community of patientMDDts, provides information on MDD, symptoms, and treatment through forums and resources, helping patients stay engaged in their treatment journey. Data on PLM can be

harnessed to gain insights into patient perspectives on MDD symptom management, medication switches, and treatment goals and measures.

**Theme:** MDD

**Collaborator:** Takeda Pharmaceuticals USA

Raveendran S, Singh D, Burke MC, et al. Design of a real-world, prospective, longitudinal, observational study to compare vortioxetine with other standard of care antidepressant treatments in patients with major depressive disorder: a PatientsLikeMe survey. BMC Psychiatry. 2023;23(1):464. Published 2023 Jun 26. doi:10.1186/s12888-023-04922-6

### [Alpha-1 Insight: A Mixed Method, Retrospective/Prospective Descriptive Study of Diagnostic Journey, Experiences, and Insights of Patients with Alpha-1-Antitrypsin Deficiency](#)

Alpha-1 antitrypsin deficiency (AATD) is an uncommon genetic condition characterized by increased risk of lung/liver disease. We investigated AATD patient experiences including perceptions on sharing clinical data for AATD research of those with self-reported AATD registered on PLM. Semi-structured interviews were conducted and qualitatively evaluated from interview transcripts. Findings show that AATD was commonly misdiagnosed; patients experienced barriers to diagnosis and treatment but were generally satisfied with AATD-related care, particularly after identifying appropriate health care providers/professionals.

**Theme:** AATD

**Collaborator:** University of Chicago and Takeda Pharmaceuticals USA

Raveendran S, Burke M, Singh D, Karim A, Macrayan ER, Patton A, Hogarth DK, Blair C, Hinson J, McCue M. Alpha-1 Insight: A Mixed Method, Retrospective/Prospective, Descriptive Study of Diagnostic Journey, Experiences, and Insights of Patients with Alpha-1-Antitrypsin Deficiency. Journal of Allergy and Clinical Immunology. 2023 Feb 1;151(2):AB182.

## 2019

### [Patient perceptions of their glycemic control and its influence on type 2 diabetes outcomes: an international survey of online communities](#)

**Theme:** Patient perception, diabetes

**Collaborator:** AstraZeneca

This study aimed to assess awareness of glycated hemoglobin (A1C) testing and targets, perceived level of glycemic control and risk of complications, attitudes toward medications and self-management, and regimen-related distress in an international sample of patients with type 2 diabetes (T2D). Limited patient

awareness of their A1C value and the potential complications of poorly controlled T2D, particularly regarding cardiovascular complications, may be a widespread problem. Furthermore, greater patient support may be needed to improve self-management of T2D and to reduce regimen-related distress.

Simacek K, Curran C, Fenici P, Garcia-Sanchez R. Patient perceptions of their glycemic control and its influence on type 2 diabetes outcomes: An international survey of online communities. *Patient Preference and Adherence* 2019; 13:295–307.

## [Measurement of Quality of Life in Patients with Mycosis Fungoides/Sézary Syndrome Cutaneous T-Cell Lymphoma: Development of an Electronic Instrument](#)

**Theme:** PROs, cancer, quality of life

**Collaborator:** Actelion

Mycosis fungoides is a form of lymphoma that affects the skin. Quality of life is affected in a variety of ways but was poorly measured by generic patient reported outcome questionnaires designed for milder dermatological complaints like eczema or psoriasis, and so failed to capture the range of issues affecting patients with this disease. Using the PatientsLikeMe Open Research Exchange, we rapidly developed a 12-item instrument, drawing on data from 126 patients living with the disease in order to validate and test the instrument, which is shared under Creative Commons License and is already in use in clinical practice and trials.

McCaffrey S, Black RA, Nagao M, Sepassi M, Sharma G, Thornton S, Kim YH, Braverman J. Measurement of Quality of Life in Patients with Mycosis Fungoides/Sézary Syndrome Cutaneous T-Cell Lymphoma: Development of an Electronic Instrument. *J Med Internet Res* 2019;21(1):e11302.

## [A Modular Health-Related Quality of Life Instrument for Electronic Assessment and Treatment Monitoring: Web-Based Development and Psychometric Validation of Core Thrive Items](#)

**Theme:** Disease management, self-management, PROs

Most patient-reported outcome measures were developed for paper-and-pencil, not the digital world, and so have a number of limitations that limits their use. They are often long and burdensome, negatively framed, have onerous licensing restrictions, and are either too specific to one disease or so generic as to not apply to real patients. We sought to address this by developing “Thrive”: a universal, modular questionnaire system that has a shared core of items around symptoms, abilities, and “thriving” but which can also layer in additional items for patients with multiple comorbidities. In this study we validated the new Thrive items against comparison measures in a sample of adults with chronic diseases and lay out plans to scale the tool to other areas of medicine.

Wicks P, McCaffrey S, Goodwin K, Black R, Hoole M, Heywood J. A Modular Health-Related Quality of Life Instrument for Electronic Assessment and Treatment Monitoring: Web-Based Development and Psychometric Validation of Core Thrive Items. J Med Internet Res 2019;21(1):e12075.

## [Lunasin does not slow ALS progression: results of an open-label, single-center, hybrid-virtual 12-month trial](#)

**Theme:** ALS, Trials

**Collaborator:** Duke, MGH

In response to media reports of a purported “reversal” of an individual’s ALS, we partnered with Duke and MGH to undertake a study of the nutritional soy peptide Lunasin. Fifty participants were enrolled at a single study site and then completed all follow-up activities virtually via PatientsLikeMe, completing their ALSFRS-R, side effects, treatment evaluation, and weight online with a final study visit at 12 months. Overall, we found this study to have high recruitment, retention, and adherence, though sadly we found no benefit for Lunasin on slowing disease progression. We hope that others will take what we’ve learned about the benefits of conducting a trial semi-virtually to accelerate research and minimize participant burden.

Bedlack R, Wicks P, Vaughan T, Opie A, Blum R, Dios A et al. Lunasin does not slow ALS progression: results of an open-label, single-center, hybrid-virtual 12-month trial. Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration. 2019;1-9.

# 2018

## [Patient: Study Thyself](#)

**Theme:** Patient centeredness, trials

The past 15 years have seen the emergence of a new paradigm in medical research, namely of people living with medical conditions (whether patients, parents, or caregivers) using digital tools to conduct N-of-1 trials and scientifically grounded research on themselves, whilst using the Internet to form communities of like-minded individuals willing to self-experiment. In this editorial, Paul Wicks lays out three case studies of patients leading the way on self-experimentation and lays out a vision of the future to navigate pitfalls in order to empower people to solve some of their own challenges scientifically.

Wicks P, Patient: Study Thyself, BMC Medicine 2018 16:217.

## [Pharmacogenetic profile and major depressive and/or bipolar disorder treatment: a retrospective, cross-sectional study](#)

**Theme:** Bipolar disorder, depression, pharmacogenetics      **Collaborator:** Pathway Genomics

This project sought to connect patient reported treatment history and tolerability, captured through PLM survey tool, with genetic profiling, done by Pathway Genomics. An association was found between patient reported medication efficacy and the Pathway Genomics pharmacogenetic panel, however there was no association between reported efficacy and the panel. The result of this study shows that pharmacogenetic profiling is potentially useful in predicting treatment efficacy in depression.

Tonozzi TR, Braunstein GD, Kammesheidt A, Curran C, Golshan S, Kelsoe J. Pharmacogenetic profile and major depressive and/or bipolar disorder treatment: a retrospective, cross-sectional study. Pharmacogenomics. 2018 Oct; 19(15):1169–1179.

## [DigitalMe: a journey towards personalized health and thriving](#)

**Theme:** Digital health, omics, personalized health, thriving      **Collaborator:** N/A

The experience of health is unique to each individual, yet health care has traditionally focused on what is known about the disease rather than what is known about the person with the disease. This invited commentary describes the vision developed at PatientsLikeMe to create DigitalMe, a personalized virtual avatar made possible through the digitization of individual level data from all available sources.

Okun S, Wicks P. DigitalMe: a journey towards personalized health and thriving. BioMedical Eng Online. 2018;17(1):119.

## [It's time to stop saying "the mind is unaffected" in ALS](#)

**Theme:** ALS, Cognition      **Collaborator:** N/A

Since ALS was first described in 1868, a myth has persisted that while the muscles of the body weaken and atrophy, "the mind is unaffected". However, while the past few decades have produced ample research to show this is not the case, it remains widely held in the clinical and patient literature that cognitive and emotional signs are not a feature of the disease. This editorial in Neurology seeks to overturn that belief, highlighting a recent study showing that by the time all regions of the body are involved, most patients actually do have some type of cognitive, emotional, or behavioral abnormality. Only by facing up to the hard truth of this disease will families and clinicians be better able to understand it and fight back.

Wicks P, Alpert SM. It's time to stop saying "the mind is unaffected" in ALS. *Neurology*. 2018;91(15):679–681.

## [Validating a Framework for Coding Patient-Reported Health Information to the Medical Dictionary for Regulatory Activities Terminology: An Evaluative Study](#)

**Theme:** Health informatics, Data curation, Patient-generated health data

**Collaborator:** FDA

This study aimed to compare the coding of patient generated health data (PGHD) on the PatientsLikeMe (PLM) platform to how the same reports would be coded if they were received by the Food and Drug Administration (FDA). Unlike many other platforms where PGHD exists, PGHD on PLM is captured in structured data fields, reviewed by a team of health care professionals, and coded to medical ontologies such as ICD10 and MedDRA. An FDA coding expert retrospectively reviewed a dataset of PGHD and evaluated the medical accuracy and appropriateness of the codes assigned by the PLM Health Data Integrity team. Overall, the FDA coding expert agreed with the coding for >97% (3,140/3,234) of the reports. PLM MedDRA coding of PGHD was generally comparable to how the FDA would code similar data.

Brajovic S, Blaser DA, Zisk M, Caligtan C, Okun S, Hall M, Pamer CA. Validating a Framework for Coding Patient-Reported Health Information to the Medical Dictionary for Regulatory Activities Terminology: An Evaluative Study. *JMIR Med Inform*. 2018;6(3):e42.

## [The Impact of Disease-Modifying Therapy Access Barriers on People With Multiple Sclerosis: Mixed-Methods Study](#)

**Theme:** Cost sharing, multiple sclerosis, patient adherence

**Collaborator:** Novartis

This study describes the barriers people with Multiple Sclerosis (MS) face when trying to access to a disease-modifying therapy (DMT) and the impact of these barriers on their adherence and relapses. Results are based on a survey of 507 PatientsLikeMe members with relapsing-remitting MS along with follow-up interviews with 10 participants who had difficulty accessing a DMT and who reported having a relapse during the time of this difficulty. Nearly half (46%) of participants had ever experienced problem getting access to the DMT prescribed by their doctor, and among those who experienced access issues, about half had a relapse during that time. The major barriers to access were authorization issues by insurance companies and out-of-pocket costs. Among those who faced barriers, getting access involved hours spent contacting many stakeholders, including doctors, pharmacies, insurers, and pharmaceutical companies, among others. That is, at a time when people with RRMS are likely to be feeling at their worst, they have to do the most work and overcome the financial barriers to get the medication that can help them to feel better or prevent disease progression.



Simacek KF, Ko JJ, Moreton D, Varga S, Johnson K, Katic BJ. The Impact of Disease-Modifying Therapy Access Barriers on People With Multiple Sclerosis: Mixed-Methods Study. J Med Internet Res. 2018;20(10):e11168.

### Understanding 'Good Health care' from the Patient's Perspective: Development of a Conceptual Model Using Group Concept Mapping

**Theme:** Concept mapping, Patient-centered, Conceptual development, Performance measures

**Collaborators:** University of Maryland, Robert Wood Johnson Foundation

The primary objective of this study was to conceptualize "good healthcare" and aspects of healthcare that matter most to patients by utilizing concept mapping (CM). Secondary objectives including an evaluation of the relative priorities of concept mapping domains among (1) various patient populations, including different demographic and clinical populations, and (2) patients and stakeholders (clinicians, researchers, purchasers, measure developers, health IT). This process generated more than 1200 statements which were then mapped into 10 major concepts and revealed ten key areas to describe "good care": Active Patient Role in Care, Effective Treatment Selection, Effective Treatment Delivery, Focus on Outcomes, Doctor or Provider Competence, Individualized and Empathic Care, Collaborative Care, Effective Staff Communication, Care Accessibility and Cost, and Office Management. This comprehensive qualitative and quantitative investigation is an important first step in developing patient-reported outcome performance measures that capture the aspects of health care that are most important and relevant for patients.

McCaffrey SA, Chiauuzzi E, Chan C, Hoole M. Understanding 'Good Health care' from the Patient's Perspective: Development of a Conceptual Model Using Group Concept Mapping. Patient. 2018 Jul 2.

### Patients' role and rights in research

**Theme:** Patient centeredness, Trials

**Collaborator:** None

While patient involvement and engagement is growing in stature, critics contend that for many researchers it is merely "virtue signaling", helping to keep the status quo entrenched and not truly working for the interests of patients. In this editorial, a group of patient research advocates highlight the stumbling blocks in involving patients in research and announce a new policy for the BMJ group of journals that will enshrine requirements to provide details of how patients and other relevant communities will be informed about their research.

Wicks P, Richards T, Denegri S, Godlee F. Patients' roles and rights in research. BMJ. 2018;362:k3193.

## [Validation of the shortened Perceived Medical Condition Self-Management Scale in patients with chronic disease](#)

**Theme:** Disease management, self-management, PROs      **Collaborator:** Vanderbilt University

How people living with chronic illness perceive themselves at managing their condition (“self-efficacy”) is a crucial part of helping them achieve wellness. Using our Open Research Exchange, our collaborators at Vanderbilt University were able to quickly validate a shortened version of the Perceived Medical Condition Self-Management Scale (PMCSMS), cutting the number of items in half while still maintaining good psychometric properties, leading to increased efficiency in using it in research and clinical practice.

Wild MG, Ostini R, Harrington M, Cavanaugh KL, Wallston KA. Validation of the shortened Perceived Medical Condition Self-Management Scale in patients with chronic disease. *Psychol Assess*. 2018 Oct;30(10):1300–1307.

## [Scaling PatientsLikeMe: Web-based Survey of Benefits Arising for Members with Chronic Illness Using a “Generalized” Platform](#)

**Theme:** Patient-centeredness, Self-management, Activation      **Collaborator:** AstraZeneca

Why does PatientsLikeMe exist? To help our members, of course, but how do we know if we’re actually helping? In this survey of over 7,000 members we learned that most members felt they had a better understanding of their condition as a result of using the site (67% agreed), which treatments were available (60%), and making decisions about treatments (58%). About half of patients (52%) felt they had better conversations with their healthcare professional, and 33% said we’d helped them be more adherent to their medication. This study demonstrates how patients can help patients to live better every day.

Wicks P, Thorley EM, Simacek K, Curran C, Emmas C. Scaling PatientsLikeMe via a “Generalized Platform” for Members with Chronic Illness: Web-Based Survey Study of Benefits Arising. *J Med Internet Res*. 2018;20(5):e175.

## [Understanding How Chorea Affects Health-Related Quality of Life in Huntington Disease: An Online Survey of Patients and Caregivers in the United States](#)

**Theme:** Patient-centeredness, Huntington’s disease, Quality of Life

**Collaborator:** Teva Pharmaceuticals

As a hereditary, neurodegenerative disease with progressively worse motor, cognitive and emotional-behavioral symptoms, Huntington’s disease (HD) has a profound impact on patients and families’ lives. There is no cure and treatment is limited. One of the hallmark symptoms in HD, chorea – characterized as dancelike, irregular, and unpredictable movements – can be treated pharmacologically, though

effectiveness varies. This survey asked patients their experience and perspective on living with HD, with a particular focus on chorea, and how this symptom impacts quality of life and functioning. Patients felt that management of HD-related chorea to be important, and those experiencing a greater impact of chorea on HRQoL and overall functioning indicated a greater desire to participate in medical decision making. When evaluating the impact of chorea on HRQoL among patients, anxiety, stigma, and their outlook on their well-being are important contextual factors that need to be considered in treatment plans.

Thorley EM, Iyer RG, Wicks P, Curran C, Gandhi SJ, Abler V, Anderson KE, Carlozzi NE. Understanding How Chorea Affects Health-Related Quality of Life in Huntington Disease: An Online Survey of Patients and Caregivers in the United States. *Patient*. 2018.

### [Patient engagement in type 2 diabetes mellitus research: what patients want](#)

**Themes:** Diabetes mellitus, Patient involvement, Research priorities

**Collaborator:** Janssen

Patients are increasingly being included as collaborators in research, however few studies have asked patients for their thoughts about participating in research. Based on a series of posts in the Type 2 Diabetes (T2D) forum, we asked PatientsLikeMe members what they think about the state of T2D research and about their own participation in research. We discuss the ways that patients want to be involved in research, and the merits of using an online forum to learn about patient preferences.

Simacek KF, Nelson T, Miller-Baldi M, Bolge SC. Patient engagement in type 2 diabetes mellitus research: what patients want. *Patient Preference and Adherence*. 2018;12:595-606.

### [No tears in heaven: did the media create the pseudo-phenomenon “altitude-adjusted lachrymosity syndrome \(AALS\)”?](#)

**Theme:** Pseudo-bulbar affect

**Collaborator:** N/A

Have you ever cried while watching a movie on a plane? Many celebrities say they have, and some have even suggested that being in an airplane may somehow increase the likelihood that any of us would cry or feel more tearful. That hypothesis is intriguing, because it could tell us a lot about a neurological condition called pseudobulbar affect (PBA) which causes uncontrollable weeping in people with ALS, MS, stroke, and dementia. We conducted a survey of over 1,000 recent flyers who had seen a movie both on a plane and on the ground and were surprised to find there was no difference at the likelihood of crying in the air vs. on the ground. We concluded this was an urban legend perpetuated by social media because people choose to watch more dramas and weepy “guilty pleasure” films when they fly, bringing this urban legend firmly back to earth.

Wicks P, Lancashire L. No tears in heaven: did the media create the pseudo-phenomenon “altitude-adjusted lachrymosity syndrome (AALS)”?. *PeerJ*. 2018;6:e4569.

## [Free-Living Physical Activity Monitoring in Adult US Patients with Multiple Sclerosis Using a Consumer Wearable Device](#)

**Themes:** MS, Activity monitors, Wearable physical activity monitoring      **Collaborator:** Biogen

This study captured mobility patterns in data in MS patients using Fitbits. It is possible to estimate participant activity levels from as little as 2-day step estimates and self-reported disability. This may reduce the burden on patients, clinicians, and researchers when monitoring clinical status in MS.

DasMahapatra P, Chiauuzi E, Bhalerao R, Rhodes J. Free-Living Physical Activity Monitoring in Adult US Patients with Multiple Sclerosis Using a Consumer Wearable Device. Digit Biomark. 2018;2:47-63.

## 2017

## [Comparison of rates of nausea side effects for prescription medications from an online patient community versus medication labels: an exploratory analysis](#)

**Themes:** Pharmacovigilance, Real-world evidence, Crowdsourcing

**Collaborator:** AstraZeneca

While medication labels are considered the authoritative resource for medication information, emerging research suggests that patient-generated health data (PGHD) are a valuable tool to understand the ways in which patients experience medications in real world settings. This study compared adverse drug reaction rates from FDA medication labels with those self-reported by patients from PatientsLikeMe. Nausea rates for 163 medications were compared between the two data sources. In general, patient reported rates of nausea were lower than those reported in medication labels. This discrepancy demonstrates that medication labels may not comprehensively describe the patient experience. Results suggest that a combination of information from different sources may provide a more rounded and holistic view on medication safety and tolerability.

Blaser DA, Eaneff S, Loudon-Griffiths J, Roberts S, Phan P, Wicks P, Weatherall J. Comparison of rates of nausea side effects for prescription medications from an online patient community versus medication labels: an exploratory analysis. AAPS Open. 2017. 3:10.

## [Health Informatics: An Interprofessional approach \(2nd Edition\)](#)

**Themes:** Health informatics, ePatient, mHealth

**Collaborator:** N/A

This textbook for health professionals describes how information technology intersects with health care. PatientsLikeMe staff Sally Okun and Christine Caligtan authored the chapter on ePatients, mHealth and patient networks for both the first and second edition. Additional chapters cover topics including information systems and applications such as electronic health records, clinical decision support, telehealth, and social media tools, data science and analytics. The first and second editions won American Journal of Nursing Book of the Year award for 2013 and 2017 respectively.

Nelson, R, Staggars, N Health informatics: An interprofessional approach (2<sup>nd</sup> Edition). 2017. St. Louis, Missouri: Elsevier.

## [Patient perspectives on neuromyelitis optica spectrum disorders: Data from the PatientsLikeMe online community](#)

**Themes:** Neuromyelitis optica, Symptoms

**Collaborator:** AstraZeneca

NMO (also known as Devic's disease) is a rare disorder once thought to be a variant of multiple sclerosis (MS). In this paper we describe the patient experience of living with NMO based on data from our community on PatientsLikeMe. Median delay from symptom onset to diagnosis was about a year, with the three most frequently reported symptoms being fatigue, pain, and stiffness/spasticity. Relative to MS patients, NMO patients had more vision problem and fewer cognitive problems.

Eaneff S, Wang V, Hanger M, Levy M, Mealy MA, Brandt AU, Eek D, Ratchford JN, Nyberg F, Goodall J, Wicks P. Patient perspectives on neuromyelitis optica spectrum disorders: Data from the PatientsLikeMe online community, MS and Related Disorders. 2017. 17:116-122

## [Building a learning health community: By the people, for the people](#)

**Themes:** Patient empowerment, Learning health system

**Collaborator:** N/A

This paper describes the development of the Patient and Caregiver Journey Framework and related patient-informed principles for design and measurement created by PatientsLikeMe in partnership with patients and caregivers using qualitative research methods, immersive observation and directed one-on-one conversations. These tools provide a person-centric foundation upon which the knowledge and experience of patients and caregivers are collected, curated, aggregated and shared to support a data-driven learning health community continuously powered by the people and for the people.

## Patient and Stakeholder Engagement in Designing Pragmatic Clinical Trials

**Themes:** Pragmatic clinical trials, Stakeholders, Patient-centeredness

**Collaborator:** University of Maryland

To ensure trials are truly reflective of what is meaningful to patients, patients and stakeholders should be engaged during the entire trial process—from planning the trial through conducting the trial to disseminating the results. Identifying, recruiting, training, continually engaging, and compensating an advisory committee of patients and stakeholders to serve as a resource and guide through the trial process is one way to increase the patient-centeredness of a trial. Patients can help design recruitment and retention strategies, co-develop endpoints, review consent forms, and identify appropriate dissemination channels. Patient involvement throughout the trial benefits not only the research team but the patients themselves as well as future patients. This chapter explores how to increase patient-centeredness in clinical trials by engaging patients and stakeholders throughout the pragmatic clinical trial process.

Hung A, Baas C, Bekelman J, Fitz-Randolph M, Mullins C D. Patient and Stakeholder Engagement in Designing Pragmatic Clinical Trials. In: Birnbaum, H G, Greenberg, P E (Eds.), *Decision Making in a World of Comparative Effectiveness Research: A Practical Guide*. 1st ed. ADIS; 2017. 137–152.

## The experience of weight loss and its associated burden in patients with non-small cell lung cancer: results of an online survey

**Themes:** Non-Small Cell Lung Cancer, Weight loss

**Collaborator:** Helsinn Therapeutics

Cachexia is unintentional significant weight loss that can occur with diseases like lung cancer. In this survey, 95 PatientsLikeMe members with advanced non-small cell lung cancer were asked about hunger, weight loss, and the impacts on their lives. Patients who experienced considerable weight loss reported worse function, lower health-related quality of life, and more symptoms such as fatigue and changes in food taste than those who did not have weight loss. The reported fatigue also directly impacted their ability to do daily chores, climb stairs, or manage daily activities such as showering. Identifying and managing cachexia should improve cancer patients' lives.

Rodriguez A M, Braverman J, Aggarwal D, Friend J, Duus E. The experience of weight loss and its associated burden in patients with non-small cell lung cancer: results of an online survey. *Journal of Cachexia, Sarcopenia and Muscle – Clinical Reports*. 2017. 2:2.

## [Clinical trials from the patient perspective: survey in an online patient community](#)

**Theme:** Clinical Trials

**Collaborator:** N/A

Developing new medicines relies on the successful completion of clinical trials, but it is difficult to recruit and retain patient volunteers. This study sought to describe drivers and barriers to trial participation, as well as condition-specific trial preferences. We found that most patients are willing to enroll, yet very few are invited. Trial participation is often burdensome, but patients are willing to help improve trial design. Researchers should allow patients to help design better trials to overcome recruitment and retention issues and hasten the development of new medicines.

DasMahapatra P, Raja P, Gilbert J, Wicks, P. Clinical trials from the patient perspective: survey in an online patient community. BMC Health Services Research. 2017. 17:166.

# 2016

## [PatientsLikeMe: Crowdsourced Patient Health Data as a Clinical Tool in Psychiatry](#)

**Themes:** Psychiatry, Crowdsourcing

**Collaborator:** National Network of Depression Centers

A recent survey found that almost half of psychiatric patients use social media—and that most activity is focused on searching for information about mental disorders, services, and medications. However, much of the information available on mental health websites may be non-specific and difficult for patients to apply in a personalized way. Online resources that allow patients to better identify other patients like themselves, find support, and share meaningful information about treatment options may offer significant clinical advantages. This article reviews ways in which clinicians can integrate patient research networks such as PatientsLikeMe to enhance treatment delivery.

Chiauzzi E, Lowe M. PatientsLikeMe: Crowdsourced Patient Health Data as a Clinical Tool in Psychiatry. Psychiatric Times. 2016. 33(9), 24G-24H.

## [Communicating laboratory test results for rheumatoid factor: what do patients and physicians want?](#)

**Themes:** Rheumatoid Arthritis, Patient-Centeredness

**Collaborator:** Genentech

Most laboratory test reports are not designed to be patient-friendly. This study explored patient and physician attitudes toward laboratory test reports and ways these reports could be improved. A total of 348 physicians in four specializations (general practitioners, internal medicine, rheumatology, and allergy/immunology) across four countries (US, UK, Germany, Switzerland) were surveyed about their current opinions about laboratory test reports and possible improvements. Both physicians and patients expressed a desire for patient-friendly information on laboratory reports to improve communication. Physicians expressed a need for education for patients around false-positive and false-negative results within laboratory reports, while patients sought context around the meaning of results, relevance to other tests, and follow-up steps.

Kelman A, Robinson C, Cochin E, Ahluwalia N, Braverman J, Chiauzzi E, Simacek K. Communicating laboratory test results for rheumatoid factor: what do patients and physicians want? Patient Preference and Adherence. 2016. 10:2501-2517.

### [Getting stem cell patients “on the grid”](#)

**Themes:** ALS, Learning Health System

**Collaborator:** N/A

When an editorial in Nature Biotechnology decried the number of unlicensed stem cell clinics offering unregulated treatment in the United States, we outlined the history of this practice in ALS research spanning the range from genuine and rigorous research through to outright fraud. The widespread availability of high-quality patient-reported outcome measures such as the ALSFRS-R and of historical control data remove any excuse for unlicensed practitioners to contribute their data to illuminate the potential for new discovery. However we caution that the medical system itself should also adopt the same approach.

Wicks P, Heywood J. Getting stem cell patients 'on the grid'. Nature Biotechnology. 2016;34(12):1228-1230.

### [It's a long shot, but it just might work! Perspectives on the future of medicine](#)

**Themes:** Innovation, Learning Health System

**Collaborator:** Various

What does the future of medicine hold? In collaboration with BMC Medicine we asked six researchers to share their most ambitious and optimistic views of the future, grounded in the present but looking out a decade or more from now to consider what's possible. They paint a picture of a connected and data-driven world in which patient value, patient feedback, and patient empowerment shape a continually learning system that ensures each patient's experience contributes to the improved outcome of every patient like them, whether it be through clinical trials, data from consumer devices, hacking their medical devices, or defining value in thoughtful new ways.



Wicks P, Hotopf M, Narahayn VA, Basch E, Weatherall J, Gray M. It's a long shot but it just might work! Perspectives on the future of medicine. BMC Medicine. 2016. 14:176.

### [PatientsLikeMe Online Epilepsy Community: patient characteristics and predictors of poor health-related quality of life](#)

**Theme:** Epilepsy

**Collaborator:** UCB

In collaboration with UCB, PatientsLikeMe established a community for people living with seizures in January 2010 which allows members to track their seizures, medication, and quality of life. As part of the community we designed a study using validated quality of life measures to learn more about the major drivers of distress in the community. From 3,073 respondents surveyed we found a high rate of key symptoms such as memory problems, fatigue, and problems concentrating. Many patients reported medication side effects and quality of life was significantly poorer in those with symptoms, side effects, tonic-clonic seizures, and recent onset. The study suggests taking a holistic approach to management.

De la Loge C, Dimova S, Mueller K, Phillips G, Durgin TL, Wicks P, Borghs S. PatientsLikeMe Online Epilepsy Community: Patient Characteristics and Predictors of Poor Health-Related Quality of Life, Epilepsy & Behavior. 2016. 63:20-28.

### [Attribute Development Using Continuous Stakeholder Engagement to Prioritize Treatment Decisions: A Framework for Patient-Centered Research](#)

**Themes:** Methods Development

**Collaborator:** University of Maryland

Many types of quantitative analysis require the use of lists of thematic concepts, which are usually developed qualitatively. This methodology paper describes an approach for selecting, validating, and prioritizing attributes for health care decision making analyses by leveraging patient input in a structured qualitative fashion.

dosReis S, Castillo W, Ross M, Fitz-Randolph M, Vaughn-Lee A, Butler B. Attribute Development Using Continuous Stakeholder Engagement to Prioritize Treatment Decisions: A Framework for Patient-Centered Research. Value in Health. 2017. 19(6):758-766.

### [The real-world patient experience of fingolimod and dimethyl fumarate for multiple sclerosis](#)

**Theme:** MS

**Collaborator:** Novartis

Disease-modifying therapies in MS were for many years only available to patients as an infusion or an injection, which could be burdensome to patients who struggled with adherence and side effects. In the past few years a number of oral medications have been approved for the treatment of MS which allow patients to treat their condition with daily pills, however it remains unclear what the real-world experience of these treatments might be in comparison with results from controlled clinical trials. In a survey of 281 patients who had taken either fingolimod or dimethyl fumarate we used validated measures to probe their perceived efficacy, side effects, burden, and overall satisfaction to better understand the patient treatment journey.

Wicks P, Rasouliyan L, Katic B, Nafees B, Flood E, Sasane R. The real-world patient experience of fingolimod and dimethyl fumarate for multiple sclerosis. BMC Research Notes. 2016. 9:434.

### [Women's experience of menopause in an online MS cohort: A case series](#)

**Themes:** MS, Qualitative

**Collaborator:** MGH

There are many mysteries about MS including its higher prevalence among women and its onset during peak reproductive years. Some researchers have also observed a reduction in relapse during pregnancy or variability in response to hormone replacement therapy, and in an earlier study the authors of the present study identified that MS severity scores worsened after early or surgical menopause. In this follow-up study we analysed the qualitative free text data from the earlier study. Many women reported an overlap between MS symptoms and menopausal symptoms and complained of hot flashes triggering pseudoexacerbations. This work underlines the pressing need to understand the impact of menopause and hormones more generally on MS.

Bove R, Vaughan T, Chitnis P, Wicks P, De Jager PL. Women's experience of menopause in an online MS cohort: A case series, MS and Related Disorders. 2016. 9:56-59.

### [What Do Ovarian Cancer Patients Expect From Treatment?](#)

**Themes:** Ovarian Cancer, Qualitative

**Collaborator:** AstraZeneca

Although ovarian cancer remains one of the deadliest forms of cancer, treatments are improving, and it's becoming more likely that patients will be living in remission for longer. Patients reported that when they were first diagnosed, they were focused on survival and getting the cancer removed, with surgery typically being the first treatment. After they got through treatment, there was a shift to thinking about the long term and what that means for their quality of life going forward. Many felt that the long-term side-effects of treatments were not fully made clear to them, and some struggled to adjust to their new normal. We suggest that as treatments become better, providers need to help patients understand how this will impact their lives in the longer term, beyond immediate side effects of the treatment.

Simacek K, Raja P, Chiauzzi E, Eek D, Halling K. What Do Ovarian Cancer Patients Expect From Treatment?: Perspectives From an Online Patient Community. Cancer Nurs. 2017 Sep/Oct;40 (5):E17-E27.

## [Understanding preferences for type 2 diabetes mellitus self-management support through a patient-centered approach: a 2-phase mixed-methods study](#)

**Themes:** Diabetes, Disease-management

**Collaborator:** Janssen

Although shown to be effective across various health outcomes, diabetes management support programs lack patient participation. Understanding patient perspectives on diabetes management can be useful for improving support programs. Quantitative and qualitative data reveal that patients in programs most frequently name diet and weight loss their central concern, and health-care professionals their most preferred source of support. Results also revealed that patients prefer online and printed diabetes education materials, which could inform patient-provider support collaborations.

Lopez J, Katic B, Fitz-Randolph M, Jackson R, Chow W, Mullins CD. Understanding preferences for type 2 diabetes mellitus self-management support through a patient-centered approach: a 2-phase mixed-methods study. BMC Endocrine Disorders. 2016;16(1):41.

## [Exploring concordance of patient-reported information on PatientsLikeMe and medical claims data at the patient level](#)

**Theme:** Data Validity

**Collaborator:** Genentech

While data gathered on PatientsLikeMe has been used for a variety of purposes, questions remain about the quality of information gathered in this new way. Data collected online in other contexts such as online restaurant reviews may be subject to faking, so how can we be confident that patients are who they say they are? In this study we were able to link 94% of respondents to insurance claims using linking methods, thereby increasing confidence in the data through methods that maintain patient anonymity.

Eichler GS, Cochin E, Han J, Hu S, Vaughan TE, Wicks P, et al. Exploring concordance of patient-reported information on PatientsLikeMe and medical claims data at the patient level. J Med Internet Res. 2016;18(5):e110.

## [Increasing patient involvement in drug development](#)

**Theme:** Patient Centeredness

**Collaborator:** Novartis

While patients are increasingly invited to play a role in shaping medical research through funding initiatives like PCORI or dissemination channels that are “Patients Included”, pharmaceutical development remains an area where manufacturers fail to engage with patients in identifying real unmet needs, choosing outcomes that matter to patients, or designing trials to minimize burden. Through stakeholder interviews we identify potential areas for improvement and propose a framework to incorporate patient-centeredness to pharma.

Lowe M, Blaser DA, Cone L, Arcona S, Ko J, Sasane R et al. Increasing patient involvement in drug development. *Value in Health*. 2016 Sep-Oct;19-(6): 869-878.

### [Factors in patient empowerment: A survey of an online patient research network](#)

**Theme:** Patient Centeredness

**Collaborator:** Genentech

In this site-wide survey of almost 4,000 PLM members we described some of the challenges faced by a subset of dissatisfied patients including difficulty setting treatment goals with their physician and getting enough time with their healthcare professionals. By sampling across different conditions, we could also identify those conditions where patients appeared relatively disempowered such as fibromyalgia or chronic fatigue syndrome, highlighting the importance of stigma in empowerment. Many patients feel their main healthcare provider doesn't do enough to monitor their condition. A sense of ownership and control by the patient could perhaps be increased by a peer community of patients.

Chiauzzi E, DasMahapatra P, Cochin E, Bunce M, Khoury R, Dave P. Factors in patient empowerment: a survey of an online patient research network. *The Patient - Patient-Centered Outcomes Research*. 2016.

### [Crowdsourcing Advancements in Health Care Research: Applications for Cancer Treatment Discoveries](#)

**Themes:** Crowdsourcing, Oncology

**Collaborator:** NCI

Crowdsourcing refers to a range of approaches to solving problems through a distributed network of solvers. Examples of the crowdsourcing in the modern world include Wikipedia, SETI@Home, and the connected arrays of GPS and phone apps that allow commuters to avoid traffic. While nascent, there are increasing forays of crowdsourcing into healthcare such as the protein-folding videogame FoldIt, the ALS Prize4Life Biomarker Challenge, and online platforms such as 23andMe and PatientsLikeMe. In this book chapter we review the fundamentals, provide case studies, and recommend methods for engagement.

Chiauzzi E, Eichler G, Wicks P. Crowdsourcing advancements in health care research: applications for cancer treatment discoveries. In: Hesse B, Ahern D, Beckjord E, ed. *Oncology Informatics: Using Health Information Technology to Improve Processes and Outcomes in Cancer*. 1st ed. Elsevier; 2016. p. 307-326.

## [Clinical Trial Blinding in the Age of Social Media](#)

**Themes:** Trials

**Collaborator:** N/A

While the double-blind randomized placebo-control trial remains the gold standard in testing the potential efficacy and safety of new treatments, the widespread availability of information and networks enabled by the Internet mean that such trials can easily become unblinded. While trial unblinding has been known in the past (particularly in HIV during the 1980's), the ease with which patients can use Twitter, Facebook, or PatientsLikeMe to crowdsource side effects and outcomes in order to draw their own conclusions represents a new challenge to researchers. In this book chapter we review the history, provide cases, and make recommendations on forging a new social contract with patients as partners.

Wicks P. Clinical trial blinding in the age of social media. In: Robertson C, Kesselheim A, ed. by. Blinding as a solution to bias. 1st ed. Elsevier; 2016. p. 97-106.

## [Partnering With Patients to Rapidly Develop a Quality-of-Life \(QoL\) Measure in Mycosis Fungoides/Sézary Syndrome Type Cutaneous T-cell Lymphoma](#)

**Themes:** PROs, Oncology

**Collaborator:** Actelion

Mycosis Fungoides is a rare form of Non-Hodgkin's Lymphoma that affects the skin. Measuring the impact of the disease is important for research and clinical care purposes, yet we identified an absence of a validated measure specific to the condition, meaning that researchers were dependent on generic measures of skin problems or quality of life. Using PatientsLikeMe's Open Research Exchange we were able to rapidly field online concept elicitation (with telephone follow-ups) to 21 patients, obtain item feedback from 42 patients online, and then conduct a full fielding to 126 patients for psychometric and test-retest fielding in a matter of weeks.

Wicks P, Sepassi M, Sharma G, Carlson Delogne M. Partnering with patients to rapidly develop a Quality-of-Life (QoL) measure in Mycosis Fungoides/Sézary Syndrome Type Cutaneous T-cell Lymphoma. Am J of Manag Care. 2016;22(8).

## [Patients' motivations and interest in research: characteristics of volunteers for patient-led projects on PatientsLikeMe](#)

**Themes:** Qualitative

**Collaborator:** Robert Wood Johnson Foundation

The research community increasingly recognizes the importance of involving patients throughout the research process. In this study, we examined obstacles and barriers toward patients' research participation through a qualitative analysis of applications submitted for patient-led projects through the PatientsLikeMe platform. In general, we found patients want to participate in research to facilitate patient-provider communication, improve understanding of medical information, understand the cause and nature of disease, and bring a more individualistic approach to healthcare.

Bradley M, Braverman J, Harrington M, Wicks P. Patients' motivations and interest in research: characteristics of volunteers for patient-led projects on PatientsLikeMe. *Research Involvement and Engagement*. 2016. 2:1.

### [Concept elicitation within patient-powered research networks: A feasibility study in chronic lymphocytic leukaemia](#)

**Themes:** PROs, Oncology

**Collaborator:** HRA, Janssen

Concept elicitation traditionally requires in-depth interviews lead by trained interviewers, whether individually or as part of a focus group. Online patient networks may provide an alternative means for patients to share their experience of living with disease such as impact of symptoms, in a manner that is more scalable, rapid, and convenient. In this mixed-methods study, 50 PatientsLikeMe members with CLL were invited to complete a survey with a subset taking part in follow-up interviews. The concepts elicited were similar to those gathered through more traditional means.

McCarrier K, Bull S, Fleming S, Simacek K, Wicks P, Cella D et al. Concept Elicitation Within Patient-Powered Research Networks: A Feasibility Study in Chronic Lymphocytic Leukemia. *Value in Health*. 2016.19:1;42-52.

# 2015

## [How Common are ALS Plateaus and Reversals?](#)

**Theme:** ALS

**Collaborator:** Duke, MGH

Although ALS is considered an untreatable and relentlessly progressive disorder, anecdotal evidence suggests that some patients stabilize or even regain function, albeit temporarily, during the course of their disease journey. Using data from the PRO-ACT database our team identified a small subset of patients (7–25%) that did not decline over periods ranging from 6 to 18 months. 14% of patients had a six month window in which their rate of decline was negative, although fewer than 1% had a sustained response lasting at least 12 months. Small temporary plateaus and reversals are more common than we thought, and should not necessarily be interpreted as an ALS treatment effect.

Bedlack R, Vaughan T, Wicks P, Heywood J, Sinani E, Selsov R et al. How common are ALS plateaus and reversals? *Neurology*. 2015;86(9):808–812.

## [Acquisition, Analysis, and Sharing of Data in 2015 and Beyond: A Survey of the Landscape](#)

**Theme:** Cardiovascular Disease

**Collaborator:** American Heart Association

Where once patients were just the passive source of data they are increasingly becoming active generators, interpreters, and owners of data from sources as diverse as genetic data, medical records, wearable sensors, and even clinical trials. This report describes a 1.5-day stakeholder meeting convened to determine what patients living with cardiovascular disease want for themselves and to explore what role the AHA might play. A great many cardiovascular events such as stroke and heart attack are attributable (at least in part) to daily behaviors and modifiable risk factors that can increasingly be tracked, quantified, shared, and perhaps even built into clinical management pathways.

Antman E, Benjamin E, Harrington R, Houser S, Peterson E, Bauman M et al. Acquisition, analysis, and sharing of data in 2015 and beyond: a survey of the landscape. *J Am Heart Assoc*. 2015;4(11):e002810.

## [“Trust but Verify” – Five Approaches to Ensure Safe Medical Apps](#)

**Theme:** Smartphone Apps

**Collaborator:** N/A

While smartphones have become ubiquitous in the modern world and we use their applications to message, play games, order taxis, and plan our journeys, the safety and efficacy of apps in medical contexts has remained untested. Following a set of studies that showed that insulin dose calculators risk systematically overdosing patients with diabetes, that asthma apps show poor inhaler techniques, and that even the supposedly validated NHS App Store had widespread failings, we proposed five different approaches that could be used to ensure safety for patients while delivering the potential benefits that apps offer.

Wicks P, Chiauuzi E. ‘Trust but verify’ – five approaches to ensure safe medical apps. BMC Medicine. 2015;13(1).

## [New Approach for Analyzing Self-Reporting of Insomnia Symptoms Reveals a High Rate of Comorbid Insomnia Across a Wide Spectrum of Chronic Diseases](#)

**Theme:** Insomnia

**Collaborator:** Northwestern, Merck

Sleep disturbances are increasingly being recognized as an important comorbidity to chronic health conditions. In this study over 5,000 patients with a range of chronic health conditions were surveyed on their National Sleep Foundation-defined risk of early, middle, and late insomnia. Although the majority of patients with conditions such as epilepsy, depressive disorders, and fibromyalgia reported very high rates of insomnia, few had been formally diagnosed by a physician suggesting they might be under-treated and require better management.

Katic B, Heywood J, Turek F, Chiauuzi E, Vaughan T, Simacek K et al. New approach for analyzing self-reporting of insomnia symptoms reveals a high rate of comorbid insomnia across a wide spectrum of chronic diseases. Sleep Medicine. 2015;16(11):1332-1341.

## [Partnering with Patients Using Social Media to Develop a Hypertension Management Instrument](#)

**Theme:** PROs

**Collaborator:** Villanova University

The aim of this study was to develop a patient-reported hypertension instrument that measured attitudes, lifestyle behaviors, adherence, and barriers to hypertension management using patient-reported outcome data. The Open Research Exchange PRO platform was used to rapidly gather feedback during item development from 21 patients and psychometric data was collected from 360 patients in just 7 days. The Kear Hypertension Management Instrument is freely available and licensed under Creative Commons.



Kear T, Harrington M, Bhattacharya A. Partnering with patients using social media to develop a hypertension management instrument. Journal of the American Society of Hypertension. 2015;9(9):725-734.

### [Patients Optimizing Epilepsy Management \(POEM\) – An online social network improves self-management and self-efficacy in Veterans](#)

**Theme:** Epilepsy

**Collaborator:** VA, UCSF, UCB

Veterans living with seizures are often isolated and struggle with self-management. In a six-week trial of PatientsLikeMe as an intervention, a collaboration with the VA Epilepsy Centers of Excellence found a significant improvement on patient self-management and self-efficacy using validated patient-reported outcomes. Internet based psychosocial support may be a scalable approach to improving patient self-management in challenging chronic conditions.

Hixson J, Barnes D, Parko K, Durgin T, Van Bebber S, Graham A et al. Patients optimizing epilepsy management via an online community. Neurology. 2015;85(2):129-136.

### [An Innovative Approach to Informing Research: Gathering Perspectives on Diabetes Care Challenges from an Online Patient Community](#)

**Themes:** Diabetes, Patient-centeredness

**Collaborator:** Kaiser Permanente

The diabetes-focussed SUPREME-DM network (led by Kaiser Permanente) realized they needed patient input on priorities and preferences for an upcoming stakeholder meeting to determine what research projects the group should prioritize. Top patient challenges were lifestyle concerns (diet, physical activity, weight, and stress) and interpersonal concerns (trying not to be a burden to others, getting support from family/friends), and were gathered quickly and efficiently through an online tool.

Schroeder E, Desai J, Schmittiel J, Paolino A, Schneider J, Goodrich G et al. An innovative approach to informing research: gathering perspectives on diabetes care challenges from an online patient community. Interact J Med Res. 2015. 4:2:e13.

## Patient-Centered Activity Monitoring in the Self-Management of Chronic Health Conditions

**Theme:** Wearable Sensors

**Collaborator:** N/A

The past few years have seen the launch of a variety of consumer devices (including smartphones) that allow users to measure their activity, steps, sleep, stress, and other vital signs. People living with chronic health conditions may have different goals, challenges, and constraints in using these devices, but may well have the greatest opportunity to benefit by better managing their conditions. This editorial explores the opportunities and pitfalls that such monitoring may present to patients and other stakeholders.

Chiauzzi E, Rodarte C, DasMahapatra P. Patient-centered activity monitoring in the self-management of chronic health conditions. BMC Medicine. 2015;13:1.

## Research Led by Participants: A new Social Contract for a New Kind of Research

**Theme:** Patient-centeredness

**Collaborator:** University of Zurich

Traditional ethical protections such as research ethics committees (RECs) or institutional review boards (IRBs) assume a power imbalance between participants and researchers; but what happens when participants themselves convene online and self-organize to conduct their own scientific research? In this report from an ethics workshop, the discussants propose that participant-led research should be held to the same standards as traditional research, with approaches for building this infrastructure to protect patients.

Vayena E, Brownsword R, Edwards S, Greshake B, Kahn J, Ladher N et al. Research led by participants: a new social contract for a new kind of research. J Med Ethics. 2015. 42:4:216–219.

## How Engaging Patients Will Change Clinical Trials for the Better

**Theme:** Trials

**Collaborator:** Genentech

Patients are increasingly being consulted by researchers, grant awarding bodies, and regulators like the FDA to give their perspective on the challenges of managing their condition in order to prioritize resources and improve outcomes. This “home study” article advocates for pharmaceutical companies to systematically engage patients and provides guidance on overcoming hurdles, with a case study from our partnership with Genentech.

Wicks P, Gilbert J, Barr C. How engaging patients will change clinical trials for the better. Clinical Researcher. 2015. 29:2:20-25.

## [Preferred Features of Oral Multiple Sclerosis Treatments and Predicted Adherence: An Online Patient Choice Experiment](#)

**Theme:** MS

**Collaborator:** Novartis

People living with Multiple Sclerosis (MS) have more treatment options every year, with newer therapies including oral medications and infusions on top of the traditional injection-based therapies. In this study we used a tradeoff technique, "conjoint analysis", The risk of adverse events was the most salient driver of patient preferences, followed by delay to disability progression and then other side effects. The study also identified a number of predicted drivers of medication non-adherence in MS.

Wicks P, Brandes D, Park J, Liakhovitski D, Koudinova T, Sasane R. Preferred features of oral treatments and predictors of non-adherence: two web-based choice experiments in Multiple Sclerosis patients. Interact J Med Res. 2015. 4:1:e6.

## [Time to deliver patient centered care](#)

**Theme:** Patient-centeredness

**Collaborator:** BMJ

In this lead editorial to the BMJ's Spotlight on Patient Centered Care, the authors argue that we must harness the energy, insight and expertise of patients, carers, and the communities that support them to help drive change.

Richards T, Coulter A, Wicks P. Time to deliver patient centered care. BMJ. 2015. 350:h530-h530.

## [Measuring what matters: The case for patient-generated PROMs](#)

**Theme:** PROs

**Collaborator:** N/A

PROs in widespread use today suffer from a number of limitations that limit their potential use to support research, clinical management, and patient self-knowledge. For instance, many PROs were developed without sufficient patient input. Technology is increasing the potential to more rapidly develop better measures and today patients themselves are becoming able to develop their own PROs to ensure we matter what matters.

Wicks P. Measuring what matters: The case for patient generated PROMS. British Medical Journal. 2015. 350:h54.

## [Increasing patient participation in drug development](#)

**Theme:** Patient-centeredness

**Collaborator:** Novartis

While agencies such as PCORI and the FDA are increasingly incorporating patients as partners and decision-makers in prioritizing and providing input to their work, the pharmaceutical industry lags significantly behind. In this comment the authors call for increased patient participation in earlier phases of drug development to guide innovation.

Wicks P, Lowe M, Gabriel S, Sikirica S, Sasane R, Arcona S. Increasing patient participation in drug development. *Nat Biotechnol.* 2015. 33:2:134-135.

## [Can we use social media to support content validity of PRO instruments in medical product development?](#)

**Theme:** PROs

**Collaborators:** FDA, Janssen, Novartis

A panel of experts lay out opportunities for digital online communities to support the development of PROs and discuss the proof points needed to validate this new approach.

Rothman M, Gnanaskathy A, Wicks P, Papadopoulos E. Can we use social media to support content validity of patient-reported outcome instruments in medical product development? *Value in Health.* 2015.18:1:1-4.

## [Patients report worse MS symptoms after menopause: findings from an online cohort](#)

**Theme:** MS

**Collaborator:** Brigham & Women's

Using a cohort of 513 members of the PatientsLikeMe MS community, a team of researchers identified significant worsening of symptoms associated with menopause, particularly for those women who had undergone surgical menopause or were younger at the onset of menopause. Use of the MS Rating Scale, Revised (MSRS-R) helped to identify MS symptoms such as vision issues and arm function that do not overlap with menopause.

Bove R, Healy B, Secor E, Vaughan T, Katic B, Chitnis T et al. Patients report worse MS symptoms after menopause: findings from an online cohort. *Multiple Sclerosis and Related Disorders.* 2015. 4:1:18-24.

# 2014

## Patients and Health Care Teams Forging Effective Partnerships

**Theme:** Patient-centeredness

**Collaborator:** Institute of Medicine

In this discussion paper from the IOM's Roundtable on Value & Science-Driven Healthcare, the authors explore how patients view their role in team-based care and explain what is needed to foster effective partnership of patients and health-care providers.

Okun S, Schoenbaum S, Andrews D, Chidambaran P, Chollette V, Gruman J, Leal S, Lown BA, Mitchell PH, Parry C, Prins W. Patients and health care teams forging effective partnerships. Washington DC: Institute of Medicine. 2014.

## The ALS Ice Bucket Challenge – Can a splash of water reinvigorate a field?

**Theme:** ALS

**Collaborator:** N/A

The “viral” ALS Ice Bucket Challenge that swept social media in the summer of 2014 brought awareness of ALS to millions, and a new bolus of funding to support ALS research. In this editorial we examine data quantifying the impact on awareness online.

Wicks P. The ALS Ice Bucket Challenge – Can a splash of water reinvigorate a field? Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration. 2014.15:7-8; 479-480.

## Stigma and adherence in epilepsy: Evidence for a link and mediating processes

**Theme:** Epilepsy

**Collaborator:** Columbia University

In a survey of 140 patients with epilepsy, researchers established a link between higher epilepsy-related perceived stigma and lower levels of medication adherence, information, motivation, and behavioral skills. This model could help to develop interventions targeted at improving the outcomes of people living with seizures.

Chesaniuk M, Choi H, Wicks P, Stadler G. Perceived stigma and adherence in epilepsy: Evidence for a link and mediating processes. Epilepsy & Behavior. 2014. 41:227-231.

## [Data donation could power the learning health care system, including special access programs](#)

**Themes:** Patient-centeredness, ALS

**Collaborator:** N/A

In this peer commentary, PatientsLikeMe's Paul Wicks and Jamie Heywood consider the medical potential and ethical pitfalls for "special access programs" and reflect on their experiences in the ALS field – they argue that data donation is a key pre-requisite.

Wicks P, Heywood J. Data donation could power the learning health care system, including special access programs. The American Journal of Bioethics. 2014.14:11:27–29.

## [Adaptation and validation of the Treatment Burden Questionnaire \(TBQ\) in English using an Internet platform](#)

**Themes:** Patient-centeredness, PROs

**Collaborator:** Paris Descartes University

The Open Research Exchange (ORE) is a unique platform that allows researchers to rapidly develop and validate patient reported outcome measures (PROs) through concept elicitation, psychometric validation, reliability testing, test-retest, and analysis of changes over time. In the first output of this Robert Wood Johnson Foundation-funded study, an international team of health researchers was able to translate and validate a new measure of treatment burden in a global sample of over 600 patients in just two months.

Tran V, Harrington M, Montori V, Barnes C, Wicks P, Ravaud P. Adaptation and validation of the Treatment Burden Questionnaire (TBQ) in English using an internet platform. BMC Medicine. 2014. 12:1:109.

## [Patients leading the direction of clinical research](#)

**Theme:** Patient-centeredness

**Collaborator:** N/A

Patients are increasingly becoming engaged in research, whether as citizen scientists, research partners in the development of studies, or as engaged participants in clinical trials. In this transcript and video Q&A, VP of Innovation Paul Wicks outlines the new era of participant-led research including the potential ethical issues, scientific challenges to be overcome, and innovative new platforms such as the Open Research Exchange.

Wicks P. Video Q&A: Patients leading the direction of clinical research – an interview with Paul Wicks. BMC Medicine. 2014.12:1.

## Could digital patient communities be the launch pad for patient-centric trial design?

**Theme:** Trials

**Collaborator:** N/A

The system of medical discovery does not revolve around patients as unique individuals with preferences, needs, and desires. Rather it revolves around scientific scrutiny, the needs of the sponsor, and the desires for regulatory approval. The patient is only a subject. Is it any wonder, then, that some patients have rejected the current medical paradigm and sought to find their own path? This editorial highlights the potential of patient powered research networks to close the gap between trial designers and patients.

Wicks P. Could digital patient communities be the launch pad for patient-centric trial design? Trials. 2014. 15:1:172.

## Patient-Centeredness in the Design of Clinical Trials

**Theme:** Trials

**Collaborator:** University of Maryland

There is growing concern that recruitment and retention of patients in clinical trials is becoming increasingly challenging. Without reliable trials, the development of new treatments and improved outcomes for patient's risks being delayed significantly. We propose that certain innovative trial designs such as pragmatic trials, adaptive trials, and the use of Bayesian statistics in trials might provide more appealing trial designs than the traditional experimental double-blind randomized placebo-controlled trial.

Mullins CD, Vandigo J, Zheng Z, Wicks P. Patient-centeredness in the design of clinical trials. Value in Health. 2014;17:4:471-475.

## Stakeholder Engagement in Patient Centered Research: High Touch or High Tech?

**Theme:** Patient-centeredness

**Collaborator:** N/A

While digital technology becomes widespread and offers exciting opportunities to accelerate research, it is worth remembering that traditional research methods still have a number of benefits. In this Expert Review of Pharmacoeconomics & Outcomes Research, discussants Danielle Lavalley (University of Washington), Paul Wicks (PatientsLikeMe), Rafael Cristancho (University of Washington) and Daniel Mullins (University of Maryland) outline the opportunities and challenges for more traditional "High Touch" research (e.g. focus groups) and modern "High Tech" approaches (e.g. online communities).

Lavalley D, Wicks P, Alfonso Cristancho R, Mullins CD. Stakeholder engagement in patient outcomes research: high-touch or high-tech? Expert Review of Pharmacoeconomics & Outcomes Research. 2014. 14:3:335-344.

## How Digital Technology and Patient Empowerment is Influencing the Future Direction of Clinical Trials

**Theme:** Trials

**Collaborator:** N/A

In this Future Medicine e-book chapter, Paul Wicks (PatientsLikeMe), Craig Lipset (Pfizer), and Tjeerd Van Staa (Clinical Practice Research Datalink) discuss the importance of trends in clinical trials; participant-led research using online communities, “virtual” clinical trials conducted remotely, and point-of-care trials carried out during routine clinical practice.

Wicks P, van Staa T, Lipset C. Advances in collating and using trial data. 1st ed. London: Future Medicine; 2016.

## Subjects No More: What Happens When Trial Participants Realize They Hold the Power?

**Theme:** Trials

**Collaborator:** N/A

In this editorial, the PatientsLikeMe research team explores how online tools have the potential to affect the blinding of randomized controlled clinical trials. As patients have found it easier to connect online, so too have they started organizing online; they’ve begun sharing their data, and even attempting to run their own analyses as to whether the trials they are enrolled in might yield benefits before the study has concluded. To address this, our team argues that a new social contract must be drawn up that respects the autonomy of patients as individuals while also maintaining scientific rigour, a challenge that must be faced before trials are irrevocably harmed.

Wicks P, Vaughan T, Heywood J. Subjects no more: what happens when trial participants realize they hold the power? BMJ. 2014. 348:g368–g368.

## Social Networking Sites and the Continuously Learning Health System

**Theme:** Patient-centeredness

**Collaborator:** Institute of Medicine

There have been rapid increases in the use of online social networking sites for sharing health experiences like disease diagnosis, treatments, or methods for coping with illness. This discussion paper by the Institute of Medicine cites the results of our PatientsLikeMe survey that revealed 92% of users agreed with sharing their health data with researchers and as many as 78% would let drug companies see the same information. Around three quarters believed their health data could be used without their knowledge, or to deny them benefits or job opportunities, but they continued to share their data anyway.

Grajales F, Clifford D, Loupos P, Okun S, Quattrone S, Simon M, Wicks P, Henderson D. Social networking sites and the continuously learning health system: a survey. Institute of Medicine Discussion Paper, National Academy of Sciences; 2014.



## [Feasibility of a Web-Based Survey of Hallucinations and Assessment of Visual Function in Patients with Parkinson's Disease](#)

**Theme:** Parkinson's Disease

**Collaborator:** Harvard Medical School

Assessing vision is an important task of care, but trained ophthalmologists are in short supply. Partnering with the Mass Eye and Ear Infirmary we sought to assess the feasibility of online vision testing using online methods to identify problems with visual contrast sensitivity and hallucinations, comparing people with Parkinson's to disease controls. Online vision testing is feasible and could be cost-effective for screening.

Jackson M, Bex P, Ellison J, Wicks P, Wallis J. Feasibility of a web-based survey of hallucinations and assessment of visual function in patients with Parkinson's Disease. *Interact J Med Res*. 2014. 3:1:e1.

## 2013

### [Innovations in e-health](#)

**Theme:** Patient-centeredness

**Collaborator:** ISOQOL

Social networks form just one part of the new wave of innovations in E-health. In this editorial from a panel at the International Society of Quality of Life Researchers (ISOQOL), a person living with Parkinson's disease, a pediatric oncologist, a researcher, and a physiotherapist share their perspectives on the potential for these new technologies to improve patient outcomes.

Wicks P, Stamford J, Grootenhuis M, Haverman L, Ahmed S. Innovations in e-health. *Qual Life Res*. 2013. 23:1:195-203.

### [Quality of Life in Organ Transplant Recipients Participating in an Online Transplant Community](#)

**Theme:** Organ Transplant

**Collaborator:** Novartis

Online communities represent one mechanism to continue engaging organ transplant recipients in research after they have left the hospital. In this study, we explored how members of PatientsLikeMe's transplant community differ from the broader U.S population and the challenges they face to maximize their quality of life.

Wicks P, Sulham K, Gnanasakthy A. Quality of life in organ transplant recipients participating in an online transplant community. *The Patient – Patient-Centered Outcomes Research*. 2013. 7:1:73–84.

## **The Virtuous Cycle of the Quantified Self: A Human Computational Approach to Improved Health Outcomes**

**Theme:** Patient-centeredness

**Collaborator:** Max Little

The past 30 years have seen the introduction of a new form of distributed problem solving – patients helping one another using digital technologies. In this book chapter from the *Handbook of Human Computation*, TED Fellows Paul Wicks and Max Little recount the history of the e-patient movement (electronic, engaged, and empowered) and discuss the possibilities for crowd-sourcing medical data through devices and social networks.

Wicks P, Little M. The Virtuous Circle of the Quantified Self: A human computational approach to improved health outcomes. *Handbook of Human Computation*. 2013. 105–129.

## **Virtual Visits for Parkinson’s Disease**

**Theme:** Parkinson’s disease

**Collaborator:** Johns Hopkins

Parkinson’s patients benefit immensely from seeing a movement disorder specialist. However, these doctors are few and far between, and Parkinson’s symptoms make it difficult for patients to travel. Virtual visits that utilize webcams may help bridge the gap and in this study, 55 patients were offered this type of hour-long appointment. The high degree of satisfaction suggests these telemedicine consultations could effectively supplement real-world clinic visits.

Venkataraman V, Donohue S, Biglan K, Wicks P, Dorsey E. Virtual visits for Parkinson disease: a case series. *Neurology: Clinical Practice*. 2013. 4:2:146–152.

## **Patient-Reported Outcome Measures in Safety Event Reporting: PROSPER Consortium Guidance**

**Theme:** Patient-centeredness

**Collaborator:** PROSPER Consortium

The PROSPER consortium comprises industry, regulatory authority, academic, private sector and patient representatives who are interested in the area of patient-reported outcomes of adverse events (PRO-AEs). While current safety reporting and risk assessment processes remain heavily dependent on healthcare professionals, these guidelines lay out a path to wider acceptance of patient reported safety reporting.

Banerjee A, Okun S, Edwards I, Wicks P, Smith M, Mayall S et al. Patient-reported outcome measures in safety event reporting: PROSPER Consortium Guidance. *Drug Saf.* 2013. 36:12:1129-1149.

## [Making the Case for Continuous Learning from Routinely Collected Data](#)

**Theme:** Patient-centeredness

**Collaborator:** Institute of Medicine

The IOM believes in a “learning health system,” but that system cannot rely solely on sporadic and expensive randomized clinical trials or academic studies. The data that is collected continuously as part of routine care, whether through electronic medical records or shared by patients themselves, must be harnessed to forge the new system.

Okun S, McGraw D, Stang P, Larson E, Goldmann D, Kupersmith J, Filart R, Robertson RM, Grossman C, Murray M. Making the case for continuous learning from routinely collected data, Institute of Medicine Discussion Paper, National Academy of Sciences; 2013.

## [Evaluation of an Online Platform for Multiple Sclerosis Research: Patient Description, Validation of Severity Scale, and Exploration of BMI Effects on Disease Progression](#)

**Theme:** Multiple sclerosis

**Collaborator:** Brigham & Women’s

PatientsLikeMe represents a new type of real-world evidence, and that requires constant comparison and validation against existing data sources. In partnership with the Partners Multiple Sclerosis Center at Brigham & Women’s Hospital we found that members of PatientsLikeMe were slightly younger and more likely to be female, but that the differences were very small. We also validated the MSRS-R as a patient-reported outcome against a neurological examination and observational testing.

Bove R, Secor E, Healy B, Musallam A, Vaughan T, Glanz B et al. Evaluation of an online platform for Multiple Sclerosis research: patient description, validation of Severity Scale, and exploration of BMI effects on disease course. *PLoS ONE.* 2013. 8:3:e59707.

## [Blog: The Patient Engagement Pill – Lessons from Epilepsy](#)

**Theme:** Epilepsy

**Collaborator:** UCSF School of Medicine

As part of a special issue on “patient engagement” funded by PCORI and the Robert Wood Johnson Foundation, PatientsLikeMe was invited to write a blog post for Health Affairs, often described as the “Bible” of health policy. In this piece we ask: If patient engagement was a pill, what would it look like? Who would make it? How would it be distributed? Patients themselves may be the secret ingredient.

Wicks P., Hixson J. The Patient Engagement Pill: Lessons from Epilepsy. Health Affairs Blog. 2013.

## [Quantifying Short-Term Dynamics of Parkinson’s Disease Use Self-Reported Symptom Data from an Internet Social Network](#)

**Theme:** Parkinson’s disease

**Collaborator:** MIT

Could frequent patient-reported data tell us more about a disease than clinical trial data? We collaborated with Oxford/MIT mathematician and TED Fellow Dr. Max Little to assess the shape and variability of Parkinson’s disease on PatientsLikeMe compared to publicly available clinical trial data-set, DATATOP. Using the validated, self-reported UPDRS-III Sections 1 & 2, we found that even random fluctuations experienced by patients week-to-week may be as large as the “clinically meaningful” differences identified in trials.

Little M, Wicks P, Vaughan T, Pentland A. Quantifying short-term dynamics of Parkinson’s disease using self-reported symptom data from an internet social network. J Med Internet Res. 2013;15:1:e20.

# 2012

## [Chapter: Web-based Resources](#)

**Theme:** ALS

**Collaborator:** ALS Research Group

Given our experience in helping patients with ALS to successfully harness the Internet to improve their care, we were commissioned by the ALS Research Group to write a chapter in the latest handbook for doctors treating these patients.

Wicks P, Heywood J. Web-based resources. In: Bedlack R, Mitsumoto H, editors. In: Amyotrophic Lateral Sclerosis: A Patient Care Guide for Clinicians. New York: Demos Medical Publishing, LLC; 2012. p. 231-246.

## Chapter: The Evolving E-Patient

**Theme:** Patient-centeredness

**Collaborator:** N/A

Two of our leading nurses, Sally Okun and Christine Caligtan, were invited to contribute to a core textbook on health informatics and provide their unique insights on the patient perspective.

Okun S, Caligtan C. The evolving ePatient. In: Nelson R, Staggers N, ed. by. Health Informatics: An interprofessional approach. Missouri: Elsevier Mosby; 2012. p. 212-224

## Patient Assessment of Physician Performance of Epilepsy Quality-of-Care Measures

**Theme:** Epilepsy

**Collaborator:** AAN

“Quality Measures” are guidelines proposed by specialist institutions such as the American Academy of Neurology (AAN) to the National Quality Forum (NQF) in diseases like epilepsy. In this study we partnered with the chair of the AAN’s Epilepsy quality measures group to survey patients for their views of whether quality measures were being performed correctly. We found different rates of quality between physician specialties and made recommendations for future training of neurologists.

Wicks P, Fountain N. Patient assessment of physician performance of epilepsy quality-of-care measures. Neurology: Clinical Practice. 2012. 2:4:335-342.

## The Multiple Sclerosis Rating Scale, Revised (MSRS-R): Development, Refinement, and Psychometric Validation Using an Online Community

**Themes:** Multiple sclerosis, PROs

**Collaborator:** N/A

PatientsLikeMe offers researchers novel ways of developing, validating, and refining patient reported outcome measures or PROs. In this paper, we report on our work to develop a new scale that helps patients with multiple sclerosis (MS) measure their disability over time. The concise, simple instrument was released under a Creative Commons license, so it can be used or altered by anyone.

Wicks P, Vaughan T, Massagli M. The multiple sclerosis rating scale, revised (MSRS-R): Development, refinement, and psychometric validation using an online community. Health and Quality of Life Outcomes. 2012.10:1:70.

## [Communicating with Patients on Healthcare Evidence](#)

**Theme:** Patient-centeredness

**Collaborator:** Institute of Medicine

The IOM states that “by the year 2020, 90 percent of clinical decisions will be supported by accurate, timely, and up-to-date clinical information, and will reflect the best available evidence.” In this white paper, the IOM outlines data and best practices for communicating evidence to patients facing decisions.

Alston C, Paget L, Halvorson G, Novelli B, Guest J, McCabe P et al. Communicating with patients on health care evidence, Institute of Medicine Discussion Paper, National Academy of Sciences; 2012.

## [Perceived Benefits of Sharing Health Data Between People with Epilepsy on an Online Platform](#)

**Theme:** Epilepsy

**Collaborator:** UCB

In this survey of our users, our epilepsy community (sponsored by UCB) demonstrated significant perceived benefits (improved seizure knowledge, medication adherence, reduced ER visits), but also underscored significant need; prior to joining the site, a third of epilepsy patients had never met another epilepsy patient.

Wicks P, Keininger D, Massagli M, de la Loge C, Brownstein C, Isojärvi J et al. Perceived benefits of sharing health data between people with epilepsy on an online platform. *Epilepsy & Behavior*. 2012. 23:1:16–23.

## [Online Assessment of ALS Functional Rating Scale Compares Well to In-Clinic Evaluation: A Prospective Trial](#)

**Theme:** ALS

**Collaborator:** Charité University Hospital

How does patient reported data compare to the clinician’s view? In this collaboration with the Charité University Hospital in Berlin, we compared patients’ self-reported ALS function scores that were collected over the Internet to the scores generated by the associated clinical teams; they correlated  $r=0.96$ .

Maier A, Holm T, Wicks P, Steinfurth L, Linke P, Münch C et al. Online assessment of ALS functional rating scale compares well to in-clinic evaluation: a prospective trial. *Amyotrophic Lateral Sclerosis*. 2012.13:2:210–216.

## Core Principles & Values of Effective Team-Based Health Care

**Theme:** Patient-centeredness

**Collaborator:** Institute of Medicine

As we shift from acute infections and late-detected malignancies to chronic and long-term illnesses, the need for multi-disciplinary and team-based care becomes ever greater. In this discussion paper from the IOM, participants discussed the optimization of team-based care.

Mitchell P, Wynia M, Golden R, McNellis B, Okun S, Webb CE et al. Core principles & values of effective team-based health care, Institute of Medicine Discussion Paper, National Academy of Sciences; 2012.

## Reassessing Received Wisdom in ALS – Pain is Common When Studied Systematically

**Theme:** ALS

**Collaborator:** N/A

After a population registry identified a high rate of pain in a representative sample of patients with ALS, R&D Director Paul Wicks was invited to write an editorial about how listening to patients can help researchers and clinicians overcome and update some of the widely held misconceptions about ALS (e.g., pain, cognitive dysfunction, or bedsores are uncommon).

Wicks P. Reassessing received wisdom in ALS – pain is common when studied systematically. European Journal of Neurology. 2012;19:4:531-532.

## Information Wants to be Free, But When it Comes to Clinical Trials, Can We Afford to Let it Be?

**Theme:** Trials

**Collaborator:** N/A

An editorial by our R&D Director considers the impact of patient-centeredness and the availability of data as it relates to clinical trials. While patients sharing their data can accelerate research, we may also need to re-think how we “blind” patients in traditional randomized control trials.

Wicks P. Information wants to be free, but when it comes to clinical trials can we afford to let it be? Clinical Investigation. 2012. 2:2:125-127.

## [Mining Online Social Network Data for Biomedical Research: A Comparison of Clinicians' and Patients' Perceptions About Amyotrophic Lateral Sclerosis Treatments](#)

**Theme:** ALS      **Collaborator:** University of Utah

How do patients and doctors differ in their perceptions of systematic treatments in ALS? In collaboration with the University of Utah, we compared the passively entered symptom and treatment data on PatientsLikeMe to an earlier study from the published literature. We found that doctors and patients broadly agree but that patients suggested a number of new treatment options being used.

Nakamura C, Bromberg M, Bhargava S, Wicks P, Zeng-Treitler Q. Mining online social network data for biomedical research: a comparison of clinicians' and patients' perceptions about Amyotrophic Lateral Sclerosis treatments. J Med Internet Res. 2012;14:3:e90.

## 2011

## [Accelerated Clinical Discovery using Self-Reported Patient Data Collected Online and a Patient-Matching Algorithm](#)

**Themes:** ALS, Trials      **Collaborator:** N/A

Following the publication of a small Italian study that provocatively suggested the drug lithium could slow ALS, hundreds of patients started taking the drug. Using an innovative data collection system and matching algorithm, we refuted this study with a sample ten times larger than the original trial.

Wicks P, Vaughan T, Massagli M, Heywood J. Accelerated clinical discovery using self-reported patient data collected online and a patient-matching algorithm. Nat Biotechnol. 2011. 29:5:411-414.

## [Use of an Online Community to Develop Patient-Reported Outcome Instruments: The Multiple Sclerosis Treatment Adherence Questionnaire \(MS-TAQ\)](#)

**Themes:** Multiple sclerosis, PROs      **Collaborator:** Novartis

Since the launch of our multiple sclerosis (MS) community in 2007, our users have been sharing their frustrations with using the current range of needle-based disease modifying treatments available to slow their progression. Needle-based treatments are burdensome and may have an impact on adherence; in this study, we used our online community to construct a novel rating scale of barriers to adherence that was a more accurate predictor of missed doses than clinical or demographic variables.



Wicks P, Massagli M, Kulkarni A, Dastani H. Use of an online community to develop patient-reported outcome instruments: the Multiple Sclerosis Treatment Adherence Questionnaire (MS-TAQ). J Med Internet Res. 2011. 13:1:e12.

## Patient-Reported Outcomes as a Source of Evidence in Off-Label Prescribing: Analysis of Data from PatientsLikeMe

**Theme:** Patient-centeredness

**Collaborator:** N/A

PatientsLikeMe members report taking treatments for a variety of indications, some of which have been approved by the FDA, others that haven't and are considered "off-label." In this study (winner of the Medicine 2.0 inaugural award), we analyzed the pattern of off-label usage for two drugs; amitriptyline and modafinil.

Frost J, Okun S, Vaughan T, Heywood J, Wicks P. Patient-reported outcomes as a source of evidence in off-label prescribing: analysis of data from PatientsLikeMe. J Med Internet Res. 2011. 13:1:e6.

## Concordance Between Site of Onset and Limb Dominance in Amyotrophic Lateral Sclerosis

**Theme:** ALS

**Collaborator:** Oxford University

In collaboration with Oxford University, we found a statistically significant association between site of arm-onset symptoms in ALS and handedness. This was not found in the leg-onset patient group, which lends evidence to the theory that ALS is related to lifetime exertion of affected limbs or neurological pathways.

Turner M, Wicks P, Brownstein C, Massagli M, Toronjo M, Talbot K et al. Concordance between site of onset & limb dominance in amyotrophic lateral sclerosis. Journal of Neurology, Neurosurgery & Psychiatry. 2011. 82:8:853-854.

# 2010

## Potential for Electronic Health Records and Online Social Networking to Redefine Medical Research

**Theme:** Patient-centeredness

**Collaborator:** N/A

There is a broader movement of electronic health records and online tools for the purpose of information sharing and participation in research. This paper by PatientsLikeMe geneticist Dr. Catherine Brownstein and colleagues maps out opportunities like pharmacovigilance, comparative effectiveness, research, and what some of the potential challenges will be for electronic and observational studies.

Pearson J, Brownstein C, Brownstein J. Potential for electronic health records and online social networking to redefine medical research. *Clinical Chemistry*. 2010. 57:2:196–204.

## [Sharing Health Data for Better Outcomes on PatientsLikeMe](#)

**Theme:** Patient-centeredness

**Collaborator:** N/A

After collecting anecdotal evidence that the site was having a positive effect on our members over time, the PatientsLikeMe “user survey” found that patient members reported a number of perceived benefits, including greater health literacy, improved quality of life, and greater social support as a result of using the site.

Wicks P, Massagli M, Frost J, Brownstein C, Okun S, Vaughan T et al. Sharing health data for better outcomes on PatientsLikeMe. *J Med Internet Res*. 2010.12:2:e19.

## [Modifiable Barriers to Enrollment in American ALS Research Studies](#)

**Themes:** ALS, Trials

**Collaborator:** ALS Research Group

As part of an effort to enroll more ALS patients in a study carried out in collaboration with MGH and the Northeast ALS Consortium, we attempted to understand why some patients chose not to participate. We found that ALS patients frequently misunderstood the research process (e.g., believing that a blood sample given for a routine lab test would automatically be reused for genetic discovery).

Bedlack R, Wicks P, Heywood J, Kasarskis E. Modifiable barriers to enrollment in American ALS research studies. *Amyotrophic Lateral Sclerosis*. 2010. 11:6:502–507.

## [The Potential Research Impact of Patient Reported Outcomes on Osteogenesis Imperfecta](#)

**Theme:** Osteogenesis imperfecta

**Collaborator:** N/A

We have been invited on several occasions to design what our “vertical communities” might look like in different applications. This paper describes what a community for patients with osteogenesis imperfecta might look like.

Brownstein C, Wicks P. The potential research impact of patient reported outcomes on Osteogenesis Imperfecta. Clinical Orthopaedics and Related Research®. 2010. 468:10:2581–2585.

## [The PatientsLikeMe Multiple Sclerosis Community: Using Online Marketing to Shift the Health Data Privacy Paradigm](#)

**Theme:** Multiple sclerosis

**Collaborator:** N/A

Designing a clinically relevant and scientifically valid community is important, but if we can't get any patients to visit it then it can't do much good! In this paper, Chief Marketing Officer David Williams explains how we executed the marketing launch of our MS community through segmentation, PR, and targeted recruitment.

Williams III D. The PatientsLikeMe® Multiple Sclerosis Community: Using online marketing to shift the health data privacy paradigm. Journal of Communication in Healthcare. 2010. 3:1:48–61.

# 2009

## [Measuring Function in Advanced ALS: Validation of ALSFRS–EX Extension Items](#)

**Themes:** ALS, PROs

**Collaborator:** ALS patient

One of our patients, Cathy Wolf, has had ALS for more than 10 years and has been a psychologist engaged in research for 30+ years. She expressed dissatisfaction in our forum that the ALSFRS–R, the “gold standard” measure of ALS function, was insensitive at lower levels. Patients like her, or Stephen Hawking, could be rated a zero in terms of function, despite the fact they could still work, use the computer, and manage their affairs. With her help as a co-investigator, we developed an extension to the ALSFRS–R that is in use today in clinical research.

Wicks P, Massagli M, Wolf C, Heywood J. Measuring function in advanced ALS: validation of ALSFRS–EX extension items. European Journal of Neurology. 2009. 16:3:353–359.

## [The Power of Social Networking in Medicine](#)

**Theme:** Patient-centeredness

**Collaborator:** N/A

Following an article about PatientsLikeMe in Nature Biotechnology, we wrote this piece describing updates to our system and our work on the lithium experiment in ALS.

Brownstein C, Brownstein J, Williams D, Wicks P, Heywood J. The power of social networking in medicine. Nat Biotechnol. 2009. 27:10:888-890.

## [Pathological Gambling Amongst Parkinson's Disease and ALS Patients in an Online Community \(PatientsLikeMe.com\)](#)

**Themes:** ALS, Parkinson's

**Collaborator:** Southern General Hospital

After hearing complaints about compulsive gambling in our Parkinson's disease (PD) community resulting from medication side effects, we decided to compare our PD population to the literature. More of our members experienced compulsive gambling (13%) than had been reported in prior literature (~7%). We also used ALS as a control group so that we could compare two neurological populations, a rarity in clinical research.

Wicks P, MacPhee G. Pathological gambling amongst Parkinson's disease and ALS patients in an online community (PatientsLikeMe.com). Mov Disord. 2009 24:7:1085-1088.

## [PatientsLikeMe the Case For a Data-Centered Patient Community and How ALS Patients Use the Community to Inform Treatment Decisions and Manage Pulmonary Health](#)

**Theme:** ALS

**Collaborator:** N/A

We were asked to consider what a community for patients with chronic obstructive pulmonary disease (COPD) might look like.

Frost J, Massagli M. PatientsLikeMe the case for a data-centered patient community and how ALS patients use the community to inform treatment decisions and manage pulmonary health. Chronic Respiratory Disease. 2009. 6:4:225-229.

## [Sharing Information with PatientsLikeMe](#)

**Theme:** Patient-centeredness

**Collaborator:** N/A

As our patient communities expanded from ALS to other neurological conditions such as MS, Parkinson's disease, and the Parkinson's Plus syndromes, we were invited to share our experiences with the field journal for neuroscience nursing.

Wicks P. Sharing information with patients like me. British Journal of Neuroscience Nursing. 2009. 5:3:132-133.

# 2008

## [How the Social Web Supports Patient Experimentation with a New Therapy: The Demand for Patient-Controlled and Patient-Centered Informatics](#)

**Theme:** ALS

**Collaborator:** N/A

In a paper presented at the American Medical Informatics Association (AMIA) annual meeting, we analyzed the uptake in forum conversations about the controversial drug lithium carbonate and its potential for use in the ALS population, including description of our lithium tool.

Frost J, Massagli M, Wicks P. How the social web supports patient experimentation with a new therapy: the demand for patient-controlled and patient-centered informatics. AMIA Annu Symp Proc. 2008. 217-221.

## [PatientsLikeMe: Consumer Health Vocabulary as a Folksonomy](#)

**Theme:** Patient-centeredness

**Collaborator:** University of Wisconsin

In a second AMIA paper, we discussed the use of PatientsLikeMe as a platform to gather the patients' voice in describing symptoms and conditions. The goal was to compare patient descriptions with existing terminologies such as SNOMED, and potentially integrate them into these widely used databases.

Smith C, Wicks P. PatientsLikeMe: consumer health vocabulary as a folksonomy. AMIA Annu Symp Proc. 2008. 682-686.

## [Social Uses of Personal Health Information Within PatientsLikeMe, an Online Patient Community: What Can Happen When Patients Have Access to One Another's Data](#)

**Theme:** ALS

**Collaborator:** N/A

We analyzed interactions between members of our site and found that patients sharing their health data are willing to frequently engage in medically-focused conversations about their health and decision-making.

Frost J, Massagli M. 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.

## ALS Patients Request More Information About Cognitive Symptoms

**Theme:** ALS

**Collaborator:** N/A

We asked the PatientsLikeMe ALS community to answer novel questions that would not normally attract the research funding or study numbers required to produce compelling answers. This was our first fully-fledged research study published in a peer-review journal, and it answered an important question that had previously driven physicians not to inform their patients about the possibility of cognitive symptoms of their disease. This study confirmed that bias and found conclusively that two-thirds of patients wanted to be told.

Wicks P, Frost J. ALS patients request more information about cognitive symptoms. Eur J Neurol. 2008. 15:5:497-500.

# 2007

## Excessive Yawning is Common in the Bulbar-Onset Form of ALS

**Theme:** ALS

**Collaborator:** N/A

A case report published in Acta Psychiatrica Scandinavica prompted us to perform our own investigation of excessive yawning in our ALS population. We were able to go from idea conception to publication in less than 12 weeks.

Wicks P. Excessive yawning is common in the bulbar-onset form of ALS. Acta Psychiatrica Scandinavica. 2007. 116:1:76-76.