



NATIONAL HEALTH COUNCIL

2017 Chief Medical/Scientific Officers Conference  
*Lessons Learned and Pathways Forward:  
Practical Experiences in Patient Engagement*  
A Portfolio of Case Examples

September 19, 2017

## Introduction

This portfolio provides a collection of case examples of patient engagement in research and care delivery. The cases highlight emerging good practices, include successful methods used, and identify shortcomings and gaps that need to be filled.

The case studies included here were submitted to the National Health Council (NHC) in response to a call for case examples that was announced in the summer of 2017 by the NHC Chief Medical/Scientific Officers (CM/SO) Conference Planning Committee. Four of the case examples in the portfolio were selected for podium presentations at the 2017 National Health Council CM/SO Conference on September 18, 2017.

This portfolio is intended to be a living document and will be updated with new cases as they are identified and through future NHC calls for case examples.

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\*Abstracts are listed in alphabetical order by first organization name.

\*\*Version 1

## Engagement of Advocacy Organizations in an ICER Analysis: the MS Coalition Experience

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**Organization(s):** Accelerated Cure, Institute for Clinical and Economic Review (ICER), Multiple Sclerosis Coalition, and National Multiple Sclerosis Society

### **Description:**

In 2016, the nonprofit Institute for Clinical and Economic Review (ICER) initiated an assessment of the health and economic outcomes of disease-modifying therapies (DMTs) for multiple sclerosis (MS). ICER solicited input from individual advocacy organizations and the MS Coalition, a collaborative, national network of independent MS organizations. The Coalition provided a detailed, collective response to the scoping document, and offered to implement a survey of people with MS to ensure their perspectives were represented in ICER's report. ICER and the Coalition jointly developed the survey, which asked about factors affecting treatment choice and other topics. Coalition members surveyed their constituents and received over 15,000 responses.

The Coalition provided continued input throughout the assessment, including commenting on preliminary findings and a draft report. ICER invited the Coalition and individual member organizations to participate in the public meeting of the California Technology Assessment Forum (CTAF), where a revised report was discussed.

Due to the Coalition's input, ICER's report and meeting included more information on the factors patients consider when choosing a therapy, including the DMT's clinical effectiveness, the ability to work and perform normal daily activities, provider recommendation, long-term risks, insurance access restrictions, and cost. The CTAF incorporated this information into their votes on key questions raised by the report. Following the vote, a policy roundtable of patient advocates, clinicians, drugmakers, and insurers discussed how to apply the evidence and votes to ensure sustainable access to MS treatments.

### **Lessons Learned:**

- This was the first time ICER incorporated a large-scale patient survey into a review. The survey supported ICER's goals of improving patient engagement in its process and reports, based on this experience similar surveys have since been included in other ICER reports.
- While not all patient advocacy organizations have the capacity to deploy a large survey, this joint effort demonstrated that input from patient organizations influences discussions around the value of care.

## Advocate Feedback on a Clinical Trial Questionnaire

**Author(s)/Titles:** Nikki Levy, Vice President, Patient Engagement

**Organization(s):** Alkermes, Inc.

### **Description:**

This abstract offers information about an industry/advocate collaborative process to create a short questionnaire to better understand what symptoms consistent with the Montgomery-Åsberg Depression Rating Scale (MADRS) matter the most to patients with difficult-to-treat depression. Although this survey was added at the end of a related clinical study, it had a separate protocol and consent form. The initial two-question survey was drafted internally at Alkermes, Inc. using standard MADRS descriptors. The questions were reviewed by a national advocacy organization dedicated to improving the lives of people who have mood disorders. The organization provided critical feedback, which led to significant re-wording of the symptom language as well as the addition of a third question about wellness. A key aspect of advocate feedback was that studies on depression typically measure symptoms when both a decrease in symptoms and an improvement in feelings of wellness matter to patients. They suggested comparing and contrasting symptoms, the impact of those symptoms and overall feelings of wellness. Alkermes used a five-item Likert-type scale in patient-friendly language for the third question on wellness. This study is ongoing, and we remain in the data collection stage. Upon receipt of complete results, we will analyze the data and look for information and insights specific to the correlation between symptoms, patients' clinical trial results and connections to wellness.

### **Lessons Learned:**

- Engaging individuals with lived experience and those who represent them in survey design ensures the ultimate survey questions are understandable to the end-user.
- Adding a wellness question provided an opportunity to measure an outcome that was important to patients.

## Multi-Step Advocacy Collaboration: How Patient Feedback Loops Inform Program Development

**Author(s)/Titles:** Nikki Levy, Vice President, Patient Engagement

**Organization(s):** Alkermes, Inc.

### **Description:**

This abstract presents a three-part program in which Alkermes, Inc. and the Depression and Bipolar Support Alliance (DBSA) partnered to better understand the experiences, feelings, and needs of people with difficult-to-treat depression. DBSA is a national patient-led advocacy organization with a mission to provide hope, help, support, and education to improve the lives of people who have mood disorders. Alkermes is a pharmaceutical company focused on discovering and developing potential treatments for mental health and substance use conditions.

*Advisory Board I:* In fall 2015, 11 patients with lived experience with mood disorders and diverse backgrounds attended a one-day panel and were asked questions about their perspectives and needs. The purpose was to learn about individuals' experiences and what is most important to people living with difficult-to-treat depression when seeking treatment. The discussion included the individuals' experience with depression; their experience finding effective treatments; and feelings about mental health research. Feedback informed Step 2, a peer survey, which validated the initial Advisory Board's discussion and gathered additional information.

*Survey:* Based on Step 1 results, a 22-item survey was developed to assess whether the perceptions of the small group with lived experience reflected those of others with difficult-to-treat depression. Individuals were invited to complete the survey through DBSA's newsletter, chapters, and social media pages. The survey was also shared with other mental health organizations for distribution. In February 2016, 896 people living with depression completed the Depression Experiences and Treatment Survey.

*Advisory Board II:* In August 2016 the same initial group came together to participate in a second Advisory Board in which survey findings were reviewed and key learnings were discussed. The individuals learned how their feedback informed the survey design, heard survey results, and had a chance to provide input and feedback to both Alkermes and DBSA program ideas.

### **Lessons Learned:**

- Patients are motivated to share their experiences (both in live meetings and via surveys) when they know the information they share will be used to benefit the patient community.
- Working with patient organizations to design survey instruments lead to questions that are more meaningful to people with lived experience.

## Understanding Patient Preferences about Schizophrenia Treatment Side Effects

**Author(s)/Titles:** Nikki Levy, Vice President, Patient Engagement

**Organization(s):** Alkermes, Inc.

### **Description:**

This abstract offers information about how Alkermes, Inc., actively solicited and integrated feedback from leading mental health advocates and patients with schizophrenia in designing a questionnaire to understand patient preferences regarding the importance of side effects in treatment decision making (specifically the impact of weight gain) to inform the development of an investigational medicine for schizophrenia.

*Step 1:* Alkermes solicited feedback on the design and language used in an internally-developed questionnaire from leading advocacy groups as well as individuals living with the disease. This feedback was instrumental in developing a patient-centric survey.

*Step 2:* Stakeholder feedback led to three valuable insights that proved instrumental in revising the questionnaire. Insights pertained to accessibility of questions for patients with cognitive issues, symptom descriptors that were too clinical and not easily understood by the lay person, and that multiple question formats are confusing.

*Step 3:* Rather than include the questionnaire in the clinical development program, Alkermes made an important decision to work with a community clinic to reach a more real-world population versus those who self-select for a clinical trial. Feedback led to an effective survey tool, which contributed to improved survey participation and completion. For illustration we have provided advocate and patient quotes.

“Ranking questions are way too complicated for people with schizophrenia in general. I would take each item and write it as a separate question with a different rating scale.”—Advocate

“I looked over the updated questionnaire and I think it’s fine. This one I could do. The first one I would have had frustration over...this is a big improvement...”—Person with schizophrenia

### **Lessons Learned:**

- Engaging individuals with schizophrenia and those who advocate for them in survey design ensures the survey questions are both accessible and understandable to patients with cognitive issues.
- The decision to work with a community clinic allowed us to reach a real-world patient population versus those who self-select for a clinical trial.

## Many Ways of Learning from People Who Have Lupus – Views of Their Disease and Research

**Author(s)/Titles:** Marion Dickson, Clinical Development Director; Kay Warner, Patient Engagement Lead; Greg Powell, Team Leader, Classic and Established Products; and Lorrie Schifano, SERM Senior Director on behalf of GSK

**Organization(s):** GSK

### **Description:**

People, from a variety of countries, who have lupus have been teaching drug developers in many ways about the impact of lupus symptoms on daily life and how to meet their needs. In interviews, women with lupus reported fatigue, painful joints, cold feet and fingers, prolonged mouth ulcers, and feeling depressed, as they could not always go outside or to work. Exposure to sunlight provoked or aggravated skin symptoms. Skin lesions were itchy, thick, and might occur anywhere on their bodies, including their heads or faces, which could isolate them socially. They said that they needed several different treatments at the same time and would prefer a topical treatment rather than an additional oral treatment. They would accept intravenous treatment if it would make them generally better. They wanted a new treatment to prevent or reduce the flares – ideally curing them.

Drug developers used qualitative research to select and develop patient-relevant, sensitive, and disease-specific measures for trials. Specific questionnaires were developed for product studies to measure what matters to people with lupus, in the most precise and accurate way for capturing treatment effects.

People with lupus reported high satisfaction with the treatment, which improved their symptoms as well as their ability to work and conduct daily activities. Drug developers also analyzed lupus patients' public de-identified social media posts as safety data, complementary to traditional sources, looking for real-world use, benefits, and risks. Specific points of interest were lack of effect and waning effects, number of patients taking treatment holidays, outcomes and restart rates, effects on activities of daily living or quality of life, effects on end organ function, and tapering or weaning off other drugs, especially steroids. Among 4,133 eligible posts, all questions of interest received some mention. Product developers gained more insight into patients' views and experiences. Additional product teams have continued to build on this learning for their longer-term pharmacovigilance.

### **Lessons Learned:**

- People living with lupus described a number of physical symptoms, social impacts, and the need for more convenient and effective treatments. This information was helpful in creating more patient-relevant and disease-specific measures for clinical trials.
- Publicly available social media data without personal identifiers provided complementary information around patients' views of their treatments, concerns about their effects, and behaviors with regard to dosing that can help to create more effective and convenient treatments more aligned to patients' priorities.
- Complementary sources of data can be helpful in gaining a more complete picture of a range of patients' needs and experiences.



## Improving Patient Involvement in Multi-State HIV Service Quality Learning Collaboratives

**Author(s)/Titles:** Michael Hager, President

**Organization(s):** Hager Health, LLC

### **Description:**

The Health Resources Services Administration HIV/AIDS Bureau (HAB) occasionally funds HIV quality improvement (QI) learning collaboratives (LC) across multiple states. Over the last 10 years, three LC have been formed to address pressing issues related to HIV patient care. Some of these LCs did not emphasize consumer involvement and others made patient involvement a core criterion for state participation. There were important differences between LCs based on the level of patient involvement.

Pressure applied by patients at LC tables increased the rigor of performance-measurement analyses and generated additional quality interventions to test. Starting in 2008, a five-state collaborative without much patient involvement, focused on a single problem, and involved very few interventions over two years. Metrics for that initiative were based on process and not on patient outcomes. While there was a high level of participation in these states, it was hard to determine what the ultimate impact was for patients.

Starting in 2011, a four-state LC focused on two problems in less than two years and involved an array of interventions, in part informed by patient participation in discussions. Patients were trained in quality improvement (QI) basics, including interpretation of basic health statistics and were encouraged to challenge HIV provider and LC leader assumptions and actions. In this case, patient involvement led to enriched discussion and a wider array of QI interventions, but the project focus still focused on process instead of outcomes.

Starting in 2014, a five-state collaborative focused on patient health outcomes related to the HIV care continuum and involved state-specific QI interventions. Patient-specific training on health literacy and health numeracy went beyond QI basics and pushed patients to be equal partners in the LC. Improvement was seen in each state around HIV outcomes.

### **Lessons Learned:**

- It is essential to meet people where they are in the most genuine sense and not make any assumptions about what they know and what they don't know – never make assumptions about motivations. By front-loading work aimed at assessing consumer participants wants, needs, skills, strengths, and weaknesses, specialized training and supports can be developed to maximize participation and effect.
- Do not put too much pressure on individuals or small groups to be the central voice and representatives of all consumers within a given area. People are diverse like snowflakes, and so are their needs. It is unlikely that any one person or small group will effectively understand all the needs in their area, because they won't know all the people affected in their area. One is better served by creating self-sustaining networks of consumers that come together to share their experience and ideas with light administrative support from a champion organization.
- Never talk down to people or demean their interest or efforts. Even slight affront can disrupt trust and bonding among consumers and between consumer and provider participants in learning collaboratives.

## Measuring Patient and Physician Benefit-Risk Preferences in Antipsychotic Clinical Trials

**Author(s)/Titles:** Eva G Katz<sup>1</sup>, PhD, MPH, Associate Director, Benefit-risk Assessment, Department of Epidemiology; Brett Hauber<sup>2</sup>, PhD, Vice President of Health Preference Assessment; Srihari Gopal<sup>1</sup>, MD, Senior Director and Clinical Leader; Angie Fairchild<sup>3</sup>, Research Economist; Amy Pugh<sup>4</sup>, Medical Student; Rachel Weinstein<sup>1</sup>, PhD, Director, Department of Epidemiology; Bennett Levitan<sup>1</sup>, MD, PhD, Senior Director, Benefit-risk Assessment, Department of Epidemiology;

**Organization(s):**<sup>1</sup>Janssen Research & Development; <sup>2</sup>RTI Health Solutions; <sup>3</sup>Duke Clinical Research Institute; <sup>4</sup>The University of California, San Francisco (UCSF)

### Description:

*Purpose:* Long-acting injectable (LAI) antipsychotics have simpler dosing regimens than oral antipsychotics, but their efficacy and safety profiles may differ. This study measured clinical trial participants' and investigators' judgments of the relative importance of efficacy and safety attributes for antipsychotic treatments for schizophrenia, assessed the impact of formulation and adherence, and determined benefit-risk tradeoffs.

*Methods:* Discrete-choice experiment surveys were completed by patients with schizophrenia and physician investigators participating in phase-3 trials of an LAI antipsychotic. Respondents were asked to choose between hypothetical antipsychotic profiles defined by varying levels of efficacy, safety, and formulation. Data were analyzed using random-parameters logit and probit models. To the best of our knowledge, this is the first collection of preferences within clinical trials.

*Results:* Patients (N=214) and physicians (N=438) regarded improvement in positive symptoms (e.g. delusions, hallucinations) as more important than improvement in any other attribute studied. Both groups significantly preferred 3-month and 1-month LAIs to oral formulations, irrespective of prior patient adherence. Physicians were willing to accept treatments with reduced efficacy to switch patients from an oral to 3-month LAI, with a maximum acceptable reduction in efficacy of 9.8% for adherent patients, 25.4% for patients with a history of missing 20% of doses, and at least 30% for patients missing half their doses. For patients, the maximum acceptable reduction in efficacy was 10.1% for adherent patients, while they would accept any reduction in efficacy over the range studied for nonadherent patients to switch to an LAI.

*Conclusion:* Both patients and physicians regarded positive symptoms as most important and accepted reduced efficacy in exchange for switching a patient from an oral formulation to an LAI, with greater reductions in efficacy acceptable for switching less adherent patients to an LAI. These results enable developing clinical development strategies and defensible benefit-risk assessments that incorporate the patient perspective.

### Lessons Learned:

- While patient preferences are generally assessed in on-line panels, using a clinical trial sample may be important when a patient sample with a confirmed diagnosis is needed, adequate sample size is difficult to obtain elsewhere, or when direct experience with a novel study drug is required.
- Schizophrenia patients and physicians regarded improvement in positive symptoms (e.g. delusions, hallucinations) as more important than improvement in any other benefit or risk studied. Both groups also preferred 3-month and 1-month LAIs to oral formulations, irrespective of prior patient adherence.
- The risk tolerance of subjects participating in a clinical trial may differ from subjects who opt out of the trial.

## **Trial Simulation Workshops to Gather Input from Phase II Investigators, Sites, and Patients in the Netherlands, Spain, and the United States.**

**Author(s)/Titles:** Ben Hartog, Director, R&D Operations, Janssen Research & Development; Elise Felicione, Sr. Director, R&D Operations Innovation, Janssen Research & Development; Abbe Steele, Chief Executive Officer, HealthiVibe

**Organization(s):** Janssen Research & Development and HealthiVibe

### **Description:**

To optimize trial design and implementation of a multinational Phase IIb early Alzheimer's trial, a mock run-through (simulation) of key trial activities was conducted at hospital research centers in the Netherlands, Spain, and the United States. The simulations involved four former Phase IIa participants and their caregivers (Netherlands and Spain), along with eight "research naïve" age-matched individuals and caregivers (all countries), along with trial investigators and research staff.

The mean age of simulation participants was 74 years (range 69-84), and caregivers 66.5 years (range 53-81). Seven of the 12 participants reported having completed at least some university education, versus three of eight persons in the caregiver group (NL and US, SP not disclosed due to privacy legislation).

Factors influencing willingness to participate in the trial include reputation of the investigator and trial site; accessibility of the site; and fear of Alzheimer's disease. US participants voiced concerns about the general length of the trial and lifestyle fit; in Spain, less so, provided they felt their contribution was valued. Continuity of trial staff and empathy was deemed important, as was access to physicians in response to other health concerns. Most former Phase IIa trial participants felt the study had been very well explained; most trial naïve participants reported "information overload." Education, engagement, and support of the caregiver were noted as critical to engagement of the participant.

This simulation project explored the feasibility of conducting trial simulations at research sites and to assess the degree of geographic variability. Results were generally similar across the different participating countries.

### **Lessons Learned:**

- Implementing simulations at academic institutions required additional administrative steps as compared to other settings such as market research facilities, but was deemed justified given the authenticity of the environment.
- The findings will inform implementation of programs around participant education and caregiver engagement.

## Direct Patient Engagement Driving Changes to Target Product Profile and Development Plans

**Author(s)/Titles:** Katherine Capperella, Global Patient Engagement Leader, Janssen Pharmaceuticals

**Organization(s):** Janssen Research & Development

### **Description:**

Janssen engaged directly with patients to understand what they expect from future products, including their most critical unmet needs with the goal of finding better treatments in a target disease area. Because of the direct patient engagement, a development team changed its Target Product Profile (TPP) for a lead compound.

Janssen teams immersed themselves in patient experience feedback sessions and interviews of patients with a target disease. The teams found that because the symptoms of the disease have such a significant impact on quality of life, patients are more likely to have a higher tolerance for less convenient dosing/administration than anticipated if the treatment proved effective in the management of their disease and ability to lead a “normal” life. Patients also expressed their desire to know more quickly if a therapy is working. Based on these patient insights, Janssen tailored its TPP to prioritize medication efficacy and speed to disease remission.

Janssen also assembled a patient advisory board that brought together highly engaged and informed patients who were willing to provide ongoing input on development considerations. When patients were asked to describe their day-to-day experiences, there was a common theme that a critical symptom of the disease needed to be better measured. This prompted Janssen to shift its focus to patient-reported outcomes (PROs) that included measures of these symptoms, as well as exploration of the use of biomarkers and wearables during trials.

### **Lessons Learned:**

- There is no substitute for understanding diseases through the lens of people living with the condition. Engaging directly with patients caused this team to refine what a new medicine should do, and to measure something they were not previously measuring, because of what matters to patients. Directly engaging with patients early in the development of medicines can result in solutions that better meet needs.

## Driving Innovation Through Insights – Janssen’s Approach to Patient Engagement

**Author(s)/Titles:** Katherine Capperella, Global Patient Engagement Leader, Janssen Pharmaceuticals

**Organization(s):** Janssen Research & Development

### **Description:**

Janssen has fundamentally reshaped its relationship with patients – moving from doing things “for” patients to doing things “with” them – via a new operating model: Janssen Patient Engagement (PE).

Janssen established a multidisciplinary Global PE Leadership Team (LT) responsible for developing and driving PE strategy and measuring progress over time. The LT is focused on leading Janssen to systematically incorporate direct patient voice early and throughout the entire product lifecycle, beginning with disease area strategies and including product development, formulation decisions and clinical-trial strategies.

### *Its approach:*

- Cross-functional structure: The LT includes representatives from all areas that are responsible for developing and delivering medicines – from R&D, to supply chain and commercial teams. The shared commitment, support of Janssen leadership, and dedicated resources enable Janssen to incorporate the voice of the patient throughout the product life cycle.
- Updated tools and processes: Janssen has updated its Target Product Profile template (TPP) to include the patient as stakeholder, and the process for making formulation decisions now requires the patient perspective. We have also introduced a new Patient Voice in Clinical Trial Design process, as well as a patient Insights Playbook and Integrated Patient Journey (IPJ) tool. Teams have been trained on these new tools and expectations have been set regarding use.
- Measurement tool to ensure accountability: Janssen created the PE Dashboard to monitor how a select list of compounds is being developed with patient insights in mind. It measures whether teams have incorporated direct patient feedback in disease area strategies, compound development strategies, and clinical trial design.

### **Lessons Learned:**

- Having a cross-functional leadership team in place helps to embed patient engagement behaviors broadly, including R&D, supply chain, etc.
- Senior leader sponsorship enables change and action.
- Measuring patient engagement for a targeted list of compounds in development drives culture and behavior change.

## Community-Engaged Treatment Preferences and Priorities for Duchenne Muscular Dystrophy

**Author(s)/Titles:** John F. P. Bridges, PhD; Norah L. Crossnohere, MHS; Ryan Fischer, BA; Ellen Janssen, PhD; Holly Peay, PhD

**Organization(s):** Johns Hopkins Bloomberg School of Public Health, Parent Project Muscular Dystrophy, and RTI International

### **Description:**

Knowing the priorities and treatment preferences of patients and caregivers is essential to promoting patient-focused drug development. Patient preference information can help in developing treatments, conducting clinical research, and improving decision-making. Continued efforts seek to balance patient engagement with scientifically rigorous and validated preference elicitation methods.

Duchenne muscular dystrophy (DMD) is a rare, genetic neuromuscular disorder for which there is currently no cure. Over the past five years, the advocacy organization Parent Project Muscular Dystrophy (PPMD), the Johns Hopkins' Stated Preference research team, and RTI International have collaborated to engage DMD patients, caregivers, and other stakeholders in the development of novel patient-centered instruments that assess treatment preferences and needs of DMD patients and caregivers in the United States. This sustained collaboration uses dynamic approaches to engage a diverse group of stakeholders in developing study inclusion criteria, designing surveys, and refining attributes for stated-preference tools.

To-date, this collaboration has produced seven DMD preference-elicitation exercises: a best-worst scaling (BWS) case 2 combined with simple conjoint analysis to assess caregiver treatment preferences; a BWS case 1 to assess parental worries; a BWS case 2 and simple conjoint analysis to assess pulmonary outcomes; a discrete choice experiment using a "research as an event" approach to assess treatment preferences among diverse stakeholders, and; a BWS case 1 to elicit treatment priorities. This collaboration also informed PPMD's Draft Guidance for Industry regarding procedures to design and test DMD therapies, which was submitted to the FDA. Working with patients, caregivers, and clinicians in instrument development adds personal insight to improve the content, acceptability, and personal meaningfulness of stated-preference tools and allows researchers to better understand how end-users engage with instruments.

### **Lessons Learned:**

- Engagement with stakeholders is a vital step to develop meaningful tools that help translate patient voice into the quantifiable and scientifically rigorous language often preferred by policy makers.
- Patients have distinct preferences both for their medical treatments and for how their voices are incorporated into the research process. Listening to and honoring these voices improves the quality of research and its regulatory impact.
- Patients are the experts of their own lives and experiences. Openly recognizing this expertise is the first step in producing genuinely patient-centered research.

## Health and Research Ambassador PCOR/CER Training

**Author(s)/Titles:** Dory Kranz, Chief Executive Officer

**Organization(s):** National Alopecia Areata Foundation (NAAF)

### **Description:**

The NAAF empowered an existing community network to roll out training on Patient-Centered Outcomes Research and Comparative Effectiveness Research (PCOR/CER) at the grass roots level. With \$160,000 from PCORI, NAAF engaged three alopecia areata patients who were part of a Health and Research Ambassador (HARA) pilot program to review and customize for our community training materials developed by the University of Maryland and NORD. The training tailored to alopecia areata by people with alopecia areata – with advice from the National Health Council, - was delivered to 17 HARA participants in March. As of May, nine of these volunteers had scheduled trainings in their local area.

One initial trainee summed up the value as follows, “This was very empowering. I think it has been a long time coming. There is more and more research. Now we have the tools and the language to go back and advocate for ourselves to educate and develop treatments for our desired outcome.”

A participant in a grass-roots training wrote afterward, “[The trainer] connected well with the audience, and made the information very relatable. I believe everyone was thankful for the opportunity to become more informed about research and how we can better contribute to it as well.”

The three HARAs on the Curriculum Development Committee will deliver the training to 256 community members at NAAF’s Patient Conference in June of 2017 and NAAF will invite interested individuals to join this HARA PCOR/CER training cadre. And so it grows.

This is equipping our patient community with the skills and knowledge to meaningfully engage in research, including an upcoming Patient-Focused Drug Development meeting with the FDA and developing a patient-reported outcome measure so that treatments developed will affect what matters to patients and industry partners will be able to demonstrate that benefit to the FDA and payers.

### **Lessons Learned:**

- Patients like to be included, to have a chance to share their experience and shape the work together. They share their excitement about being included with their networks and it drew more people into the program.
- Patients engaged in meaningful ways are like magnets for others to become engaged.
- As much as we listen, there is no substitute to having patient input through every step of development and delivery. We were sometimes surprised by what patients wanted or noticed that we would have entirely missed.
- Patients like learning from each other. Our training was so well received and a lot of that has to do with it being not just designed but also delivered by people living with the disease.

## Collecting Patient Perspectives and Experiences to Inform Policy Positions and Advocacy

**Author(s)/Titles:** Bruce Bebo, PhD, Executive Vice President, Research

**Organization(s):** National Multiple Sclerosis Society (NMSS)

### **Description:**

A brief online survey was widely distributed to constituents to understand their perspectives and experiences in accessing multiple sclerosis (MS) medications. Survey questions were adapted from Kaiser Health and Consumer Reports surveys so we could compare the MS population with the general population.

### *Methods*

An electronic survey, modeled after Kaiser Health and Consumer Reports surveys, was sent to 1.3 million people in the NMSS database; 123,484 of which identify as living with MS. The survey queried all recipients about their perceptions of the cost of MS disease modifying therapies and queried people with MS about their experiences with access due to cost.

### *Survey Results*

There were 11,194 respondents, 8,778 of which identified as living with MS. Of all respondents, 79.1% felt the cost of prescription MS treatments is unreasonable; compared to 72% of Kaiser respondents. People with MS reported more difficulty paying for their medication: 38.9%; compared to 24% of the general population. Also, 28.3% of people with MS reported it was somewhat or very easy to afford their medication; compared to 72% of the Kaiser respondents. Thirty percent of people with MS skipped doses or stopped treatment due to cost, and 40.3% were utilizing a patient assistance program. Due to cost, 21% spent less on entertainment and dining out; 16.4% used a credit card more; nearly 15% asked their health care provider for a less expensive version of their medication; and nearly 14% postponed paying other bills.

### *Use of the Results*

Results of the survey informed the work of an Advisory Committee in developing recommendations to improve access to medications. Survey results were exhibited in a poster session at the Consortium of MS Centers annual meeting. The results also continue to inform our advocacy efforts and provide personal stories and experiences to support our policy positions.

### **Lessons Learned:**

- High response rate to the survey indicates that cost and access to medications is a top concern among people living with MS.
- The experience of people with MS added considerable credibility to NMSS policy positions and advocacy.



## Determining and Defining Research Priorities: The PDF Community Choice Research Award

**Author(s)/Titles:** Karlin Schroeder, Beth Vernaleo, Diane Cook, James Beck, Veronica Todaro

**Organization(s):** Parkinson's Disease Foundation

### **Description:**

*Objective:* To understand how engaging people with Parkinson's disease (PD) and care partners in defining research priorities can help address unmet needs and advance research.

*Introduction:* The Parkinson's Foundation is committed to ensuring people living with PD are partners throughout the research process. This partnership begins identifying patient needs and priorities as determined by patients. The Parkinson's Foundation launched the Community Choice Research Award (CCRA) in 2013. The purpose of this award was to ask the patient community their priorities and fund a workshop to address research questions based on these priorities.

*Methods:* Research priorities were determined by questionnaires administered to people with PD and care partners via the Parkinson's Foundation website and in person via electronic tablet. Research areas were reviewed and selected by The Parkinson's Foundation's scientific advisory board based on criteria described above. The impact of patient involvement was determined via a survey administered to researchers and clinicians who attended the meetings to determine opinions on patient engagement.

*Results:* Responses for research priorities were varied but could be categorized into general areas. Parkinson's therapies represented the most common topic area. Based on these results the Parkinson's Foundation funded four workshops covering: gastrointestinal issues, fatigue, cognition, and dystonia. The issues are not well-managed with current therapies, have limited published research regarding etiology or treatment, are particularly disabling aspects of PD that lead to reduced quality of life. Researchers felt patient input enriched the meeting dialog and were willing to including patients in future scientific conversations.

### **Lessons Learned:**

- Engaging people with PD and their care partners in determining research priorities leads to identifying unmet needs that warrant attention by scientists.
- Researchers who collaborate with patients find that it adds value, influences outcomes, and increases the likelihood of patient engagement in future scientific conversations.

## Patients Improving Clinical Trials: Partnership Between Pfizer and Parkinson's Foundation

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**Organization(s):** Parkinson's Disease Foundation and Pfizer

### **Description:**

*Objective:* The partnership between Pfizer and the Parkinson's Disease Foundation (PDF), a division of the Parkinson's Foundation, was designed to: 1. increase understanding of the patient experience; 2. better align clinical trial protocols with this experience; and 3. identify potential patient reported outcome (PRO) tools.

*Methods:* Seven PDF Parkinson's Advocates in Research (PAIR) were selected via convenience sample by PDF staff to participate in a one-day patient panel. PDF Research Advocates provided community insights, opinions, and experiences on: challenges to functioning; activities of daily living; and quality of life; as well as barriers to clinical trial participation. A series of open-ended questions were used, developed by PDF staff in partnership with Pfizer staff. Data were transcribed and content was analyzed using descriptive statistics and thematic analysis.

*Results:* PDF generated a report with eight recommendations to: clarify PD-specific definitions on PRO tools; alter PRO tools to employ a one-week recall period; and promote positive messaging and communications between research sites and people with PD to attract more people into clinical trials. Three of these recommendations directly resulted in modifications to a clinical trial design. Other insights were incorporated indirectly. Pfizer staff viewed this facilitated and curated interaction as an efficient and effective way to increase the voice of the patient in trial design.

### **Lessons Learned:**

- Early conversations are key for setting expectations and for creating nondisclosure agreements and other legal contracts. Industry partners can successfully work with their legal teams to be able to discuss study protocol and get feedback from patients in a way that does not violate pre-approval marketing regulatory prohibitions.
- A successful patient panel requires a diverse set of stakeholders so that all parties work together to develop the conversation.
- A reciprocal relationship, in which information is shared bi-directionally and patients know how their participation made an impact, is a key factor in successful patient engagement in research. This relationship stems from shared purpose, accountability, transparency and ensures continuity in the collaboration.

## A Case Study About Direct Involvement of Patients in a Prospective Study to Measure Patient Expectations

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### **Description:**

*Objectives:* In this case study, we describe how we involved patients' in the design and setup of a prospective observational study using a novel approach to assess the patients' perspectives and expectations on antiepileptic treatments choices.

*Methods:* A Discrete Choice Experiment (DCE) as a novel methodology was introduced in a prospective observational study to explore patient perspectives and expectations on treatment decisions in a real-life setting. The objective was to understand several aspects in the decision-making process from a patient's perspective, e.g. influence of the physician-patient interaction, therapy outcomes, and side effects on patient preferences. For this study the research team partnered with patient ambassadors from the beginning of the project to include the patient's opinion on all aspects of the study. This includes study design, instrument preparation, and discussions with clinical experts. A patient ambassador was included on the study team from the outset of designing the study concept through to final protocol and patient selection. In addition, it is intended to collect patient experiences in the study systematically and discuss the feedback with a dedicated patient panel on a regular basis.

*Conclusions:* Involving patients early in designing this case study had a positive impact on the discussions with the clinical team and with clinical experts. Also, giving patients an opportunity to share their perspectives and listen to their opinions improved the overall value of this patient-led study and its acceptance at ethical committees and with involved physicians. To incorporate patients from design through to final protocol required novel approaches to study development. Some of the unique considerations this study had to account for included patient education, compliance, and legal considerations. This process of incorporating patient perspectives was not only beneficial for the outcome of this study but also will help inform future studies moving forward.

### **Lessons Learned:**

- Patients and pharma working together allows transparency for pharma, reducing past barriers and gaining trust from patients.
- This approach supports recruitment and retention of patients.
- Also in our study we learned how important it is for patients to have their voice heard and be an advocate in their condition, especially during the design discussions with clinical experts.
- We also learned the importance to adapt to the language and terminology preferred by patients, meaning the research is more accessible and understood when recruiting and when patients are participating in the research.