



Dear Friends,

This month we describe in greater detail an important ACP initiative that has been mentioned in several recent issues of the newsletter. [iConquerMS™](#) is our patient-powered research network that enables people with MS to contribute data and samples, intelligence and ideas to influence research on MS topics they care about. Currently numbering almost 3000 people, iConquerMS™ is led by a majority of people with MS and provides a structure and framework for people with MS to participate in research from design to dissemination.

In addition, we shine a light on an upcoming fundraising event being organized by Kemp Jaycox, a long-term Cleveland-based supporter of ACP.

iConquerMS™: Blazing the Trail for Patient Engagement in MS Research

By Katina Leodas and Hollie Schmidt

Accelerated Cure Project (ACP) was founded 15 years ago by an individual who was diagnosed with multiple sclerosis. The creation of the organization was grounded in the belief that people who have the disease can and must play a critical role in directing the course of MS research. From the beginning, our efforts to catalyze collaborative research have included people with the disease as partners, most importantly as participants in our biorepository, from which samples have been distributed to scientists around the globe. In 2014, as understanding of the value of patient involvement in research was growing, we embarked on an initiative that takes this practice several giant steps forward -- way beyond our original concept of patients as biospecimen donors or clinical study subjects.

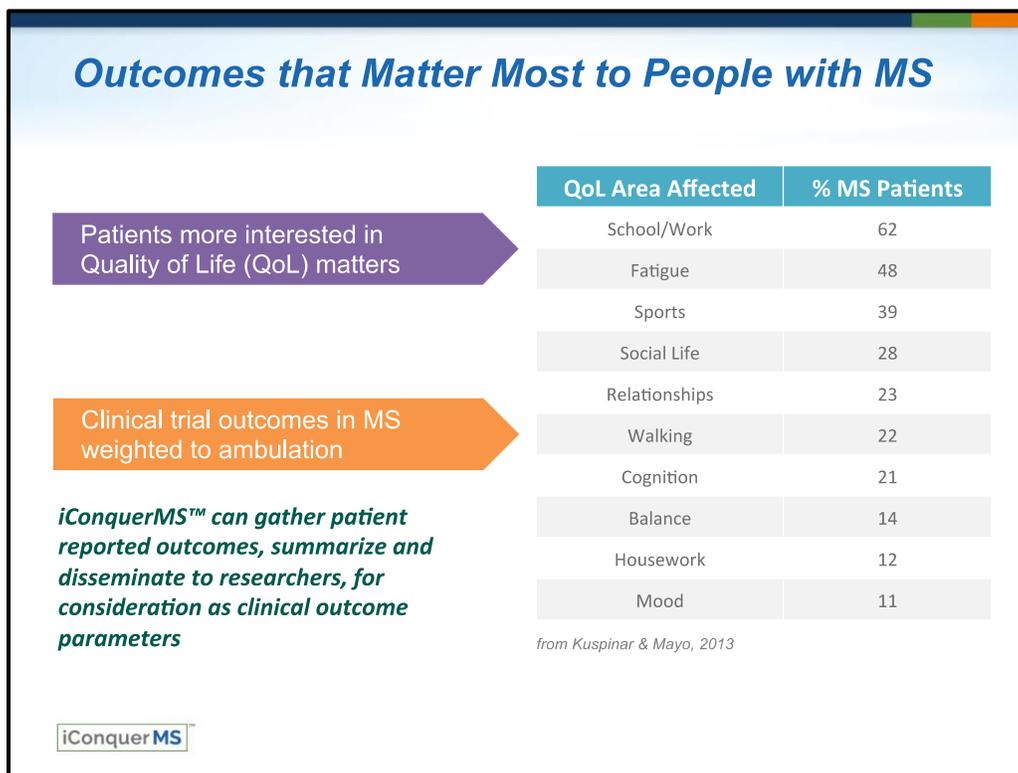


With funding from the Patient-Centered Outcomes Research Institute, known by its acronym PCORI, we began building the country's only patient-powered research network for people with MS. Called iConquerMS™ (www.iConquerMS.org), the network is one of 20 groups funded by PCORI to engage patients with a wide range of diseases and conditions. Other networks similar to iConquerMS™, and funded by PCORI, focus on Alzheimer's Disease, epilepsy, arthritis, heart disease and depression, to name just a few. Together with 13 Clinical Disease Research Networks, we make up something much bigger than any one group working on any one disease. We are PCORnet, a national network of networks that now includes over 100 million Americans. Their electronic health records, health data and

opinions about research into their own diseases have been collected, to support research projects being conducted by organizations within the partner networks as well as from other research institutions and funders.

So what does the term “patient-centered outcomes research” mean and why does it matter? PCORI defines it as “research that helps people and their caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options.” It is research that identifies the best interventions that lead to the best and most relevant outcomes, *as defined by people with the disease*. It elevates the importance of the opinions of patients and puts them on a par with the opinions of researchers and policy makers. In the instance of MS, a disease with which people can live for decades, this just makes good common sense. Who better to ask the important questions, identify the priorities and test theories against real life experience, than people with MS?

In contrast, more traditional, clinical research tracks outcomes that can be easily and/or reliably measured in a lab or clinic, like ability to walk a certain distance or the number of relapses a person with the disease experiences. But while those measures are important, they are not the only ones that matter to people with the disease, as revealed in a survey conducted by two professors from McGill University’s School of Physical and Occupational Therapy and shown on the chart below.¹

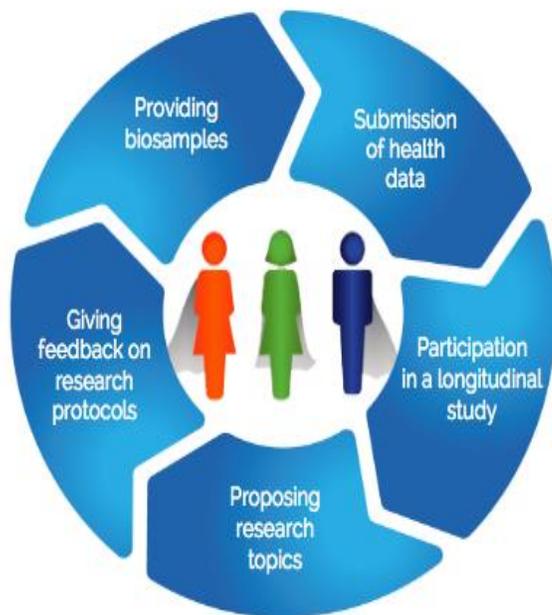


¹ Kuspinar and Mayo: “Do generic utility measures capture what is important to the quality of life of people with multiple sclerosis?” *Health and Quality of Life Outcomes* 2013 11:71.

iConquerMS was established to support patient empowerment in research and enable high-quality patient-centered outcomes research in MS. We're fairly new but we're making great progress. We officially launched in February 2015. Today, 18 months after beginning, our objectives are to:

- **Enable meaningful engagement in research for people with MS:** We are creating opportunities for people with MS to contribute their ideas, experiences, and expertise across the research continuum, from planning and design, to implementation, to results dissemination and clinical impact.
- **Achieve "Research Readiness":** We are developing processes, mechanisms and protocols so that people in the iConquerMS™ community can easily contribute to research studies that are scientifically rigorous and of interest to people with MS.
- **Develop and initiate research studies:** We are working with researchers, funders, iConquerMS™ community members, and other stakeholders to identify topics of interest to people with MS and to develop and launch research studies to investigate those topics.

iConquerMS has just achieved an important milestone: Launching the first study that has gone through



the complete iConquerMS research development and approval process. Researchers at Westat, a national research firm based in Rockville, MD, are studying the impact of adult day programs for people with MS. Adult day programs offer life enhancing services to people with MS who need assistance with activities of daily living. These programs also help family caregivers by providing a break from caregiving duties and/or time to remain in or return to the workforce. The Westat study will evaluate the costs and benefits of these programs and how they impact individuals with MS and their care partners. We are working with them to invite people with MS who don't use an adult day program to serve as "control" participants for the study. This study was reviewed by our community and approved by the iConquerMS Research Committee. An invitation to participate in the study was sent last week to all iConquerMS members. When the study is complete Westat will share its results with iConquerMS.

To date, we have registered almost 3,000 people with MS, about half of whom have completed 4 questionnaires covering their history with MS; demographic data such as age, race and geographic location; and health-related quality of life data such as levels of mobility and sleep patterns, which are relevant to people with MS and useful to researchers. Some participants have voluntarily uploaded their electronic health records. We have also piloted a collection of DNA (derived from saliva) and we have enacted a process for developing, approving and implementing research studies within iConquerMS™, with multiple concrete stages where iConquerMS™ members can participate in making decisions. We have developed a budget model that makes it possible to estimate the costs of iConquerMS™-supported studies, while explaining to researchers the many ways that working through the iConquerMS™ network can enable and enhance their work.

As of June 2016, iConquerMS™ had:

1. Submitted or collaborated on 17 letters of intent and full funding applications for proposed projects involving the iConquerMS™ network. Topics range from on-line mood interventions to telerehabilitation to response to MS disease-modifying therapies.
2. Provided preliminary data and explored study design feasibility in support of funding applications and research proposals through 4 surveys that polled the full iConquerMS™ network. Findings have been shared with collaborating investigators as well as with the iConquerMS™ community. The Westat study, described above, exemplifies this process and the impact it can have on the lives of people with MS and their caregivers.

We are currently designing and deploying REAL MS (Research Engagement About Life with MS), a study that will collect longitudinal data (data repeatedly collected at different points in time) and enable new insights into the natural history of MS over time.

Since the network's earliest days, iConquerMS™ members have been encouraged to provide their research ideas in multiple ways, such as via the iConquerMS portal-based form and in regional face-to-face meetings ("Research Studios") held across the US. Approaches for developing these community-generated ideas into research studies are currently being developed. iConquerMS™ members have also participated in the development and review of studies proposed by researchers by:

1. Volunteering to serve on study review and development panels;
2. Preliminarily reviewing and providing input into research topics and study concepts; and
3. Reviewing and commenting on full study proposals prior to their approval.

iConquerMS™ is an idea whose time has come. Patient empowerment is taking hold elsewhere as well, as regulators and private industry alike are signaling a greater receptivity to meaningful input from people with diseases. The Federal Food and Drug Administration's Center for Drug Evaluation and Research (CDER) launched its Patient-Focused Drug Development (PFDD) program in 2013, with a goal of more systematically gathering patients' perspectives on their condition and available therapies. The agency stated, "FDA believes that drug development and FDA's review process could benefit from a more systematic and expansive approach to obtaining the patient perspective on disease severity and current available options in a therapeutic area."

As part of its commitment, FDA has held close to 20 public meetings around the country to gather patient input, with each meeting focused on a specific disease area (unfortunately, MS was not among the medical conditions chosen). In the meetings that have occurred, the FDA has heard not only from patients, but from their families and care givers too, about the symptoms that matter most to them; the impact the disease has on patients' daily lives; and their experiences with currently available treatments. They have learned that in the case of diseases that are progressive and severely disabling, patients and their families sometimes consider an "ideal" treatment to be one that at minimum can halt disease progression.² In the near future, FDA intends to release "Voice of the Patient" reports that will summarize the input provided by patients and patient representatives at each of these public meetings.

² Comments included in a blog post, "FDA Invites Patient Organizations to Take a Place at the Podium," written by Teresa Mullin, Ph.D., *Director of FDA's Office of Strategic Programs in the Center for Drug Evaluation and Research*, Posted on December 28, 2015 by FDA Voice.

This isn't the first time the FDA has made decisions highly influenced by patient input in analyzing the risks and benefits of a drug. An FDA report published in 2013 documents the impact that patient input had on the Agency's decision to permit remarketing of Tysabri:

"Originally approved in 2004 for relapsing forms of multiple sclerosis (MS), Tysabri was withdrawn from the market by the manufacturer in 2005 after the drug was linked with progressive multifocal leukoencephalopathy (PML), a rare, frequently fatal neurological condition. In reviewing a restricted distribution plan to manage the risk of PML as well as additional efficacy data, in 2006, CDER³ convened an advisory committee meeting on remarketing Tysabri. At the meeting, patients, family members, and health care providers testified to the difference that Tysabri had made in the lives of MS patients, as well as the willingness of patients to continue treatment despite the risk of PML. The FDA ultimately determined that the serious risks associated with this drug were acceptable, because they were outweighed by the benefit of the drug to patients."⁴

Pharmaceutical companies are also recognizing the value of patient involvement in MS research. Several have appointed senior level executives to oversee patient engagement and are emphasizing patient-centricity in their communications both inside and outside the companies. In 2014, Sanofi led the way among the top 10 biopharmaceutical companies when it appointed a Chief Patient Officer, stating that "the appointment will help ensure the patient perspective advances our approach to meeting the unmet needs of patients. . . so [Sanofi's] future healthcare offerings can better incorporate the unique priorities and needs of patients and caregivers in a variety of activities, ranging from early stage R&D through to on-market availability of novel healthcare solutions."⁵

There has never been a better time for people with MS to get involved with MS research. iConquerMS™ is accessible online and gives you a way to shape research and voice your opinions. Regulators and pharmaceutical companies are listening. Why wait? Join [iConquerMS™](#) now to drive and shape MS research.

News from the ACP Repository

This month, we finalized four proposals for research that pertains to MS disease mechanisms. They include one novel MS diagnostic approach, and three investigations into the genetics of MS and transverse myelitis. We also worked with a diagnostic company that had originally requested a very small number of samples for a small pilot study. Finding the samples to be useful and appropriate for their research, they have now requested a much larger quantity for a larger, follow-on study.

³ FDA's Center for Drug Evaluation and Research

⁴ Structured Approach to Benefit-Risk Assessment in Drug Regulatory Decision-Making Draft PDUFA V Implementation Plan - February 2013, Fiscal Years 2013-2017

⁵ Sanofi press release, "Appointment is a First for a Top 10 Biopharmaceutical Company, " PARIS, March 31, 2014 (GLOBE NEWSWIRE).

Pledge Your Support for a Family's Walk in the Woods for MS

Kemp Jaycox and his family (pictured below), who have supported ACP for over a decade, will be vacationing in Alberta's scenic Banff National Park during the third week of July. While there, they plan to conduct their own walk for ACP. Kemp, his wife Cindy Fink and daughter Kate will be joined by Cindy's 81 year-old father, Bob Fink, as well as Kate's godmother, Robyn Clayton. To pledge support for this event, please click [here](#).

Since 2005, Kemp and his family have organized annual events to raise money for ACP, beginning with the highly successful Canine Happy Hour, a gathering that brought together dozens of people who enjoy walking their dogs, while sipping on a glass of wine or a beer. That initial effort raised close to \$5,000 for each of two years.

Around the same time, Kemp and Cindy adopted their daughter and moved from Cincinnati to Cleveland, where Kemp consults on energy efficiency and renewable energy projects. They have continued to organize annual events to support ACP in Cleveland, branching out in recent years to conduct walks in Yellowstone National Park and other beautiful parts of the United States where this nature-loving family elects to vacation. All together over the years, they have raised more than \$25,000 for ACP. We are enormously grateful to Kemp and his family and all their friends!

Have a favorite activity that friends and family members enjoy doing with you? Consider turning it into a fundraiser. Be sure to read next month's ACP newsletter for stories of 4 more friends of ACP who are independently organizing fundraisers that make it possible for us to continue our work to accelerate research towards a cure for MS. Contact Lindsey Santiago at lsantiago@acceleratedcure.org or 781.487.0013 for more information on hosting your own fundraiser for ACP.

