

Accelerated Cure Project for MS

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Accelerating research towards a cure for multiple sclerosis

Will Researchers Listen to Caregivers?

The purpose of medical research is to advance knowledge about and find better ways to treat and prevent disease, thereby improving the public health and quality of life. This is achieved through a step-by-step process in which an investigator defines the topic or question to be addressed, develops a research plan, implements the plan and collects relevant data. Once data collection is complete, the data are analyzed and scientists report their findings. The hope is the original question can be more effectively answered with the knowledge gained. Medical research can take place both in the laboratory and clinical settings. Traditionally, people living with a disease have played a somewhat passive role in this process as the research subject, one who is “tested” and observed. Caregivers have generally played a more peripheral role, for example providing transportation to and support during appointments. Ironically, those who potentially have vital input and are most affected by research results have not historically been central to the process.

People living with MS and their caregivers live the experience of the disease. Their first-hand knowledge of the challenges, symptoms, and effectiveness of treatment is from the front lines. A caregiver’s perspective is unique as they are the closest and most constant observer of the person living with MS. His or her knowledge of side effects, as well as physical or cognitive changes, and how



these affect functioning and quality of life is relevant and vitally important to include at every step of the research process.

As with the person living with MS, a caregiver's intimate point of view can improve a study's design in various ways. Their voice can inform an investigator's approach and help identify knowledge gaps. The caregiver's perspective can help ensure the research plan focuses on priorities that are relevant to individuals living with MS and assess the research plan's feasibility. Caregiver feedback can help investigators develop research tools by pointing out inadequate descriptions or language that isn't easily understood by a layperson. In addition, this information can help reveal any biases or assumptions researchers may have that could influence the research. Because researchers may be removed or isolated from the actual caregiver (or patient) experience, they may make certain assumptions that can lead to faulty interpretations. Those with direct knowledge of these topics may be the only ones able to help correct some assumptions. They may also be the best to explain and expand upon the implications of the research.

As far as study implementation goes, caregiver input may allow investigators to see where there may be difficulties in the process and help them adjust accordingly. The end result of which being the potential to minimize subject dropout rates and improve participant compliance to protocol, thereby improving data quality. Caregivers and people living with a disease, as "end users", are generally the ones applying research findings individually or with healthcare providers. Involving them in the research process will facilitate and possibly accelerate application as the research may have more meaning, may be more relevant, or may be more easily understood.

The caregiver and patient voice are essential for data analysis and interpretation. Those living with the disease or condition under study can help identify themes that researchers might miss. This perspective can also help investigators check the validity and relevance of their conclusions. Many individuals find research daunting because the presentation of data is unclear. With the caregiver and patient perspective in mind, assessing publications can result in reporting that is more meaningful and understandable for all concerned. Most people are unaware of research unless highlighted by the media, or they specifically search for it. Published research articles are frequently behind pay walls, therefore inaccessible to the general public. As a result, many rely on media reports that may sensationalize findings or must put significant effort into seeking out research on their own. Those living with disease may know of more unbiased and efficient ways to communicate research findings, in addition to communicating this information themselves. These methods might use caregiver and patient-centered resources, for example social media, trusted newsletters, or forums.



The [Research Loop](#) is a web-based application designed to close the gap that happens after research is published and before new research starts, giving caregivers and people living with a disease a chance to take part in all aspects of the research process. The Research Loop allows the layperson to [submit comments](#) on published papers, which are then sent back to the researchers or funders of the research. Individuals can comment on

everything from the study design, to the way the research was conducted, to the conclusions the researchers came to with their findings. With this feedback, researchers can then incorporate these ideas into their future projects. The Research Loop feedback form asks contributors to self-report where they encountered the research and how it affects them. This information can help researchers find areas where dissemination efforts are working and where they can improve. It can also indicate any issues with implementation and translation of findings into practice. Finally, the Research Loop website includes useful [resources](#) for those that would like to be more involved in research.



[Comparative Effectiveness Research](#) (CER) is intended to answer questions of importance to people living with a disease and their caregivers, and assist them with healthcare decision-making. In other words, it can be described as researchers doing research *with* patients, rather than *for, at* or *to* them. A [recent study](#), published in the Journal of Comparative Effectiveness Research, reveals how caregivers, patients and clinicians currently feel about engagement as partners in health research. Online surveys were administered to study participants, including more than 2,000 caregivers and patients managing rare and chronic conditions, as well as over 600 practicing clinicians. Caregiver and patient involvement in medical research was defined on the survey as getting involved in other ways besides being research subjects. Approximately half of the caregivers and patients participating in the study reported interest in research engagement, particularly in helping researchers understand patient/caregiver points of view about needed information, helping investigators decide what questions to study, making findings easier to understand, and getting results to those who can use them. The most commonly cited factors influencing decisions to engage in research were the belief that participation could result in meaningful findings, feeling respected by the researchers, and having an interest in the research being conducted. All participants endorsed the importance of improving patient care as motivating decisions to engage with researchers. Results of this study point to practical steps researchers can take to optimize the reach and value of CER.

In addition, to better partner with caregivers, researchers should make an effort to speak not only in patient-centered language, but also caregiver-centered language. For example, when discussing a protocol, study personnel should ask the caregiver whether or not he or she is willing and/or able to perform certain duties. Ideally, investigators should support the caregiver in figuring out the most effective and realistic way to comply with protocol requirements. It's important for research personnel to explain jargon used in clinical research. In



In some cases, the caregiver needs to understand what the researcher is saying, so as to effectively communicate this information to the person living with the disease. Research personnel should guide the caregiver on being an effective observer, when necessary, clearly stating what to watch for, how to identify it and how to respond. For studies in which participants receive medical treatment, it's important for researchers to prepare the caregiver and participant for the emotional aspect of ending treatment at the end of the clinical trial.

Pediatric studies most effectively model the optimal use of caregiver involvement. By necessity, pediatric specialists regularly interact with caregivers because, in the case of minor patients, the caregivers are the parents or guardians. Research participants must understand the purpose, risk and potential benefits in order to determine if participating in research is the best choice. Parents not only need to explain these risks to their child, but also must consent to their child's participation. Pediatric studies cannot proceed without parent involvement. In most cases involving adult subjects, caregivers are not the ones authorizing participation in research. Even so, they are often needed to investigate and clarify the ramifications of participating, to ensure that their loved ones understand the risks and make informed decisions about participation. Caregivers must often drive their loved one to medical appointments, help them follow protocols, observe responses to study medications and procedures, serve as liaison to study personnel, and otherwise nurture and support their loved one throughout the clinical trial process. Investigators would benefit from studying the model of pediatric clinical trials, in the interest of optimizing support of the caregiver, and ultimately the best interests of the patient community. In short, researchers should design their protocols with the caregiver in mind.

The tide is changing with regards to involving caregivers and patients in medical research and drug development. In June 2018, the Food and Drug Administration (FDA) released guidance on [Patient-Focused Drug Development](#) (PFDD). The guidance consists of four documents, addressing in a stepwise manner how investigators can collect and submit caregiver and patient experience data for research. Recognizing that this valuable input can better inform medical product development, as well as regulatory decision making



in the future, this series of documents is intended to facilitate the advancement and use of caregiver and patient input. The [Patient-Centered Outcomes Research Institute](#) (PCORI) was created in 2010 with the passage of the [Patient Protection and Affordable Care Act](#) (often referred to as the "Affordable Care Act"). Recognizing the importance of engaging all participants in healthcare throughout the research process, PCORI is committed to producing and facilitating CER. PCORI's mission is based on the premise that engagement of all stakeholders in healthcare can influence research to be more patient-centered, useful, trust worthy and ultimately reach the entire healthcare community. PCORI funded the development of [PCORnet, the National Patient-Centered Clinical Research Network](#) to accomplish its goals. PCORnet is made up of a number of [partner networks](#), all working to further PCORI's mission. As a member of PCORnet, Accelerated Cure Project launched the [Multiple Sclerosis People-Powered Research Network](#) (MS-PPRN), [iConquerMS](#), in 2015. The initiative's [portal](#) enables all people living with MS to play an active role in research. Another network, the [National Alzheimer's & Dementia Patient & Caregiver-Powered Research Network](#) (AD-

PCPRN), has put caregivers, along with patients, at its center. In dementia, a caregiver frequently administers treatments, ensures compliance with medications, and ultimately becomes the surrogate for patient reported outcomes. [Meryl Comer](#), co-Principal Investigator of the AD-PCPRN, is a caregiver for her husband and mother, both of whom suffer from Alzheimer's disease. She is a strong advocate for



caregiver involvement in the research process. She sums it up nicely when she says, “Arm us with technologies and we can be your data collectors in place, in the home... Validate our observations instead of dismissing them. Let caregivers share the spirit of research with the knowledge that we are helping other families and the next generation.... I think that combination of technology with a mission that validates what we do every day in a very hopeless space will be empowering and I think it is something we can share.”



It's also important to stress the importance of caregiver-focused research. Caring for a loved one with a long-term illness is a 24/7 job that can span decades. Those providing support are often so focused on their partner's needs they may not have time, or think to take care of their own. In this situation, over a long period of time, it's conceivable that caregivers could become second victims of any extended condition. Families living with chronic disease cannot afford to have both the patient and caregiver sick at the same time. It's

imperative for researchers to investigate caregiver health and burden, as well as coping mechanisms and strategies. Learning more about these important topics would help sustain a vital relationship that is the backbone of many families.

Caregivers play an indispensable role in their loved ones' lives. They may also play a pivotal role in medical research. By sharing their experiences of the daily burden of disease, their interpretation of unmet needs, and the types of research questions most important to them, patients and their care partners can transform the research process from one directed by sponsors and investigators to one driven by the needs of patients and their caregivers. This change in impetus has the potential to improve healthcare and outcomes not only for people affected by MS, but for the general population.