

Treating Parkinson's Patient Engagement in Research



When we hear about a new blockbuster drug coming to market, we usually think about a medical treatment. But just recently, the *Journal of Health Affairs* noted a medical breakthrough of another kind. This one is not an actual drug, but is something with the same potential to alter the research and health care landscape: the phenomenon of patient engagement. On face value, patient engagement may not sound revolutionary. Yet it represents a marked departure from traditional models of medical practice and research in which the doctor holds the knowledge and makes decisions for us. It involves a genuinely collaborative relationship between patient and doctor. It views people not

simply as patients, but as health care consumers. Patient engagement means inviting patients to weigh in on treatment options instead of simply letting the doctor choose the medication that he or she thinks is best and including the patient in determining which types of treatments should be on the market.

Where It Began and Where It's Going

Encouraging people to take an active role in their own health care, and the research that will lead to new treatments and cures is something that has been central to the work of the Parkinson's Disease Foundation® (PDF®) for more than 10 years. A decade ago, PDF saw the momentum building as patient advocates in other communities, such as breast cancer, used patient engagement to speed up the pace of drug development. Naturally, we asked: how could this be applied in Parkinson's disease (PD)? Today, the idea of patient engagement is catching on in the medical research community. We think it has the potential to become a blockbuster and we want to do all we can to promote and foster it within the Parkinson's space.

Isn't it simply common sense that becoming involved in your own health care may be good for your health? The answer is yes — and that's why, in the early stages of discussion about patient engagement, the buzz was all about personal engagement, about a person's role in his or her own care, with his or her doctor and health care team.

But over time, our understanding of patient engagement is evolving into something broader — from exploring ways in which informed patients can help improve their individual health status, to exploring ways in which they can improve care and accelerate therapies for the community as a whole.

An Idea Whose Time Has Come

Before we look forward, let's look back to understand how the general landscape of patient engagement in research has changed in the United States. In the 1980s and 1990s, people affected by HIV/AIDS and breast cancer recognized that decisions about research were impacting the treatments that could save their lives. They fought to have a say in the kinds of research that should be funded and the processes by which new drugs should be developed.

It was because of the organized protests of HIV/AIDS activists in 1988 that the US Food and Drug Administration (FDA) instituted a "fast-track policy," which allowed people with very serious diseases to have access to certain experimental drugs. If you have heard of the drug trastuzumab (Herceptin®) for breast cancer, then maybe you

(over please)



Patient Engagement In Action



"This is a hard disease. Hard because there is no cure. The only way we are going to speed up the pace of drug development is by getting involved in research. Clinical trials sorely need people with Parkinson's disease and those without it. As PDF Research Advocates, my husband Al and I bring this critical message to support groups, health care professionals, and students in our community."

Jane Denmark, Bay Minette, AL



"In my role as a PDF Research Advocate, I am working to help the California Parkinson's Disease Registry identify the people with PD living in this area. This could make a huge difference in understanding the disease."

James Wong, Ph.D., Orange, CA



"As a PDF Research Advocate now working with the FDA, my goal is to be a protector of the consumer and the liaison for people who live with neurological diseases such as Parkinson's disease. I will represent their viewpoint and their urgency, while ensuring that potential new drugs being reviewed are not only effective, but safe."

**Rich Hoffmann, Pharm.D.,
Hernando, FL**

know that it was approved largely because of a passionate and dedicated partnership between advocates and a pharmaceutical company.

What is different about this next wave of patient engagement is that the "establishment" organizations that were the targets of health advocates in the earlier era are now helping to lead the charge. One by one, we have seen a shift in the tide. Take for example, the change afoot at established agencies such as the FDA as well as the creation of new institutions such as the Patient Centered Outcomes Research Institute (PCORI).

The FDA regulates medications and medical devices — for example, the ones that are used in deep brain stimulation surgery for Parkinson's. The FDA began working with patient advocates in the 1990s, inviting advocates to serve on committees whose charge it was to evaluate drugs or medical devices that were close to approval. Now, as part of what the agency calls Patient-Focused Drug Development, they are asking for patient input earlier in the research process. For example, they are inviting advocates to help determine what drugs are developed for Parkinson's.

Meanwhile, PCORI was created in 2010 with a \$3 billion dollar budget through federal health care reform law to support research designed to help doctors and patients make informed health care decisions. A central goal of PCORI is ensuring that patients are involved within the internal workings of the organization — determining research priorities, reviewing grants and providing input on issues that relate to PCORI's mission.

Momentum Taking Hold in PD

At PDF, our commitment to fostering and advocating for increased patient involvement began ten years ago through the formation of the PD trials coalition and initiative. These programs sought participation from people with PD in research, primarily as participants in clinical studies. Much like other agencies, PDF's understanding of patient engagement is evolving over time — we now recognize that although study participation is critical, it is just one avenue for patient engagement.

So in 2008, PDF formed Parkinson’s Advocates in Research (PAIR). This program trains and supports people with PD and care partners to create a foundation from which they can become involved in research decision-making.

A New Generation of Advocates

To date, the PAIR program has trained more than 250 people with Parkinson’s and care partners to serve as Research Advocates who “pair up” with research institutions and scientists to bring about better treatments at a faster pace.

They work at the grassroots level to bring the information they have learned to others, in support groups and other settings. For example, Jane and Al Denmark of Bay Minette, AL, have visited support groups around their state to let them know about the latest in Parkinson’s disease research and how to get involved. When Jane, Al and other PDF Research Advocates surveyed individuals who had heard their presentations, 96 percent stated that they would seek out information about clinical study participation in the future!

They help to expedite local research programs. For example, Jim Wong, Ph.D., of Orange, CA, is a community advocate to the California Parkinson’s Disease Registry, which collects data from

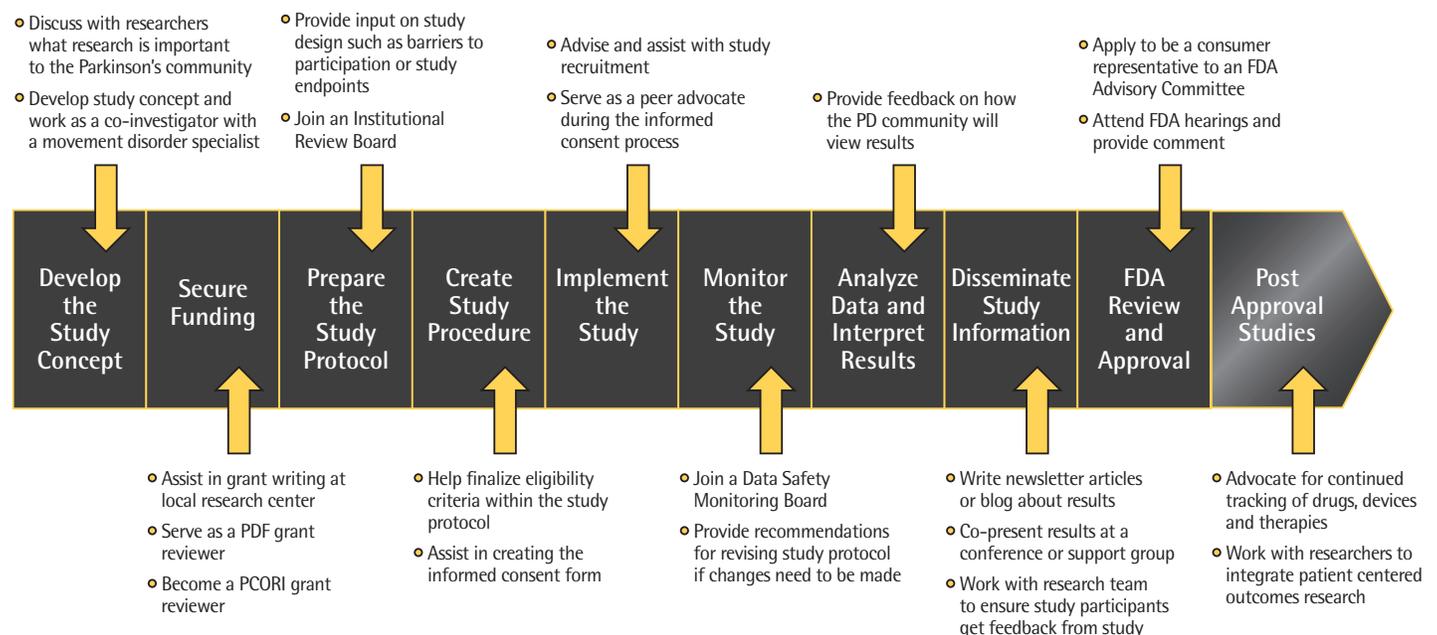
residents to accelerate new therapies. He serves as a liaison, letting his fellow residents know why it is so important to share their information with the registry, and advising the registry on ways in which it can work better with the community.

They take on national roles in the research-process. For example, Rich Hoffmann, Pharm.D., of Hernando, FL, a professional pharmacist, is serving as a consumer representative to a committee of the FDA. In this role, he helps to evaluate potential new drugs to treat neurological disorders including Parkinson’s disease.

Partners in Progress

For years, PDF Research Advocates have been calling on agencies, researchers and companies to say, in effect: we want to help you do your job better so that our lives improve. Now these same entities — agencies, researchers and companies — are calling us to learn how Research Advocates can join them in their work. In fact, the Parkinson Study Group (PSG), which focuses on clinical studies in PD, invited PDF Research Advocates to serve on working groups in which proposals for new research studies are generated and on a scientific review committee, which is charged with reviewing all research proposals.

Where Patients Engage in the Research Process



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A Tipping Point

The most exciting part of this change is the increasing recognition by the research community that the perspective and expertise of people with Parkinson's need to be part of their decision-making — that this collaboration improves and energizes the process through which new treatments are developed. We know that when advocates from other disease communities demanded a role in research it tipped the balance, and helped bring about new treatments. Parkinson's disease should be no different.

Do you think of yourself as a consumer with an expertise in Parkinson's disease? Well, you are. Your experience and opinions are invaluable to the professionals who provide your medical care and develop your therapies. Think about it: in any other business, consumers are in huge demand. If you have ever sat in a focus group, or answered a survey, you know that's the case. A part of patient engagement is ensuring that the key players in the PD drug development process — government, pharmaceutical companies and researchers — tap into your expertise and consumer preferences to help them create and improve their products to meet your needs.

Now that you know just how powerful you are, will you take action or will you sit back and hope that other people will solve these problems?

The answer: Take action! If we really want to live better with Parkinson's and find cures as soon as possible, then it will require engagement from all of us. No one understands the urgency for new Parkinson's disease treatments like the people who live with it. No one can communicate it, or advocate for it, like you can.

So what we say to you is — we can't do this without you. In this vein, it is time for us all to embrace and act on the invitation of Jay Phillips, a PDF Research Advocate from Summerville, SC, who said recently to a group of researchers: "we are here and we are ready to work with you!"

Veronica Todaro, M.P.H., is Vice President of National Programs at Parkinson's Disease Foundation.

Resources

Getting Involved in Parkinson's Research

This publication can help one to stay educated and informed about clinical study participation. Contact us for a free copy.
www.pdf.org/publications

Parkinson's Advocates in Research (PAIR)

PAIR is a signature program of PDF, the cornerstone of which is a network of 200 Research Advocates from 42 states. Contact us to apply to upcoming trainings.
www.pdf.org/pair

PAIR Online Course

This online course offers the core components of our in-person trainings from the comfort of home. Participants receive a certificate and will then have the opportunity to work with their local communities to find ways to bring about better treatments at a faster pace.
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Key



Book



Website



Organization

If you have or believe you have Parkinson's disease, then promptly consult a physician and follow your physician's advice.

This publication is not a substitute for a physician's diagnosis of Parkinson's disease or for a physician's prescription of drugs, treatment or operations for Parkinson's disease.