April 2020 Newsletter

Accelerated Cure project for multiple sclerosis

Accelerating research towards a cure for multiple sclerosis

iConquerMS™

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Patients Are the Experts

Patients are experts in what it's like to live with their condition and the effectiveness of its treatments. They know the most about symptoms and the quality of life improvements that matter most, as well as what therapeutic benefit/risk tradeoffs an individual with a given condition would be willing to make. People living with MS are the best source of information to help researchers understand the therapeutic context for MS drug development and evaluation. However, this important input hasn't been considered comprehensively in the drug development process throughout the industry until now.

In 2012, the U.S. Food and Drug Administration (FDA) established the Patient-Focused Drug Development (PFDD) initiative. PFDD is a systematic approach to ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. The FDA is developing a series of PFDD guidance documents to address how patient and caregiver input can be collected and used in this regard.

In recent years, the FDA has made progress on its goal to obtain the patient perspective on certain disease areas and incorporate patient input in its drug review process. They conducted a number of PFDD meetings in order to more systematically obtain the patient perspective on specific diseases and their treatments. These diseases included chronic pain, autism, breast cancer, chronic fatigue syndrome, fibromyalgia, lung cancer, Parkinson's disease and psoriasis (among others). These meetings provided key stakeholders, including FDA staff, patient advocates, researchers, drug developers, healthcare providers, and others, an opportunity to learn more about what matters most to individuals and caregivers impacted by each condition. Attendees learned about such topics as the impact of symptoms on daily life and the effectiveness of different treatments, as well as the challenges or barriers to accessing them. A main take-away lesson from these meetings was that patients, in general, want to be engaged in the drug development process.

At the end of 2019, EMD Serono initiated a global Phase III clinical trial (EVOLUTION RMS 1) studying the efficacy and safety of evobrutinib, compared to interferon beta-1a, in adult patients with relapsing remitting MS. Recruitment for the study is currently underway with an enrollment goal of 1,900 subjects and a planned target completion date of June 2023. Prior to the initiation of the trial, ACP entered into a year-long partnership with EMD Serono that was well aligned with the FDA guidance. The purpose of this partnership was to ensure the patients' complete MS experience was captured and meaningfully incorporated into the design and implementation of this trial. As part of this collaboration, eight members of the iConquerMS community were invited to participate in a PFDD Council. The Council worked closely with the iConquerMS project team and team members from EMD Serono, attending face-to-face meetings and periodic conference calls over the course of the collaboration. They drew from their own experience to provide feedback and insights on the choice of PROs, patient-facing materials, and endpoints in the trials (specifically regarding the relevance of PRO measures to the real-world patient experience).

The collaboration between iConquerMS and EMD Serono is the first of its kind in MS drug development research. Feedback from the Council has had a lasting effect in the whole spectrum of what is important in drug development throughout the industry (how the success of a drug is measured, what is said about it). Input from the Council changed the way the project team at EMD Serono thought about what is important to people living with MS, for example the impact of symptoms or relapses on daily life, and the way information is presented on drug labels. Active listening and feedback on follow-up actions by EMD Serono helped reinforce mutual respect and a partnership spirit in the collaboration and an overall positive experience by council members. The Council members expressed a desire for more frequent communication.

Two members of the PFDD Council, Laura Kolaczkowski and Margot Bjoring, shared their perspective on the experience at a recent meeting with EMD Serono. According to Laura, "When it comes to what life with MS is like, Margot is the expert, I'm the expert. It's together we can all get onboard and pull that expertise together… All aboard, not just some, not just a few, but all of us aboard on this journey towards getting drugs approved that are going to improve our lives." In Margot's words, "It's really challenging to bring together people with such different domains of expertise. The first thing we had to do was establish a common language, establish communication between all of these different groups… We talked about a relapse versus a flare-up. If you asked a clinician, they might say those mean the same thing. But, for people with MS, those actually mean very different things. That becomes very important when you're talking about patient reported outcomes. To know what the words you're using mean to the people who are going to be reading them… We navigated those language barriers and over the course of the year of our work, it was really incredible to see the how comments and discussions we made in the early meetings began to be incorporated into the design of the trial, the structure of the PROs, the kinds of questions that were being asked, even labelling language, patient education material… It was really rewarding and, from the point of view of the council members, a huge success. It felt like a true collaboration."

The movement to include the patient voice in drug development and evaluation is growing. More and more, patients and caregivers are being recognized as partners in the drug development process not just in MS, but across many conditions. For example, according to a recent article in the American Society of Clinical Oncology Educational Book, including patient perspectives in the trial design, regulatory approval and assessing the effectiveness of cancer treatments is critical. Oncology patients must often choose between treatment regimens with different balances of efficacy and toxicity. Therefore, it is essential for all stakeholders in the drug development process to keep in mind what constitutes meaningful benefit to cancer patients and the best source of this information are the patients themselves.

ACP and iConquerMS are leaders in the growing PFDD movement. iConquerMS members recently played a key role in ensuring the voice and experience of people living with MS is incorporated into the development of a new MS therapy. PFDD Council members were selected from network participants who provided their data by logging into the portal and completing their surveys. iConquerMS empowers everyone affected by MS to participate in research. Data from people with MS, caregivers and family members are essential to helping researchers better understand the impact of MS and its treatments. Be a part of the growing PFDD movement! If you haven't already done so, please consider enrolling in iConquerMS. Already a member? Please log in and complete your open surveys today! Whether you have MS or not, every piece of data expands the pool of information that investigators can draw from.