



Dear Friends,

It's been a busy month here at Accelerated Cure Project for MS (ACP). The longitudinal study we reported on last month, OPT-UP (which stands for Optimizing Treatment—Understanding Progression) took another big step towards launch when biotech firm Genentech became a founding sponsor by committing \$2 million over two years to learn more definitively what drug works best for what people with MS. In doing so, they join founding sponsor, EMD Serono and the National Multiple Sclerosis Society. Meanwhile, ACP is in discussions with additional potential sponsors as we aim to close the gap on remaining funds needed.

October saw ACP connecting with people across the country and around the globe. In this issue, you'll read about a series of gatherings we organized in three states to move patients into the driver's seat of MS research. The iConquerMS™ research studios brought together groups of 12-15 people with MS, as well as MS clinicians who conduct research, to identify research topics of importance to people living with the disease.

At the beginning of the month, an ACP board member and several staff attended the European Committee for Treatment and Research in MS (ECTRIMS), the world's largest annual international conference devoted to basic and clinical research in multiple sclerosis, held this year in Barcelona, Spain. For conference highlights, visit our MS Research news site, Multiple Sclerosis Discovery Forum (<http://msdiscovery.org>), to read the latest Research Roundup and check out the extra podcast interviews.

As always, please let us know if you have comments on this newsletter!

### **Gatherings Around the Country Generate Patient-Centric MS Research Topics**

“What questions do you have about multiple sclerosis when you're preparing to meet with your neurologist and, equally important, what questions occur to you after you've left her or his office?” This is the way Dr. Tim Vollmer framed the conversation at a recent gathering held in Denver, Colorado. Called a “Research Studio,” the event brought together 15 people with MS and Dr. Vollmer and his colleague, Dr. Augusto Miravalle, to generate ideas for MS research on the topics that matter most to people who are living with the disease. It was one of four similar meetings held this month across the country; the other three were in Atlanta and San Diego. A total of over 50 people with MS participated.

The Research Studios were organized under the auspices of iConquerMS™ ([www.iConquerMS.org](http://www.iConquerMS.org)), as a way to further the initiative's goal of empowering people with MS to impact the MS research agenda. They showcased the wisdom of people with MS: those who are recently diagnosed and are literally brimming with questions; those who have lived with the disease for decades; and everyone in between. In each location, neurologists and other clinicians who conduct MS research and who embrace the goal of patient empowerment participated, listening attentively and contributing their own knowledge and expertise to the conversation. In addition to Drs. Vollmer and Miravalle, these included Drs. Ben Thrower, Deborah Backus and Guy Buckle of the Shepherd Center in Atlanta and Dr. Revere "Rip" Kinkel of the University of California San Diego.

A wide range of issues affecting people with MS was discussed at the meetings and from it, a long list of possible research topics emerged. Common themes included studies about possible *causes* such as genetics, viruses and Vitamin D deficiency; *treatments* such as stem cell transplants, medical marijuana and the recently announced ocrelizumab (among many other disease modifying therapies); and the impact of *self-care options* in the areas of exercise, diet and stress management. In several meetings, participants cited the increase over the past decade in the number of African-Americans diagnosed with MS and noted that research on MS in African-Americans is far behind the curve. And speaking of diagnosis, numerous participants expressed the belief that they had MS "for a long time" before being correctly diagnosed with the disease. Said one participant, a 45-year old man with PPMS, "I believe that I've had MS for most of my life, because I experienced symptoms that are associated with MS from the time I was a child. I just didn't get the proper diagnosis until 10 years ago."

In Atlanta, topics relating to physical therapy, exercise and stress management took center stage, with several participants expressing a desire to push themselves to their maximum capability, in the hope of regaining some portion of the athleticism and the cognitive abilities they possessed before contracting MS. They raised the question of whether it's possible to reverse damage and disability through physical exercise and purposeful mental activity.

In Denver, a participant observed that, "Not all people with MS are motivated to help themselves. How do we reach them?" This precipitated a lively discussion about ways to alter the doctor/patient relationship, so that people with MS are educated and feel empowered to make the best use of their time with their doctor. The same participant who had raised the question asked, "For patients who are new to MS, could another patient serve as their advocate and accompany them to their doctor visits?" He then offered, "I would gladly go along with another patient to help them formulate questions and write down answers for later reflection."

In San Diego, participants picked up this theme, with one woman wondering, "How much of my neurologist's time can I expect?" while acknowledging that she is reluctant to take more than her fair share. Other topics that interested participants in San Diego were the impacts



and efficacy of autologous stem-cell transplants and the use of medical marijuana (only legally available in a handful of states).

In the coming month, Accelerated Cure Project staff will produce and make available a report detailing these and the many other ideas that surfaced during the Research Studios. Initially, the report will be shared with the over 2,500 people who currently make up the iConquerMS™ network, with more people joining every day, and the additional people who participated in the Research Studios.

The Research Studios represent just one means by which iConquerMS™ is gathering input from people with MS. Other means include soliciting research ideas from members via the iConquerMS™ portal. While iConquerMS™ is still in the process of developing protocols for reviewing and prioritizing research topics suggested by people with MS, in the coming months we expect these processes to be formalized. For instance, it is likely that a list of the most commonly suggested topics will be posted on the “For Researchers” section of the iConquerMS™ website, to let investigators know the topics in which the patient community is particularly interested.

*If you or someone you know has MS and hasn't yet registered with iConquerMS™, we encourage you/them to visit [www.iConquerMS.org](http://www.iConquerMS.org) and register. The process is straightforward and will ensure that you are kept abreast of future action on the topics generated in the Research Studios.*

*We thank Genentech, whose generous grant made the Research Studios possible.*

### **Genentech Joins Funders of OPT-UP Clinical Study**

On October 7, Genentech became a Founding Sponsor for the Optimizing Treatment - Understanding Progression (OPT-UP) clinical study that Accelerated Cure Project for MS hopes to launch in the coming year. If you are a regular reader of this newsletter, you have read about this initiative in previous issues. OPT-UP is a U.S.-based, multicenter longitudinal clinical research study that will enroll 2,500 people with MS, and follow them for up to five years. The goals of the study are to generate a robust evidence base of factors affecting treatment outcomes in MS to guide the choice of treatments for MS patients, and to produce knowledge and tools for developing strategies and/or medicines to slow, arrest, or reverse MS progression.



Recognizing that the unpredictability of treatment responses for individual patients can be demoralizing for people with MS and their clinicians, ACP has designed a clinical research study to address the most critical medical needs in MS today. We are delighted that Genentech, an emerging leader in MS therapeutics, has chosen to participate. In doing so, Genentech joins OPT-UP's Lead Founding Sponsor, EMD Serono, and the National MS Society in providing funding for the OPT-UP study. ACP expects that other MS stakeholders will soon join the OPT-UP Founding Sponsors group and that this important study will begin enrolling patients in 2016.

People with MS will be enrolled in OPT-UP at up to 20 MS clinics located throughout the U.S. and will be followed for a minimum of two years and up to five years as they make choices of DMTs and any subsequent switches to other DMTs. Using validated and standardized in-clinic assessments and online questionnaires, the OPT-UP study will collect high-quality data on treatment outcomes, along with biological samples and imaging data collected under standardized protocols and processed by commercial vendors under standardized operating procedures. These comprehensive resources will be analyzed by the ACP network investigators and partner organizations, as well as shared widely with research groups that can help accomplish the goals of OPT-UP.

Despite the availability of many DMTs, physicians and patients still make many treatment decisions based on trial and error. The primary OPT-UP study objective is to provide robust evidence that can enable physicians and their patients to decide on the optimal treatment strategy for each individual person living with MS. The study exemplifies a new generation of clinical studies designed to enable personalized, or precision, medicine. In addition to standard clinic-based assessments from the OPT-UP study, researchers will also have access to critically important patient-reported outcomes that will be captured online at regular intervals. ACP believes that the substantial amount of data and biosamples generated by the OPT-UP study will also be enormously valuable in furthering researchers' understanding of MS disease progression.

As of October 2015, nine clinical sites are participating in OPT-UP, including the University of California San Diego, University of Texas Southwestern Medical Center, University of Massachusetts Medical School, Tisch MS Research Center of New York, Johns Hopkins School of Medicine, Ohio State University Medical Center, the Shepherd Center in Atlanta, University of Colorado School of Medicine, and Stanford University School of Medicine. ACP is currently seeking additional qualified clinical sites for the study. Stay connected with Accelerated Cure Project for MS to hear about future progress on OPT-UP, including when the study is ready to enroll participants. In the meantime, if you have questions, please contact Hollie Schmidt of [Accelerated Cure Project for MS](#) at [hollie@acceleratedcure.org](mailto:hollie@acceleratedcure.org).