

## Accelerating MS Research by Sharing Samples and Data from ACP's Repository

*By Katina Leodas*

Since its inception in 2006, ACP has aimed to distribute samples and data from its Repository as broadly as possible within the multiple sclerosis research community. To date, we've supported over 90 studies conducted by scientists around the world. In 2014, we realized that while Repository samples and data had been used by dozens of researchers over the preceding 8 years, much more research into MS could be done with this resource if more researchers knew of its existence.



That realization prompted us to adopt a highly pro-active approach to publicizing its existence and to distributing the samples, all of which remain vital and useful to this day. If scientists weren't coming to us of their own accord in the numbers we desired, we needed to reach out to understand their needs, make them aware of this valuable resource, and help them identify ways the Repository could advance their research. In years past, this task fell to long-time ACP staff members, Hollie Schmidt and Sara Loud. Just over one year ago David Gwynne, was recruited to ACP as its Director of Alliances and Collaborations, to coordinate this activity, working closely with Sara and Hollie.

An experienced scientist and business development executive, David develops and maintains relationships with investigators who could or already do have interest in the Repository biosamples and data. Born in the United Kingdom and educated in Canada, David studied molecular genetics and obtained a B.Sc. and M.Sc. from the University of Toronto, followed by a Ph.D. from McGill and post-doctoral studies at the University of California, Davis. Before coming to ACP, he led research and drug discovery teams in biotechnology companies in Toronto, Canada and Cambridge, MA, the latter involving him in the leadership of a multiple sclerosis drug discovery effort. David's career eventually included leading business development and licensing activities for several companies, something he has also pursued as an independent consultant, with a focus on neurology.

By training and temperament, David is perfectly suited to the work. His scientific background gives him unique insights into the technology underpinning licensing opportunities. And conforming to a positive stereotype we Americans hold of our neighbors to the North, he is one of the friendliest people we know. David says, "I've got a great job! I love talking to people, I love talking about science, and I like solving problems, which is what business development is all about."

Many of the commercial clients of the ACP Repository are early-stage biotechnology companies. It's difficult, costly and time-consuming to build a successful biotechnology company. Having a novel idea is really just the first step. After raising seed capital, scientist/entrepreneurs must advance the technology consistently through successive tests and value-increasing product development milestones. All investors like to hear about companies making steady progress along a planned development pathway and this, in turn, puts scientists under pressure—some times huge pressure—to demonstrate consistent progress by meeting the milestones. By achieving that in a timely and consistent manner, entrepreneurs increase the value of their companies, decrease the investment risk and increase the likelihood of securing subsequent funding from investors.

David knows the science and he understands these pressures and cycles of growth, which makes him very good at identifying the “pain points” of biotech companies and suggesting areas where their use of samples from the ACP Repository will advance their work towards short- and long-range goals. He reads the relevant scientific journals, stays current on work being done in the field, and tracks the progress of new companies or of established firms with new products in development. Then he picks up the phone and makes cold calls to scientists and company R&D executives whom he believes can use our samples. By demonstrating his knowledge and understanding, he gets scientists' attention and effectively spreads the word on the ACP tissue repository and data assets.

David has described one area where the ACP Repository can make the biggest contribution to the growth of new therapeutics ventures. “Our sweet spot,” he says, is after companies have studied the effect of their molecule on mice. “They've gathered some positive pre-clinical efficacy data and they're planning to test their drug candidate on humans. Within a matter of weeks, we can send them samples, which enables them to test and generate human disease data.” That, in turn can help them acquire investor and other funding sufficient to complete the demonstration of clinical efficacy in humans. This is particularly important in MS, given the limited applicability and relevance of animal models to the treatment of MS in humans.

There are several ways in which our samples are most commonly used. Some companies use them to discover biomarkers that can reveal or predict disease as well as determine how a patient may respond to a particular drug. Biomarkers can also be useful to drug discovery companies to identify drug targets. A second way to use patients' samples is a drug challenge to blood cells. Some of the most useful samples in the Repository contain live cells, the white blood cells, known technically as peripheral blood mononuclear cells (PBMCs). These are blood cells, which include some key components of our immune system (lymphocytes, and monocytes). They are a critical component in the body's fight against infection. Because the PBMCs in the Repository are from individuals with MS whose immune systems are regulated differently, they can be used to reveal the biological differences between cells from MS patients and those

from donors who do not have MS. Scientists developing drugs that they hope will affect MS can expose these cells to their molecules to see whether their drugs can generate the appropriate biochemical response.

An example of this “PBMC challenge” approach was detailed in our [April 2016 issue](#). Innate Therapeutics, a New Zealand company, is using ACP samples to develop a drug that modifies the function of the immune system, in order to interrupt a recognized pathological process that occurs in progressive multiple sclerosis (MS). The hope is that their therapy will halt and, ideally reverse that process.

Increasingly our samples are used by companies seeking to reposition a drug that has already been approved to treat another disease, as a treatment for MS. Known in the industry as “repurposing,” this approach can be the fastest and least costly path to getting a new drug on the market. In fact, the majority of drugs submitted for approval to the FDA today are repurposed. Along these lines, ACP was recently approached by a company whose drug was approved a number of years ago for treating high blood pressure. Its scientists wanted to test the drug on ACP samples to see whether any impact could be detected on MS. Since the blood pressure medicine was commonly prescribed about 10 years ago (when most of the samples in our Repository were drawn), David wondered whether samples of people with MS who had taken the blood pressure medicine might already exist in the Repository. By just looking at those samples and comparing them with other samples of people with MS who did *not* take the blood pressure medicine, it seemed plausible that something valuable could be learned. Indeed, we had samples in the Repository that met this criterion (the donors had taken the blood pressure medicine) and the company is currently considering a research project that would include a comparison of samples from MS patients taking the blood pressure medicine with those who were not on the drug.

If you’re wondering, as I did, about the numbers of samples that we distribute to each company, the answer is, it’s all over the map (literally and figuratively!). Numbers of samples sent have ranged from less than 10 to over one thousand. Once a researcher expresses interest in receiving samples, David, Sara and Hollie work with them to understand the nature and scope of their research and the particular project they are undertaking. They then assist the researcher to develop and refine a clear proposal to the Repository for samples. The proposal is reviewed for scientific merit, research quality and likelihood of success by an Oversight Committee composed of many of the people who were involved in the Repository’s creation. They include the Principal Investigators from the 10 original MS clinics that gathered the blood samples; representatives from two non-profit organizations with which we partnered on creating the Repository; and ACP staff. This is followed by selection of the actual samples that meet the scientists’ criteria. ACP then notifies Precision Bioservices, the Maryland-based company that houses the Biorepository, to “pull” the samples we have selected, and within several days they are sent to points around the world.

Over the following year and a half, we maintain close contact with the researcher, tracking their progress and checking to make sure their needs are met. Sometimes, they request follow-up samples to confirm their original “pilot study” results in a larger experiment. Just as often, during the course of their work they identify other factors they need to test and this necessitates additional samples. Regardless of the outcomes, under the terms of our agreements, within 18 months of completing their studies, all researchers are required to return to ACP all data generated by the work done with ACP samples. In this manner, and as envisioned by ACP at the time we created the Repository, we are steadily building one of the largest collections of data about people with MS anywhere in the world. We are currently seeking funding to organize and analyze the data, in order to determine the most promising fruitful directions for future MS research.

More than 3,200 people contributed samples and data to the ACP Repository. It is important for them to know that their contributions are still working hard to generate new insights. While we are no longer enrolling new participants in the Repository, our online patient-powered research network, iConquerMS ([www.iConquerMS.org](http://www.iConquerMS.org)) provides everyone with MS an opportunity to power research in a similar fashion. For more information on that initiative, we encourage you to visit <http://www.iConquerms.org>.