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Partnering for a Cure

By Scott Johnson

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The Myelin Repair Foundation is creating a process for the rapid development of new treatments and cures

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In 1976, during a backpacking trip through Europe with my girlfriend, I lost the vision in my right eye and soon after experienced a peculiar numbness from the waist down. A doctor there told me that I might have multiple sclerosis (MS). I'd never heard of it. I was 20 years old.

When I returned to the United States, a neurologist confirmed what the doctor in Germany suspected. I had a relapsing-remitting form of MS in which my immune system, without warning, would attack the insulating substance on the nerves in my brain and spinal cord, called myelin. These attacks would weaken or disrupt the electrical signals passing among my nerve cells, causing a wide range of possible symptoms, including paralysis, vision and hearing loss, focus and concentration problems, and incapacitating fatigue. Today, I am one of 2.5 million people living with these unpredictable, debilitating symptoms. There is no cure.

Like many people diagnosed with MS and other chronic diseases, I did my best to hide and ignore it. Though occasional attacks slowed me down, I completed my undergraduate civil engineering degree at the University of California at Davis and an MBA at the University of California, Berkeley's Haas School of Business. I married my girlfriend, launched a career in business with the Boston Consulting Group, and eventually led three startup companies. By MS standards I have been more fortunate than most. But the attacks have taken their toll. My right arm no longer works, and without a brace on my right leg I am unable to stand or walk.

Over the past 35 years, I have experimented with several available MS treatments on the market—treatments designed to tamp down a self-destructive immune system or to reduce inflammation during attacks—but the potential benefits have eluded me. MS has affected every day of my life. And with each year and each news story promising a cure, I have hoped that I would benefit from the millions of dollars spent on finding a cure.

In 2001, I read a brief article in *Businessweek* about discoveries made at Yale University, which suggested that myelin damage in MS could be reversed. Repairing the myelin had the potential to restore lost function in MS patients. This news was especially exciting because the proposed treatment did not rely on suppressing the immune system to slow the progress of the disease. Instead, it relied on repairing and restoring the myelin damage caused by the disease.

Developing a myelin repair treatment was an irresistible problem to solve. I began to research what was known and not known about myelin biology, who the experts were, and the process of medical research. What I discovered was a large and complicated ecosystem with independent players who operated within their own cultures. Further, the incentives within these cultures were not always related to an outcome that would benefit patients. And most surprising from a business perspective, there was no plan to guide the players toward a cure. Soon it was clear why so much money was being spent on medical research with so little benefit to patients.

FREE AGENTS, COMPETING INCENTIVES

The US medical research ecosystem is a pipeline that depends heavily on the contributions of academic scientists, commercial biotech and pharmaceutical companies, and the Food and Drug

SCOTT JOHNSON is the president and founder of the Myelin Repair Foundation. His work on developing an accelerated model for medical research that turns basic biological discoveries into treatments or cures has been broadly recognized for its potential to bring new medicines to market.

ILLUSTRATION BY ANGUS GREIG

Administration (FDA). Below is a snapshot of each of their worlds.

Academic scientists are funded largely by the National Institutes of Health (approximately $35 billion annually) and by universities, philanthropic foundations, and independent research institutes (approximately $15 billion annually). For the most part, each scientist pursues an area of personal interest and hides discoveries until her work is published in a peer-reviewed scientific journal or presented at a professional conference. It can take as long as four years from the time a scientist writes a proposal until successful results are considered publishable. There are no records of failed experiments.

Each academic laboratory is like a small business. The CEO is the principal investigator, and the staff members are postdoctoral and graduate students. Academic laboratories in the same discipline compete for funding and the best students. A successful laboratory is one that can produce proposals that are funded and results that are publishable. The result of this $50 billion annual investment? Some 800,000 published papers each year.

In 2009, commercial biopharma, whose strategy is based on increasing shareholder value, invested more than $75 billion in research. But today's biotech and pharmaceutical companies are facing some steep challenges: The cost of bringing a new drug to market now exceeds $1 billion. Venture capital investment in new biotech companies has fallen off. The patents on large numbers of blockbuster drugs worth billions of dollars are expiring, creating competition from generic drug manufacturers. Studies have estimated that to meet its commitment to shareholders, pharmaceutical companies spend nearly twice as much on marketing as they do on research and development. New drug targets in the pipeline are fewer and fewer. All this adds up to a bleak picture for a once flourishing industry whose projected price-earnings ratios today are approximately half those of consumer products companies.

And finally, the FDA, whose job it is to regulate drug development, is caught in an unending balancing act: to protect consumers from ineffective or unsafe products, and to get valuable new drugs to market that will save or improve lives. As few as 9 percent of all Phase III clinical trials succeed. This statistic alone should raise important questions about an ecosystem in which such stunningly negative outcomes are the norm.

ACCELERATED RESEARCH COLLABORATION

In 2002, I founded the Myelin Repair Foundation (MRF) to solve two problems: to unravel the scientific mysteries that trigger the formation of myelin, and to transform a scientific ecosystem fraught with barriers into a more adaptive process that could fast-track new treatments.

We set out to recruit the best scientists who had expertise in myelin biology and were willing to break the rules. The groundbreakers included Stephen H. Miller of Northwestern University, Brian Popko of the University of Chicago, Ben Barres of Stanford University, Robert H. Miller of Case Western Reserve University, and David Colman of the Montreal Neurological Institute.

In exchange for funding, these scientists committed to developing and executing a research plan and to sharing their results, both successes and failures. This created a highly collaborative environment in which multiple experiments were done in parallel across labs. The experiments were overseen by a scientific advisory board of senior neuroscientists who helped us ensure that the work remained within the scope of the research plan. We call this model Accelerated Research Collaboration, or ARC.

Now in 2011, it would be difficult to find a research consortium that does not tout collaboration. But in 2004, although conversations about speeding medical research were surfacing in forums hosted by organizations such as Michael Milken's FasterCures, MRF's approach was novel and ultimately would prove groundbreaking. What most collaborations still lack is external management oversight, which keeps scientists focused on patient treatment. Back in 2002, we also put in place contracts with the participating universities to ensure that all relevant discoveries would be protected and ready for commercial licensing.

Fast forward to 2011. MRF's scientists have produced 150 drug targets against which various compounds can be tested and measured for their effect on myelin repair. They also have produced 24 new research tools—animal models and assays—that can be used more broadly in neurological research. Four patents have been awarded.

CROSSING THE VALLEY OF DEATH

In 2008, with several discoveries in hand, we began approaching pharma. We quickly learned that our best work lacked the level of validation—a rich set of data from multiple tests and animal models—that industry required for licensing. Although we had succeeded in building an academic collaborative that more rapidly produced large numbers of drug targets, crossing the valley of death from academic science to pharma was going to require more infrastructure—infrastructure that could produce industry-standard data compelling enough to attract pharma's billion-dollar investment.

We needed to add a more sophisticated level of industry expertise to our own staff. Jay Tung, our first pharma veteran, came aboard as vice president of drug discovery. Within a year we put in place a drug discovery advisory board whose members had successfully brought drugs to market. The payback of establishing this advisory group was quick and powerful. With their guidance, we have been able to identify 40 targets that are in clinical development for MS and other diseases. And we have been able to attract more top-level scientists, such as Mike Gresser, our chief scientific officer, who headed neuroscience and inflammation research at Amgen.

Our story would not be complete if I did not mention the challenges we have faced in raising the necessary funds to accomplish this work. Although we benefited early on from the generosity of many foresighted Silicon Valley entrepreneurs, venture capitalists, and foundations, including the Robert Wood Johnson Foundation and the Donaghue Foundation, we also have faced the challenge of raising money during one of the worst economic downturns in US history. It has been a job no less difficult than understanding myelin biology or executing a plan to cross the valley of death.

Not all social innovations are fast. The ARC model is a work in progress. Getting a myelin repair treatment on the market will not be the end of our story. It will be the beginning of a process to scale and replicate the model for other diseases. This is a day I hope will come sooner rather than later. ■