Tisch MS Research Center of New York's Priorities, Direction & Investigations for 2018-2021

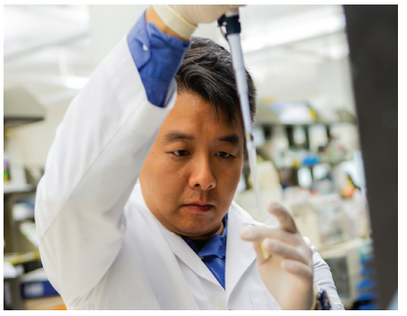
In 2001, our research center led by Dr. Saud A. Sadiq began an initiative to develop a reparative therapy for MS patients with established disability. Following several years of laboratory-based research developing a cell therapy and establishing efficacy in animal-based models of the disease, we were able to get FDA-approval for a Phase I clinical trial. Our Phase I trial was completed in 2017 and the results that were published earlier this year showed safety and tolerability, as well as encouraging efficacy trends. At Tisch MSRCNY, a commitment was made to invest in this novel treatment with the goal of bringing this therapy into clinical practice. A $5 million state-of-the-art Regenerative Medicine Laboratory was completed in March 2018 and we are now ready to embark on a landmark three-year Phase II clinical trial to hopefully establish the effectiveness of our stem cell treatment.

Moving forward we have established the following three projects as priorities for the upcoming years.

To better understand and treat coordination and balance difficulties in patients with MS. In some people with MS, the major reason for disability is a lack of coordination with their limb function. These patients have great difficulty with daily activities, including writing and walking. The area of the brain that is responsible for coordination is a cauliflower-like structure found at the base of the brain called the cerebellum. Unfortunately, this phenomenon does not respond to currently available medications and physical therapy has limited benefits. Dr. Anna Iacoangeli and her team are creating experimental models of the disease to identify the factors that lead to cerebellar dysfunction. In addition, her team is also investigating if there are unique molecular or protein signatures in the blood or cerebrospinal fluid (CSF) of patients that develop cerebellar disease. We hope that in the next three years we will translate what we learn from our experiments to develop rational therapeutic strategies. Finally, we hope to also initiate clinical trials later this year using novel approaches to better treat patients with balance difficulties.

To create an experimental model of progressive MS that will enable us to better understand and treat patients with secondary progressive MS(SPMS) and primary progressive MS (PPMS). In preliminary work, Dr. Jamie Wong and her research team have made some remarkable progress. Injecting minute quantities of CSF derived from PPMS patients directly into the CSF of the cervical spine of mice results in weakness of the limbs and demyelination.

Validation of this work is currently underway. These studies should allow our scientists to map the factors that lead to disease progression and also to develop new treatment strategies. The hope is that disease progression can be arrested in SPMS/PPMS patients so that repair strategies, such as our stem cell treatment, can be optimally effective. One approach that is being investigated is to cleanse out the damaging factors in CSF by a process of "pheresis."



Finally, and perhaps most importantly, we need to identify the initial trigger(s) that cause MS. This has been a central goal of the Tisch MS Research Center of New York since its inception. This work has recently been boosted due to a grant from the Emerald Foundation. Jerry Lin and associates have made immense strides in elucidating the CSF antibody response in patients with MS over the past decade. We have studied several thousand single antibody-producing cells (B-cells and plasma cells) and have tried to determine if they react with a number of myelin targets found in the human brain. We are also investigating the link with the Epstein-Barr virus to establish how the immune system in patients with MS escapes self-tolerance and becomes autoimmune. In some patients, we have found environmental trigger factors and are currently working on animal models to firmly establish these findings.

These priorities require additional funding and establishment of an experimental disease laboratory that we are planning to build adjacent to our recently completed Regenerative Medicine Laboratory. We are excited by the tremendous progress of our research in the past decade and are dedicated to finding the elusive cause of this disease and pave the path to an eventual cure.

Pictured above: (Left) Dr. Jamie Wong, Dr. Violaine Harris with Dr. Saud A. Sadiq (Right) Dr. Anna Iacoangeli with Research Assistants, Jerry Lin