



## **Frequently Asked Questions About Tisch MSRCNY & Our Research**

### **What is the mission of the Tisch MS Research Center of New York?**

The Tisch MS Research Center of New York is an independent nonprofit 501(c)(3) organization dedicated to finding the cause and a cure for multiple sclerosis by conducting groundbreaking medical research to accelerate the pace at which research discoveries translate from lab bench to bedside. The Center aims to identify the cause of MS, understand disease mechanism, optimize therapies, and repair the damage caused by MS while offering patient access to the best and most advanced treatment possible. The Center's medical research is designed to understand all aspects of MS, including research at the cellular and molecular levels, in order to treat and cure MS. Our stem cell studies are the most exciting of all of our research projects. The Neural Cell Regeneration and Repair study is working to perfect a treatment using the patient's own adult stem cells to regenerate and repair nerve cells and help patients recover lost functions.

### **Who is doing the research?**

Tisch MSRCNY has seven Principal Scientists and fourteen research assistants and lab technicians guided by Dr. Saud A. Sadiq, Director & Chief Research Scientist of the Tisch MS Research Center.

### **How is the research implemented at Tisch MSRCNY versus university laboratories?**

Tisch MSRCNY is the only research center located steps down the hall from the largest MS clinical practice, the International Multiple Sclerosis Management Practice (IMSMP). The seamless interaction between doctors and scientists at our state-of-the-art research facility is found nowhere else in the world. At the IMSMP, our MS specialists have the rare opportunity to bring bench research from the Tisch MSRCNY to the bedside of patients at our clinical practice. Our focus on patient-based research impacts and translates into the lives of those we treat and care for every day.

### **What are the clinical trials that are done at the Tisch MSRCNY?**

Tisch MSRCNY participates in a number of clinical trials in collaboration with their clinical affiliate, the International Multiple Sclerosis Management Practice (IMSMP).

To learn more, please visit: <http://imsmp.org/content/clinical-trials>

### **What happens in the Regenerative Medicine Laboratory?**

The recently constructed \$5 million Regenerative Medicine Laboratory is the home of Phase II of our stem cell clinical trial for MS. The lab includes a state-of-the-art cGMP (current good manufacturing practice) cell production facility. Biospherix created a specialized unit, called an isolator, which is a closed environment for cell processing. This specialized technology is ideal for cell processing because it limits contamination from the environment and consistently keeps the cells under the conditions they grow best throughout the entire manufacturing process. The new lab includes two isolators to handle the needs of our Phase II study.

### **For my friend/family with MS, how will the stem cell research being done at the Regenerative Medicine Lab help them?**

“We anticipate that these novel studies may form the therapeutic basis of reversing disability in patients with MS and if successful, these studies could have positive implications for treating other neurological diseases.” - Dr. Saud A. Sadiq

### **Is the Tisch MSRCNY affiliated with Tisch Hospital at NYU?**

No – Tisch MSRCNY is not affiliated with the Tisch Hospital at NYU Medical Center. It was in recognition of the Center's achievements in MS research and in support of Dr. Sadiq's mission to discover the cause and cure of multiple sclerosis that the Tisch family named the research center in February 2013, formally creating the Tisch MS Research Center of New York. The Tisch MS Research Center of New York is a nonprofit 501(c)(3) organization supported entirely by grants and private philanthropy.

### **What is the difference between donating money to Tisch MSRCNY and the NMSS since the NMSS also supports MS Research?**

The Tisch MSRCNY is an independent, non-profit organization and laboratory that conducts research to find the cause of and ultimately a cure for MS, and to develop reparative therapies. The National MS Society funds many research initiatives taking place all over the country, but does not conduct any research themselves. Currently, the NMSS is considering grant requests from Tisch MSRCNY in support of our Clinical Stem Cell research but **is not supporting our research at this time**. In addition to research, NMSS also funds several programs to support people who have MS.

### **What percentage of donations to the Tisch MSRCNY go directly to research?**

At Tisch MSRCNY 81% of funds are applied directly to our research, leading the Center to be referenced by Charity Navigator as “Highly Rated” and receiving a Platinum Seal from Guidestar.com

### **More information on our stem cell study & other investigations at Tisch MS Research Center of New York**

At Tisch MS we have several ongoing research projects. All investigations happening in the Tisch MS Research laboratory focus on learning more about this chronic disease and ways to end it or repair the damage it has caused.

### **Stem Cell Program/Regenerative Repair**

Our Phase I clinical trial is the only clinical trial of its kind in the United States to test freshly harvested MSC-NP cells administered intrathecally in multiple dosing regimens. Results showed dosing was well tolerated with only minor adverse events, and 15 of 20 subjects demonstrated functional neurological improvement. To make MSC-NPs, autologous stem cells are taken from a patient's own bone marrow. These are called mesenchymal stem cells (MSCs). The MSCs expanded from the bone marrow are then manipulated in the laboratory to make them into neural progenitor cells, which are MSC-NPs. Our Phase II randomized placebo-controlled study will test the efficacy of intrathecal MSC-NP treatment in patients with progressive MS.

This will be a double-blind study of 50 patients, all of whom will eventually receive treatment with MSC-NPs. Construction of the Tisch MS Center's, Regenerative Medicine Laboratory began in the summer of 2017, and it is expected to open spring of 2018. Upon completion of the build-out, we plan to begin enrollment of our Phase II Study.

### **Understand Disease Progression**

A major focus of research at Tisch MS is to investigate the underlying causes of disease progression using novel approaches based on comparative analysis of cerebrospinal fluid (CSF). Our emphasis is on identifying possible therapeutic targets for and novel biomarkers of disease activity in progressive MS patients. To accomplish this, we are studying changes in metabolism, the function of astrocytes, and the role of intrathecal delivery of methotrexate (IT-MTX) in this population. Additionally, we are developing a novel animal model to further understand the trajectory of progressive MS.

### **Discover the Cause**

To discover the cause of MS, our researchers are looking into what B-cells in the immune system are attacking. We are attempting to answer questions such as if there is a particular protein in myelin that's being attacked? Is EBV involved in MS? Can we find the trigger of MS, if there is one? It is our hope that looking at MS B-cells and the antibodies they produce, will answer some of these questions and ultimately lead us to the cause of the disease.

### **Translate the Research to Patients**

Biomarkers are specific and sensitive assays that help in better diagnosis, assessment of disease activity, and measurement of treatment responses. Using CSF analysis, we can determine how active the disease is (Fetuin A level), how likely is it to progress (osteopontin), and the level of oxidative stress (lipid peroxidation levels). Together with single B-cell analysis, for even the most complex patient cases, we can rationally decide on which treatment is most likely to be effective and how potent or aggressive a therapy is needed.

**To learn more about Tisch MSRCNY, please visit: [www.tischms.org](http://www.tischms.org)**