Institute for Translational Neuroscience at Northwestern Medicine
Center for Neurogenetics
CENTER FOR NEUROGENETICS
KEN AND RUTH DAVEE DEPARTMENT OF NEUROLOGY
AND CLINICAL NEUROLOGICAL SCIENCES

Center for Neurogenetics

With the arrival of Dimitri Krainc, MD, PhD—newly appointed Aaron Montgomery Ward Professor and Chair of the Ken and Ruth Davee Department of Neurology and Clinical Neurological Sciences—we have an opportunity to launch a vanguard Center for Neurogenetics within the Institute for Translational Neuroscience at Northwestern. Dr. Krainc is a distinguished investigator of international stature whose research has had a transformative impact in the area of neurodegenerative diseases. His studies have helped to uncover the molecular underpinnings of neurodegenerative disease and to provide a foundation for innovative therapeutic development.

The basic idea underlying Dr. Krainc’s research is that by exploring the molecular basis of rare diseases, new molecular targets will be identified for assessment in common diseases. With this research approach, the Center for Neurogenetics will focus on developing targeted treatments for neurodegenerative diseases that affect adults and children, including Alzheimer’s disease, Parkinson’s disease, Amyotrophic Lateral Sclerosis (ALS), Huntington’s disease, and rare lysosomal disorders.

The reality is that conventional drug development for neurodegenerative diseases has been very challenging. A lack of validated and mechanism-based therapeutic targets and biomarkers has been the Achilles’ heel of therapeutic efforts in these diseases. Despite significant investments of public and private funds in Alzheimer’s disease, Parkinson’s disease, ALS, Huntington’s, and other diseases, there are currently no neuroprotective therapies available to patients.

Dr. Krainc’s recent discoveries and those of others have provided a platform for new approaches to therapeutic development for rare and common neurodegenerative diseases. Building on this foundation, he and his colleagues at Northwestern will study rare diseases as a window to understanding neurodegeneration. Through the Center for Neurogenetics, we at Northwestern will initiate and pursue innovative therapeutic approaches that will address some of the previous obstacles in translational neurosciences. The center will focus on rare lysosomal diseases in order to identify specific targets and mechanisms that contribute to neurodegeneration across the lifespan. We expect that such defined targets will facilitate mechanism-based therapeutic development for neurodegenerative disorders.

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Dimitri Krainc, MD, PhD
Lysosomes serve as recycling organelles in neurons. They remove debris and toxic waste that accumulate in neurons during normal neuronal activity. When lysosomes do not work at full capacity (e.g. in neurodegenerative diseases), cells accumulate these toxic products and debris. Dr. Krainc’s goal is to improve lysosomal function and prevent toxic accumulations and the formation of Lewy bodies, plaques, and tangles that are seen in Parkinson’s disease, Alzheimer’s disease, and other neuro-degenerative diseases.

By focusing on rare lysosomal diseases to examine more common neurodegenerative disorders, Northwestern and the Center for Neurogenetics have an unprecedented opportunity to understand the molecular basis of neurodegeneration and identify novel therapeutic targets. In pursuing the development of targeted therapies for Parkinson’s disease, Alzheimer’s disease, and other dementias, we have an opportunity to revolutionize care for patients with neurodegenerative diseases just like what has been and is being done for patients with cancer.

For example, if a patient has Parkinson’s disease that is in part caused by deficient activity of a particular lysosomal enzyme, that patient will be given an activator of the enzyme as the therapy. The concept of matching a drug to a person’s illness is generally referred to as “personalized medicine,” a concept that once seemed futuristic, but that now reflects a growing segment of the treatments in medicine, especially in cancer. The formation of the Center for Neurogenetics will position Northwestern Medicine to become a leader in launching a new wave of personalized treatments for neurodegenerative diseases.

The Center for Neurogenetics will bring together investigators from various fields of expertise within the Northwestern research and clinical community. Laboratories within the Center for Neurogenetics will provide training opportunities for undergraduates, graduate and medical students, and postdoctoral fellows. The robust interfaces and collaborations that occur within the Center for Neurogenetics will undoubtedly lead to clinical advances that benefit patients and their families locally, nationally, and across the globe.
NORTHWESTERN MEDICINE

Northwestern Memorial HealthCare and Northwestern University Feinberg School of Medicine are seeking to impact the health of humankind through Northwestern Medicine. We aspire to be the destinations of choice for people seeking quality healthcare; for those who provide, support, and advance that care through leading-edge treatments and breakthrough discoveries; and for people who share our passion for educating future physicians and scientists. Our commitment to transform healthcare and to be among the nation’s top academic medical centers will be accomplished through innovation and excellence.

The Center for Neurogenetics is an important part of our new Institute for Translational Neuroscience at Northwestern Medicine. The Institute for Translational Neuroscience will empower creative scientists and clinicians to translate a deep and rigorous understanding of disease mechanisms into new preventives, diagnostics, and therapeutics for use in diseases of the nervous system.

As we move forward in creating the Center for Neurogenetics, our utmost priorities are to:

• Conduct innovative discovery research to identify molecular and genetic links between rare and common disorders, focusing on neurodegeneration.

• Promote collaborative efforts across departments, institutions, and pharmaceutical industry and voluntary organizations to develop shared resources for discovery research and clinical trials.

• Develop innovative clinical trials to test targeted therapies on smaller populations of patients.

The Center for Neurogenetics will offer a rich environment for collaboration in the development and testing of novel therapies. At Northwestern, we have a history of utilizing innovative biology as a platform for the development of mechanism-based therapeutic approaches. Our institution has an outstanding clinical trials center and multidisciplinary team members who are leaders in their respective fields.