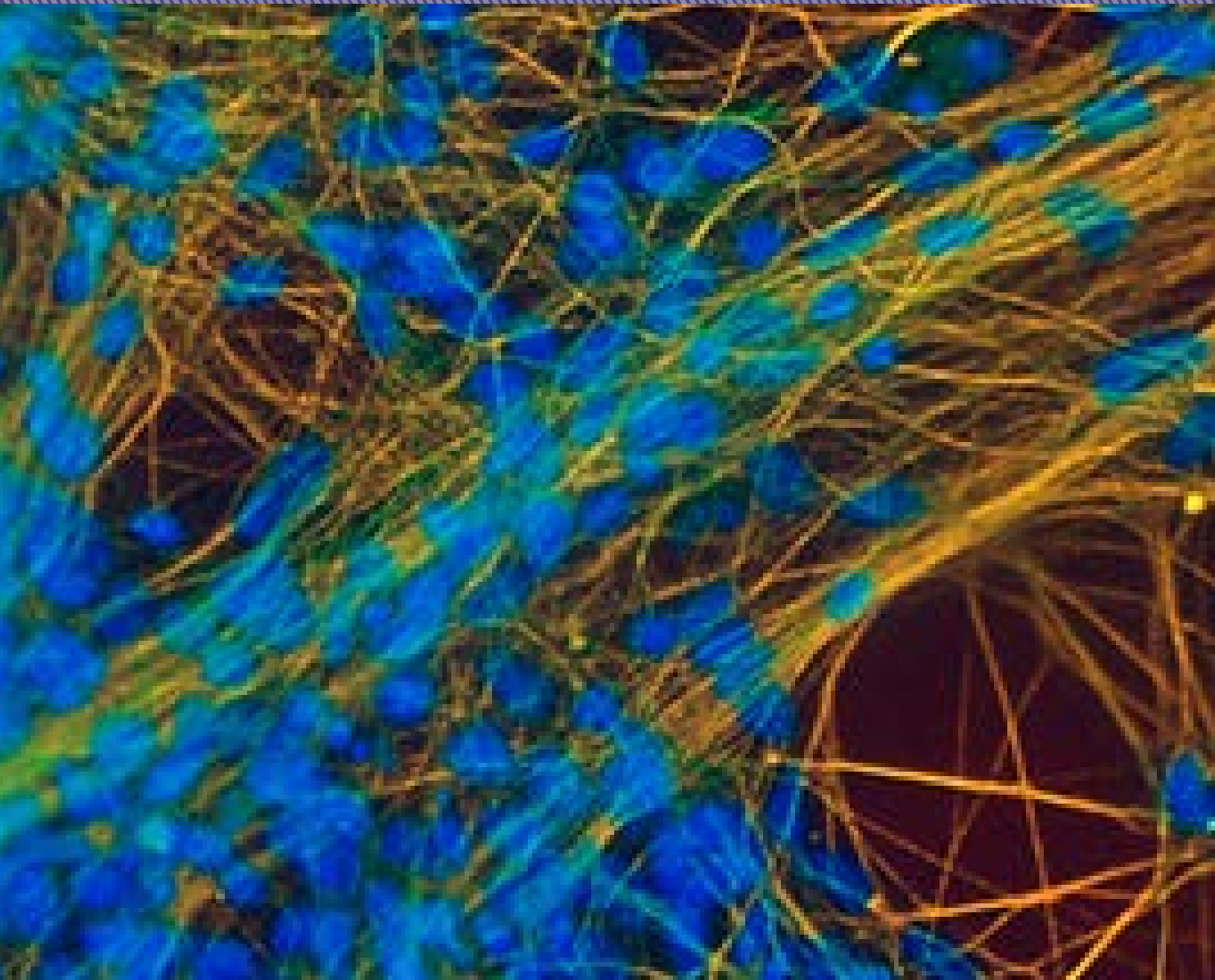


Simpson Querrey Center for Neurogenetics



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Neurodegenerative disorders, marked by the loss of neurons within the brain and/or spinal cord, threaten more than our physical health. They threaten our memories, personality, and the ability to move and speak. These disorders, which are leading causes of disability and death, can present at any age and take a tremendous toll on patients, caregivers, and communities.

Currently, there are no therapies that slow or stop the continued loss of neurons, and no cures 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 and other genetic disorders. The medications that are available today are limited and focus on treating symptoms.

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. Although our understanding of and ability to treat various neurodegenerative disorders have expanded in recent years, there is so much more to learn and discover to benefit and better serve patients and families today and in the future.

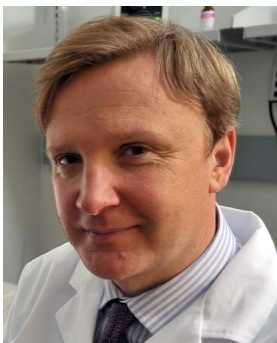
The **Simpson Querrey Center for Neurogenetics** holds exciting promise for the future with a mission to develop targeted treatments for neurological diseases that affect adults and children. The center is directed by Dimitri Krainc, MD, PhD, the Aaron Montgomery Ward Professor and chair of the Ken & Ruth Davee Department of Neurology at Northwestern.

With Dr. Krainc's leadership, the center is bringing together investigators from various fields of expertise within the Northwestern research and clinical community. Laboratories within the Simpson Querrey Center for Neurogenetics also are providing significant training opportunities for undergraduates, graduate and medical students, and postdoctoral fellows. The robust interfaces and collaborations that occur on a daily basis within the center will undoubtedly lead to clinical advances that benefit patients and their families locally, nationally, and across the globe.

The center is coalescing faculty at Northwestern across disciplines to leverage their expertise in the rigorous pursuit of new ideas that can:

- Change the course of therapeutic developments and redefine treatment options;
- Lead the way in translational or laboratory "bench to bedside" research;
- Expand patient care services; and
- Launch innovative clinical trials that provide patients with early access to the most advanced treatments.

At the Simpson Querrey Center for Neurogenetics, Dr. Krainc and colleagues are initiating and pursuing innovative therapeutic approaches to address obstacles in translational neuroscience and accelerate mechanism-based therapeutic development for neurological disorders. In pursuing the development of targeted therapies for Parkinson's disease, as well as dementias, they have an opportunity to revolutionize care for patients with neurodegenerative diseases just like what has been and is being done for patients with cancer.



"In pursuing the development of targeted therapies for Parkinson's disease and other neurodegenerative disorders, including dementias, we have an opportunity to revolutionize care for patients with neurodegenerative diseases."

Dimitri Krainc, MD, PhD

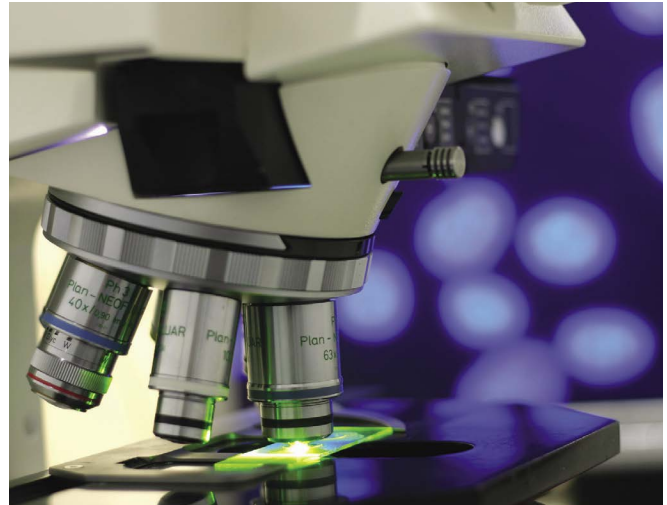
Director, Simpson Querrey Center for Neurogenetics

Aaron Montgomery Ward Professor and Chair, Ken & Ruth Davee Department of Neurology
Professor of Neurology (Movement Disorders), Neurological Surgery, and Neuroscience

For example, if a patient has Parkinson's disease that is in part caused by deficient activity of a particular lysosomal enzyme (e.g. GBA1), 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 treatments in medicine, especially in cancer. The Simpson Querrey Center for Neurogenetics will position Northwestern Medicine to become a leader in launching a new wave of personalized treatments for neurodegenerative diseases.

Dr. Krainc and his team are currently using patient-specific approaches to target illnesses. The team is reprogramming skin cells obtained from patients into patient-specific neurons, and employing genetic sequencing and CRISPR-CAS gene editing technologies to identify cellular pathways that are disrupted in these neurons. Using medicinal chemistry, they can identify small molecule drugs/compounds that can ameliorate these defects, and test efficacy so that these compounds can become treatment options for patients. They also can determine the complete DNA sequence of each individual and use bioinformatic approaches to identify disease-contributing mutations. Overlaying these two types of data enables screening for molecules that slow disease progression.

In addition to these breakthrough efforts, the Simpson Querrey Center for Neurogenetics will open genetic research opportunities augmenting the ongoing effort to identify and meet the translational needs of researchers so they can provide patients and families with the greatest chance for effective treatment.



About Our Director

Prior to joining Northwestern in 2013, Dr. Dimitri Krainc spent more than 20 years at Harvard Medical School, where he completed his research training, followed by a neurology residency and fellowship at Massachusetts General Hospital, and then served with distinction as a member of Harvard's neurology faculty. With Dr. Krainc's compelling leadership, Northwestern's Ken & Ruth Davee Department of Neurology has thrived and is now rated among the top ten departments of neurology in the nation by *U.S. News & World Report* and top five departments nationally in research funding.

Dr. Krainc has led the department's vertical trajectory in neurological care by developing several innovative research, clinical, and translational programs that foster collaboration among physicians, biomedical engineers, and neurology researchers. During his tenure, he has recruited more than 70 new faculty members to the department and has more than doubled the department's competitive National Institutes of Health research funding. Dr. Krainc is the principal founder of *Lysosomal Therapeutics, Inc.* Vanqua Bio, where he also serves as the chair of the Scientific Advisory Board. He also serves on several boards, including at Intellia Therapeutics, Prevail Therapeutics, and OrbiMed Ventures.



Join Us—An Invitation for Your Partnership

At Northwestern University Feinberg School of Medicine, we recognize that every positive contribution we have made to advance medical research and education has been made possible by forward-looking donors and friends who have entrusted us with philanthropic support. We invite interested individuals and groups to join Dr. Krainc and the Simpson Querrey Center for Neurogenetics in their quest to develop targeted treatments for neurological diseases that affect adults and children. With a strong foundation of donor investment, the center will be equipped now and in the future to accelerate the transition from basic findings to treatment, as well as establish specific milestones for future discovery efforts and launch functional working relationships between scientists and industry experts. The Simpson Querrey Center for Neurogenetics will serve as an anchor for generating these opportunities, as well as the structural oversight to ensure sustainability and success.

Philanthropic opportunities to advance this urgent work include establishing a research innovation fund (expendable or endowed) and providing support for fellows and trainees in the center. Gifts to help grow the center's endowment are also incredibly important and impactful. The center's endowment is the engine that drives and supports the daily work of the Simpson Querrey Center for Neurogenetics and its members.

For more information about supporting the Simpson Querrey Center for Neurogenetics, please contact:

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For more information about the Simpson Querrey Center for Neurogenetics, please visit:

<https://www.feinberg.northwestern.edu/sites/crnd/>