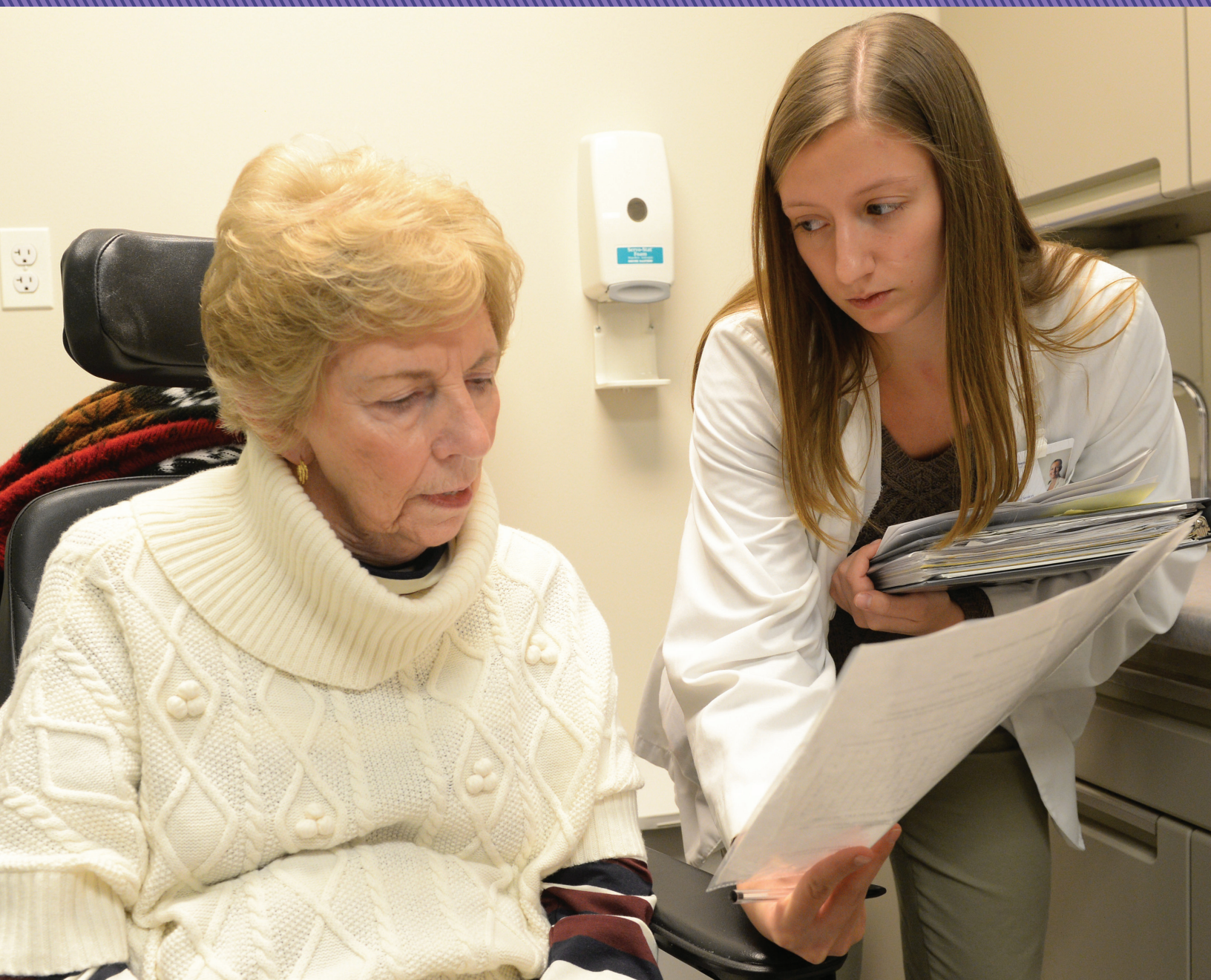
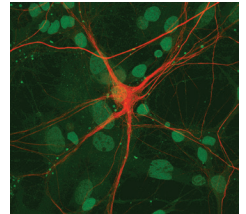


Frances Evelyn Feinberg Clinical Neuroscience Research Institute Les Turner ALS Center



Les Turner ALS Center at Northwestern Medicine

Northwestern University Feinberg School of Medicine is known the world over for leading groundbreaking ALS (amyotrophic lateral sclerosis) research, and for providing exceptional care and support to patients and families living with this complex and devastating disease. At Northwestern, we continue to make important scientific inroads and are championing the way forward to a day without ALS.



ALS, previously known as Lou Gehrig's disease, is a progressive and fatal neurodegenerative disease that often strikes people in the prime of their lives. Although some forms of ALS run in families, most do not. Anyone can be diagnosed with ALS: a mother, a son, a sister, or a grandfather.

Did you know, the lifetime risk of developing ALS is 1 in 300? No two cases of ALS are alike, and no two people living with ALS are exactly alike. The symptoms and progression of the disease can vary greatly. There are an estimated 140,000 new cases of ALS each year worldwide, with an average survival of three to five years. The degeneration of neurons leads to muscle weakness and impaired speaking, swallowing, and breathing, eventually causing paralysis and death. There is no cure.

In 2014, we launched the **Les Turner ALS Center** with the transformational philanthropic support of the Les Turner ALS Foundation—a steadfast partner with Northwestern for more than 40 years. The Les Turner ALS Foundation is Chicagoland's leader in comprehensive ALS care. Our strong union with the Les Turner ALS Foundation has helped our scientists and clinicians to establish themselves as leaders and innovators in ALS research.



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At Northwestern, the Les Turner ALS Center effectively connects the worlds of research and patient support to ensure that the best care is provided and the brightest minds are working to find a cure. Currently, Northwestern and the Les Turner ALS Foundation are jointly leading a major effort to raise \$10 million in funds to fully endow the Center. We are over halfway and are making wonderful progress toward this goal. Our partnership is helping to provide a promising future for those fighting ALS and is accelerating progress toward ending the disease.

A Center Connecting the Worlds of ALS Research and Patient Support

The Les Turner ALS Center brings together all ALS research, the pre-eminent Lois Insolia ALS Clinic, and other ALS research, clinical, and education activities at Northwestern, all under one comprehensive umbrella. It is a part of the Ken and Ruth Davee Department of Neurology and operates within the Frances Evelyn Feinberg Clinical Neuroscience Research Institute at Northwestern Medicine.

Basic Research to Provide Hope for Patients and Families

With the inspiring leadership of Director Robert G. Kalb, MD, the Joan and Paul Rubschlager Professor and chief of the Division of Neuromuscular Disease, the Les Turner ALS Center comprises more than 70 members working across Northwestern University's Chicago and Evanston campuses to generate new knowledge and meaningful research advances.

Our expert scientists and clinicians collaborate with multiple disciplines and areas at Northwestern including neurology, physiology, chemistry, molecular biology, pulmonary medicine, and nanotechnology, among many others. Herein, we share just a few of the innovative and exciting scientific and clinical research directions that are being explored at the Les Turner ALS Center.

Our Center's laboratories have made several groundbreaking discoveries in the field of ALS over the years, including the identification of significant genetic causes of ALS and a more recent major breakthrough in the discovery of a novel gene for typical Parkinson's disease. This has led to the creation of disease models that faithfully recapitulate aspects of the human disease in mice and other experimental platforms. But a disease-modifying treatment has remained elusive. To turn ideas into discoveries, and eventually treatments, investigators at the Les Turner ALS Center are working to define the mechanisms of degeneration in a variety of neurons.



"Scientific research is inherently a risky endeavor. We are exploring uncharted territory and that requires patience and diligence. This can only be achieved by sustained support."

Robert G. Kalb, MD
Joan and Paul Rubschlager Professor and Director, Les Turner ALS Center

Our leading-edge studies of ALS are focusing on:

- Investigations of the genetics of ALS, including targeted gene therapies.
- Studies focusing on using patient-specific induced pluripotent stem cells (iPSC) to generate spinal motor neurons. In using a unique patient-derived stem cell model, our scientists can directly study and manipulate motor neurons with disease. This is a process where our scientists take a blood sample from a patient affected by ALS and “rewind the clock” on these blood cells, turning them into stem cells.
- Research aimed at understanding the cause of degeneration of cortical motor neurons, also known as upper motor neurons, in patients with ALS.
- Studies of the basic mechanisms underpinning ALS using genetically engineered mice, primary neuron cultures, *C. elegans*, and yeast disease models. This is being done to zero in on the fundamental molecular processes that go awry during disease. *C. elegans* are small worms that can teach us a great deal about how human cells work. By giving some of the worms ALS, our scientists can compare activity against control worms without ALS to learn more about how the disease impacts humans.

All of our devoted scientists are committed to bending the arc of disease and finding therapeutic treatments and cures for ALS through innovation and collaboration. They also are deeply committed to educating and mentoring our students, residents, fellows, and other trainees at Northwestern.

ALS Research Grant Program

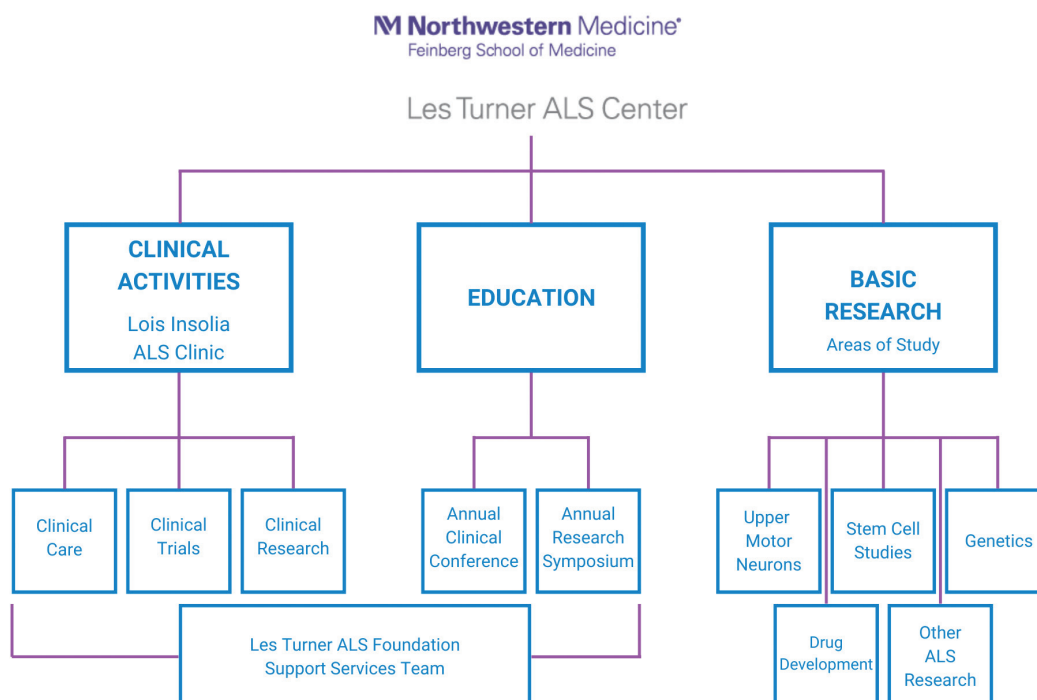
The Les Turner ALS Center sponsors an ALS research grant program and awards grants to innovative ALS pilot research projects. Funds have been awarded to Northwestern scientists in the research areas of basic science, ALS drug development, and genetics. These seed grants allow our researchers to collaborate with scientists around the world on new and novel ideas.

The hope is that successful findings from these studies will enable Northwestern scientists to receive large funding awards from the National Institutes of Health and other external funders. With these funds, they can pursue larger-scale studies in ALS.

Clinical Activities: Lois Insolia ALS Clinic — *Patient Care, Studies, and Support that Make a Difference*

The Les Turner ALS Center and the Les Turner ALS Foundation are proud to bring comprehensive, world-class care and support to people living with ALS in the Chicago area and beyond. Northwestern was recently ranked by *U.S. News & World Report* as the number one program in Illinois and one of the top five hospitals nationwide for neurology and neurosurgery, making it the top-ranked Chicago area hospital in this specialty.

The Lois Insolia ALS Clinic at Northwestern was established in 1986 with continual funding from the James V. Insolia Family Foundation and the Les Turner ALS Foundation—one of the first such clinics in the country, and the first in Chicago. Through the multidisciplinary clinic directed by Senda Ajroud-Driss, MD, associate professor of Neurology, Northwestern specialists are using a team approach to provide comprehensive multidisciplinary treatment.



THE LES TURNER ALS CENTER AT NORTHWESTERN MEDICINE EFFECTIVELY CONNECTS THE WORLDS OF RESEARCH AND PATIENT SUPPORT TO ENSURE THE BEST CARE IS PROVIDED AND THE BRIGHTEST MINDS ARE WORKING TO FIND A CURE.



Under Dr. Driss' direction, physicians staffing this clinic include neurologists Drs. Jonathan Brent, Colin K. Franz, Robert G. Kalb, Teepu Siddique, Robert L. Sufit, and pulmonologists John M. Coleman III and Lisa F. Wolfe.

The clinic is dedicated to the total care and support of people with ALS, their families, and caregivers. During a visit, patients meet with several members of the multidisciplinary team, which includes neurologists, pulmonologists, genetic counselors, respiratory therapists, occupational therapists, and a speech therapist. They also participate in dietetic counseling and coordination of clinical trial studies during visits.

After nearly 35 milestone years, the Lois Insolia ALS Clinic not only provides the pinnacle of care to those with ALS, but also remains the first and largest **fully multidisciplinary ALS clinic in the Chicagoland area**.

Here we share just a few clinical research studies and partnerships that help distinguish us as a global leader and collaborator in the field of ALS study.

Clinical Trials

- **HEALEY ALS Center Platform Trial for ALS:** The Lois Insolia Clinic was selected as a site for the Healey ALS Center Platform Trial for ALS. Platform trials are a new trial design where multiple drugs are tested at the same time, using specialized statistical tools. New regimens (drugs) can be added as they become available, thereby accelerating drug discovery in ALS. The platform remains open long-term until successful cures are found. The trial is expected to begin in 2020.
- **Answer ALS-Individualized Initiative for ALS Discovery:** This study involves the creation of a large repository of induced pluripotent stem cells (iPSC), bio-fluid samples (blood and spinal fluid (optional)), and cell lines for ALS gene identification. These are combined carefully with collected measures of the pattern of the symptoms people with ALS have and how they change over time. This is a collaborative effort between 8 large institutions, including the Lois Insolia ALS Clinic at Northwestern. We enrolled 57 patients for this study that we will be following for 1 year.
- **Effects of Oral Levosimendan (ODM-109) on Respiratory Function in Patients with ALS (REFALS):** This is an international phase 3 clinical trial, which took place in Europe, the United States, and Australia. The clinical study is looking at whether the drug (levosimendan) can improve respiratory functions in patients with ALS. At Northwestern, we have enrolled patients in this study and it is now moving to its open label extension phase when all patients who complete the study will be eligible for the actual drug.



Through the Les Turner ALS Foundation's Support Services Team, individualized resources are available to patients and their families in coordination with care provided at the Lois Insolia ALS Clinic. Services offered include visits with a Support Services Coordinator who provides valuable disease information and who can help with continuity of care; augmentative communication devices or durable medical equipment; support groups throughout the Chicagoland area; a patient resource guide; and other personalized programs. For more information, please contact the Les Turner ALS Foundation at 847-679-3311 or visit lesturnerals.org.

THROUGH NORTHWESTERN MEDICINE, WE ARE CREATING A NATIONAL EPICENTER FOR ALS HEALTHCARE, EDUCATION, RESEARCH, COMMUNITY SERVICE, AND ADVOCACY

Join Us in our Work to Create a World Free of ALS

Northwestern Memorial HealthCare and Northwestern University Feinberg School of Medicine are seeking to impact the health of humankind through Northwestern Medicine. Through the Les Turner ALS Center at Northwestern Medicine, we are enhancing the care provided to people with ALS today and in the future and furthering scientists' understanding of this very challenging disease.

Each day, we recognize that every positive contribution we have made to ALS discovery, care, training, and outreach has been made possible by donors who have continued to entrust us with their philanthropic support. We invite interested friends to join us in advancing the mission of our center through gifts of outright support and endowment.