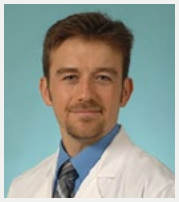
ALS CENTER RESEARCH UPDATE

Spring/Summer 2026

WashU Medicine

ALS Center



FROM THE DIRECTORS

Amyotrophic lateral sclerosis, also known as ALS or Lou Gehrig's disease, is a devastating, debilitating disease. Here at the ALS Center, we have a team of medical professionals who specialize in ALS care and provide patients with the best clinical support available. At the same time, we have research teams who work in the lab to better understand the disease and run clinical trials to test promising new therapies.

In this issue, we highlight the importance of our partners in ALS research. Some of our most important partners are the people who participate in clinical studies who donate blood, cerebrospinal fluid (CSF), and other samples or who voluntarily test new medications. Each and every study visit and sample brings us one step closer to better understanding the disease and how to stop it. Other key partners include our scientific and medical colleagues here at WashU and at other universities and clinical sites, together with whom we share data, insights, and ideas. And finally, we acknowledge the devoted group of friends and families who support our Center with financial contributions – we share the story of one such partner, the Lyle Rakers Charitable Foundation and how they are helping make the most cutting-edge research possible.

Thank you to all of our partners in ALS research.

Timothy Miller, MD, PhD and
Robert C. Bucelli, MD, PhD

PARTNERING FOR ALS RESEARCH: THE LYLE RAKERS CHARITABLE FOUNDATION

Since 2018, the Lyle Rakers Charitable Foundation has united friends, families, and communities around a shared goal: supporting the work of the Miller Lab at Washington University in St. Louis to help accelerate progress toward treatments—and ultimately a cure—for amyotrophic lateral sclerosis (ALS).

The foundation was established following Lyle Rakers' ALS diagnosis. In response, Lyle's longtime friend and business partner, Jim Rable, created the Lyle Rakers Charitable Foundation with a clear purpose: to raise funds dedicated to ALS research. What began as a response to a single ALS diagnosis has now grown into a community movement that has to date raised more than $1 million for ALS research at Washington University.

Since its creation, the foundation has mobilized community members, local businesses, and volunteers through a variety of fundraising events. All funds raised are donated directly to the Miller Lab to support the important research taking place there. Central to the foundation's fundraising efforts are two annual golf tournaments - one in September and one in October. These events have become the cornerstone of the foundation's activities, drawing participants from across the region who gather not only to enjoy a day on the course, but also contribute to a meaningful cause. A key partner in these events is the organization Tee'd Off for ALS. Their leadership and involvement have helped strengthen the reach and impact of these events while bringing additional supporters into the effort to raise funds for ALS research.

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NEWS ROUNDUP

Mark your calendars! Our **2026 ALS Symposium** will take place Saturday, July 18 at the Eric P. Newman Education Center (EPNEC) on the WashU Medical Campus. There will also be a livestream option for those unable to attend in person. Please register by **June 30, 2026** by scanning the QR code or visit **bit.ly/495sX6V**.

Congratulations to Dr. Timothy Miller, who was awarded the 2026 Potamkin Prize. One of the most prestigious prizes in Alzheimer's research, the Potamkin Prize recognizes the achievements of scientific researchers who do innovative work, find groundbreaking discoveries, and push forward the field of study in Pick's, Alzheimer's, and related brain degenerative diseases.

Dr. Robert Bucelli received the Dr. Neville Grant Award for Clinical Excellence, one of Barnes-Jewish Hospital's highest honors. The award is presented annually to a member of the attending medical staff who is an exceptional clinician, offers tremendous compassion to patients and families, shows great respect to colleagues and other caregivers, and masterfully mentors students, residents and fellows.

Dr. Joshua Newman published an article in Touch Neurology titled, "SOD1-ALS from Gene Discovery to Targeted Therapeutics: A Comprehensive Review." The review discusses the relationship of SOD1 mutations and familial ALS and the early success of tofersen.

Clinical research team members **Timi Olugbenro, Grace Rogers, and Kelly McCoy Gross** recently traveled to Nashville, TN for the HEALEY Platform Trial Investigator Meeting, learning about new and upcoming treatment regimens and networking with other ALS researchers from around the country.

A new study from **Dr. Sean Smith published in Muscle & Nerve** suggests that integrating serum neurofilament (NfL) into current diagnostic frameworks has the potential to increase diagnostic certainty and improve early identification of ALS.

Kelly McCoy Gross, RN, presented at the 2026 MDA Conference in Orlando, FL alongside Dr. Elyse Everett (Ohio State, formerly WashU) and Dr. Cara Siegel (UCLA). Their presentations focused on palliative and mental health care in ALS throughout the entire course of the disease, from diagnosis to end-of-life care.

Visit our Linktree page with articles & podcasts: https://linktr.ee/alscenterwashu

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Beyond the golf tournaments, the foundation has added additional events that continue to build awareness and support throughout the year. One of the most impactful of these is Hoops for Hope, a basketball fundraiser held at Parkway West High School and led by Parkway West basketball coach John Wright. The event brings together students and families for an evening centered on athletics, community, and philanthropy. Hoops for Hope has become a special event on the foundation's calendar while also introducing younger generations to the importance of supporting ALS research. A couple of other events include Levi's for Lyle and a monthly drawing held at the Aviston Legion.

At the heart of the foundation's work is Lyle himself. Although now wheelchair-bound and dependent on noninvasive ventilation, Lyle continues to live life to the fullest. With the unwavering support of his wife, Sarah—who has mastered the daily challenges that come with living alongside ALS—the family remains active and engaged. Together they travel frequently to support their daughter, Laurel, as she plays college volleyball and their son, Carson, as he competes in college football. They have also continued to enjoy traveling with friends and family, refusing to let ALS define or limit the experiences they share.

The Rakers Family

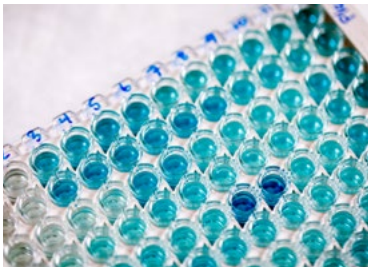
Dr. Tim Miller and Jim Rable

The determination shown by Lyle and his family continues to inspire the many friends, volunteers, and supporters who make the foundation's work possible. Those who wish to support the mission of the Lyle Rakers Charitable Foundation can make a donation by visiting www.lylerakers.com or by finding the organization on Facebook at "Lyle Rakers Charitable Foundation."

"We are deeply grateful for the work being done by Dr. Tim Miller and the entire Miller Lab at Washington University. Their dedication to advancing the understanding and treatment of ALS provides hope to patients and families affected by this disease. It is an honor for the Lyle Rakers Charitable Foundation and its supporters to contribute to the important research taking place at the Miller Lab, and we remain committed to continuing this partnership in the years ahead." – **Jim Rable**, *Founder of Lyle Rakers Charitable Foundation*

GIFT FUNDS SPOTLIGHT: LAB EQUIPMENT

Long before a drug goes to clinical trial, many hours have been spent in the lab studying the disease at a cellular level. Much of this work is funded by grants, which come from public health agencies like the NIH or from private organizations that support ALS research. Dr. Miller and his team regularly seek grants, but donations from ALS Center partners like the Lyle Rakers Charitable Foundation also play an important role in the success of the lab. Gift funds allow the lab to pursue bold new ideas that generate the preliminary data needed for grant applications. Gift funds also cover items that sometimes grants don't, like special lab equipment, which enables faster, more precise work. Two examples of such equipment purchased with the help of gift funds from the Lyle Rakers Charitable Foundation are the plate reader and ultralow freezer.



When studying ALS in the lab, experiments often rely on the detection of chemical reactions not always visible to the naked eye. A piece of equipment called a **plate reader** makes this detection possible and at speeds that greatly increase the number of experiments we can do at one time. A small tray or "plate" containing, in our case, 96 microwells holds individual liquid samples that are scanned by the plate reader. The types of chemical reactions we are measuring in the microwells can vary, but one type involves a luciferase enzyme (the same one that produces light in fireflies) to help us evaluate new drug treatments for ALS. In this type of drug screen, we can link luciferase to a known gene or protein in the disease pathway and then add different drugs to the plate microwells. The plate reader measures the levels of light produced when luciferase is active as a marker for how well the drug in each microwell worked. This plate reader, which bears a plaque acknowledging the Lyle Rakers Foundation, has greatly helped increase the speed and breadth of experiments performed in the Miller lab.

Clinical research participants contribute blood, CSF, skin, or other tissue samples to research, which are then studied in the lab. If not stored properly, these biological materials degrade over time and become unusable. Proper storage requires freezing them, but not just at 0°F like a typical kitchen freezer – they must be kept in a **special freezer** at an ultralow temperature, around -112°F (-80°C). At this temperature, DNA, RNA and proteins remain stable for a longer time, thus preserving their use for research. Thanks to the Lyle Rakers Foundation, the Miller Lab was able to purchase two new ultralow freezers this past year that will safely store these materials, which hold crucial information about ALS.

NEW TOFERSEN DATA PUBLISHED IN JAMA NEUROLOGY

New data on the long-term use of tofersen, published Dec. 22 in JAMA Neurology, suggest that over about three years of treatment, roughly one-quarter of participants in one study group experienced stabilization of symptoms and even functional improvement in grip strength and respiratory function.

Tofersen is designed to treat ALS caused by variants in a gene called SOD1, which accounts for about 2% of ALS cases. Earlier results of the phase 3 trial showed the drug reduced neurodegeneration and prompted the FDA to approve the drug in 2023 under an accelerated approval pathway. The drug, designed specifically for this type of ALS and based in part on research conducted at WashU Medicine, blocks production of the mutated SOD1 protein.

When compared with historical knowledge of ALS disease progression, the improvement in some patients receiving tofersen is life-changing, according to Drs. Timothy Miller and Robert Bucelli, co-directors of the ALS Center.

"There's variability in patient response to tofersen — it's not a panacea for everyone," Bucelli said. "But for those patients who do have a substantial response, the fact that they're able to maintain the independence they had when they went on the drug is a miracle."

"Stopping disease progression and making improvements over three to five years is unheard of in this type of ALS," said Dr. Miller. "Tofersen shows benefits compared with what we expect to see for these participants, with about 25% of participants experiencing improvement. These results provide hope that we can change the trajectory of this devastating disease, and we are optimistic we can do the same for other forms of ALS."

Please visit our **linktree page** to read the full press release and to watch the trailer for **"Another Tomorrow"**, an upcoming short film about the impact of tofersen.

WASHU PARTICIPATING IN NEW HEALEY PLATFORM REGIMEN

The WashU ALS Center recently began enrolling participants in the latest regimen of the HEALEY ALS Platform Trial. The regimen will evaluate NUZ-001, an oral therapy that targets key pathological mechanisms in ALS, including TDP-43 protein aggregation and impaired autophagy, which are central features across multiple neurodegenerative diseases. Initial Phase 1 studies support favorable safety and pharmacokinetics of the drug. NUZ-001 is developed by Neurizon Therapeutics Limited, a biotechnology company based in Melbourne, Australia, and is not yet approved for use in any country.

The HEALEY ALS Platform Trial is a multicenter, double-blind, placebo-controlled, adaptive trial for amyotrophic lateral sclerosis (ALS) created in partnership with the Network of Excellence for ALS (NEALS). The goal of the HEALEY ALS Platform trial is to accelerate the development of potential new ALS therapies. The trial tests and evaluates multiple investigational drugs simultaneously, shares infrastructure across trial sites, and improves start up and enrollment efficiencies, allowing for fast results.

If you would like to learn more about study participation, please contact our clinical research team at **als@wustl.edu** or 1-844-ALS-CARE.

MAKE A DIFFERENCE

Your thoughtful contribution enables us to continue researching possible treatments and ultimately cures for this devastating disease. **alscenter.wustl.edu/donate/**

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