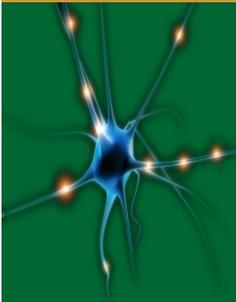


Winter,  
2017-2018



WASHINGTON UNIVERSITY DEPARTMENT OF NEUROLOGY

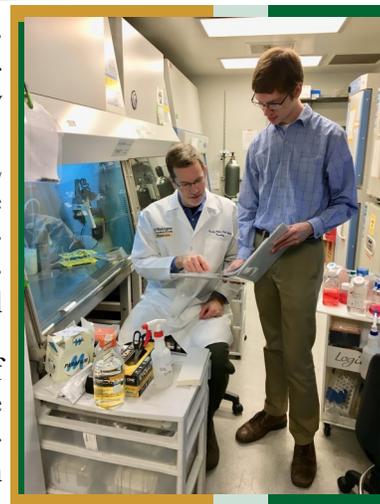
# ALS Research Update

## Answer ALS will help identify drug targets

The Answer ALS research project is the largest coordinated scientific research ever proposed for ALS, with Dr. Timothy Miller leading the efforts at the Washington University School of Medicine site. The study originated at the 2013 Team Gleason Summit, an event headed by former NFL player Steve Gleason, who lives with ALS and has become heavily involved in ALS advocacy. The funding is coordinated through the ALS Finding a Cure project and the Robert Packard Center for ALS research and other committed donors.

Answer ALS involves collecting many different types of data from patients to get a comprehensive picture of how the disease works. This includes everything from standard clinical measures such as strength testing and breathing function to innovative techniques examining genomics and cell biology. A particularly unique part of the study involves collection of blood for creation of iPSCs (induced pluripotent stem cells). These are cells created from adult blood samples and then converted into motor neurons enabling further analysis of basic ALS disease processes. All of this will lead to a better understanding of ALS and identification of future drug targets.

Answer ALS recently celebrated enrollment of its 600<sup>th</sup> subject and is well on its way toward the goal of 1,000 participants. The study is also enrolling healthy volunteers for comparison purposes, enabling more individuals to contribute to ALS research. There is still time to get involved at Washington University. Anyone interested in participating should email [neuroclinicalstudies@email.wustl.edu](mailto:neuroclinicalstudies@email.wustl.edu) or call 314-362-6159. Study visits can be conducted at regular clinic appointments or at any other scheduled time.



*Timothy Miller, MD, PhD, and Jesse Markway, Coordinator for Answer ALS, review data from a newly enrolled participant.*



## Phase 2 CENTAUR Trial Opens for Enrollment

The Miller Lab is excited to announce a new interventional drug trial for the treatment of ALS. The CENTAUR Trial is a phase 2 randomized control trial that will test the efficacy and safety of AMX0035. AMX0035 is a combination therapy designed to reduce neuronal death through blockade of key cellular death pathways originating in the mitochondria and endoplasmic reticulum.

The CENTAUR trial will enroll 132 participants at twenty-five sites nationwide. Participants will be evaluated monthly to measure their breathing, strength and ALS functional rating scale scores. Strength will be measured using a new device called the Accurate Test of Limb Isometric Strength (ATLIS). The ATLIS has been shown to provide a more reliable measure of ALS disease progression than other measurements currently being used in ALS clinical trials. This device may help researchers more accurately determine if the study drug is slowing disease progression based on strength measurements. Participants must be within 18 months of symptom onset and have ALS symptoms in 3 body regions. By studying similar patients it may enhance results to better determine if the drug is having an effect on disease progression. The hope is that this innovative trial design will not only be able to definitively determine if AMX0035 is safe and effective for the treatment of ALS but will also positively impact the design of future ALS drug trials.

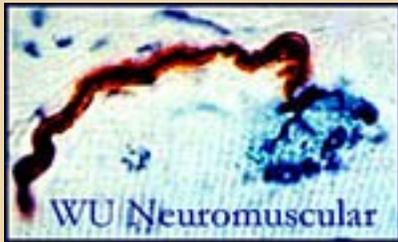
For additional information about this trial and the study drug please visit <https://www.neals.org/for-people-with-als-caregivers/educational-webinars/the-centaur-trial-a-phase-2-trial-of-amx0035-for-the-treatment-of-als>. If you are interested in participating in this trial at Washington University, email [neuroclinicalstudies@neuro.wustl.edu](mailto:neuroclinicalstudies@neuro.wustl.edu).

## Donor Spotlight

Evelyn “Ev” Luecke lost a loved one to ALS and along with her family grappled to cope with the magnitude of her loss. What Ev found was a deep resilience fashioned from thirty-eight years in education. She spent fifteen years working in American schools overseas and twenty-three years working in Missouri schools helping students with disabilities find their own resolve to unleash their potential. It is fitting that Ev’s reaction to losing a loved one to ALS was to ask how she could help the many families who have struggled or continue to struggle with the effects of this devastating disease.

What happened next was kismet as a simple internet search introduced Ev to the Miller Laboratory. Ev contacted Dr. Miller, familiarized herself with his work, and soon realized she knew how she wanted to make a difference. She would invest in research, training, and education for ALS in the Miller Lab. Ev now makes annual contributions to the Miller Lab that provide a much needed infusion of support for important priorities that require funding outside of the Lab’s current resources. In addition, Ev has arranged to provide for this purpose beyond her lifetime through an estate commitment.

Ev is an exemplar of the difference one person can make through philanthropy. The Miller Lab is grateful that Ev asked herself the powerful question of how she can ensure that what is not imaginable today is possible tomorrow. Generations of scientists will make advances that improve the lives of many because of the vision of this wonderful friend of the Miller Lab.



<http://millerlab.wustl.edu/>  
<http://neuromuscular.wustl.edu/>

*We gratefully acknowledge the support of the following organizations:*

Hope Center for Neurological Disorders

Project 5 for ALS

Muscular Dystrophy Association

The ALS Association

NEALS - Northeast ALS Consortium

National Institutes of Health

Robert Packard Center for ALS

Target ALS

University of Missouri Spinal Cord Injury and Disease Research Program

Ionis Pharmaceuticals

BioGen Idec MA Inc.

**The MDA Walk is just around the corner at a new location! Mark your calendar for Sunday, April 29th, 10:00am at Tower Grove Park.**

## *How can you help The Miller Lab?*

### *Charitable donations support ALS research*

For contributions to the Washington University ALS program, please contact Zach Silvers, Senior Director of Development, at 314-935-3498 or email [zsilvers@wustl.edu](mailto:zsilvers@wustl.edu). Those who wish to send a check should write it payable to Washington University. In the memo section, please indicate the gift is to “ALS Research Support Fund.”

Checks should be sent to:

Attn: Zach Silvers, Washington University, CB 1247  
7425 Forsyth Blvd.  
St. Louis, MO 63105



*Staff from the Washington University School of Medicine Department of Neurology teamed up at Forest Park on June 24th, 2017 to support the Walk to Defeat ALS.® This year’s Walk is Saturday, June 23rd at Forest Park.*

*Support area Walks to raise awareness about ALS!*