

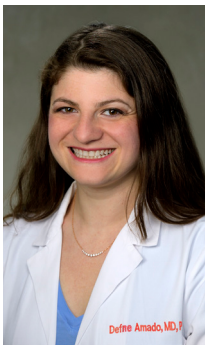


SPRING 2025

“Your Generous Donations Hard At Work”

by Dr. Defne Amado,

2024 recipient of the Suzanne Hiyama Ross Award for Research



Dr. Defne Amado

I cannot express enough gratitude to the donors who make our work possible, and to the Scientific Advisory Board at Active Against ALS who found our work worthy of sponsoring.

I met my first person living with ALS during Neurology residency. She was a young mother of a six-year-old daughter and communicated with an eye gaze device from her wheelchair. She had a tattoo on each wrist, one that said “Faith” and the other “Hope”: “Faith that a cure will be found; hope that it will be within my lifetime.” I had wondered, after earning my MD/PhD, how I would identify the disease I would want to work on for the rest of my career. That day I stopped wondering: ALS was that disease, and it had to go.

A decade later, I am a physician-scientist who cares for people living with ALS and runs a lab focused on developing gene therapy-based approaches to treat them. My scientific training has included a PhD in neuroscience and gene therapy; a postdoctoral fellowship to study disease biomarkers and develop a gene delivery strategy for frontotemporal dementia; and an additional postdoctoral fellowship to learn how to a) reduce toxic proteins in neurodegeneration and b) direct therapies to affected regions of the central nervous system (CNS). I use all of this training in my lab today, where we use gene therapy approaches to better understand ALS and develop effective treatments that

reach the brain and spinal cord regions affected by the disease.

Our most promising work targets the main pathology in ALS, the mislocalization and accumulation of a protein called TDP-43. We achieve this by reducing Ataxin-2 (ATXN2), a key contributor to TDP-43 aggregation, to restore normal TDP-43 location and function. A prior clinical trial using an antisense oligonucleotide-based strategy was unsuccessful, likely due to insufficient reduction of ATXN2 to have an effect. In collaboration with Dr. Beverly Davidson, my lab uses viruses engineered for very strong CNS targeting to deliver our treatment, which we believe stands a better chance of clinical success. In a mouse model of sporadic ALS, this treatment extended lifespan by 50%, and improved strength, motor neuron counts, and inflammation.

To translate this work to humans, we are pursuing two important studies: 1. Testing the therapy in an additional, genetic mouse model to ensure that the benefits seen are not exclusive to our current model, and to see if the therapy will work in genetic ALS; and 2. Understanding the effects in humans by studying motor neurons derived from patients who donated their stem cells for study.

Upcoming Events

Michigan Madness

April 15 & 17



The Phi Delta Theta chapter at U-M is hosting a Michigan Madness basketball tournament to

raise funds for ALS. [Click here](#) to support your favorite team! Join us and cheer on the teams!

April 15, 4:00-7:00 pm
April 17, 4:00-7:00 pm

Michigan Coliseum,
721 S. Fifth Avenue, A2

Twinkie Run

Sunday, May 4

9:00 a.m. at Gallup Park

The Twinkie® Run is back! Run or walk this fun 5K race while enjoying Twinkies®! [Click here to register or donate](#). Kids under 12 run free in honor of Coach Bob.



Rivalry Ride

September 14

Save the date for our 14th annual Rivalry Ride bike ride from Spartan Stadium in East Lansing to the Big House in Ann Arbor. [Click here to register or donate](#).





“Your Generous Donations Hard at Work” (cont’d)

This important award from Active Against ALS is critical in enabling us to bring this therapy closer to the clinic, particularly in light of ongoing threats to federal funding. The support goes to research staff salaries, covering the costs of animal and cell maintenance, and paying for the reagents and equipment needed to conduct these experiments. If successful, our studies will enable a single-dose, targeted, lasting treatment for ALS, irrespective of whether or not that person carries a genetic mutation.

To each patient I see in my clinic, I make a silent promise that I won’t stop fighting for a cure. Each member of my small, dedicated lab team has shadowed me in clinic to know who we are fighting for, and we carry the patients who are living and those whom we’ve lost in our thoughts as we discuss our findings and plan our next experiments. I cannot express enough gratitude to the donors who make our work possible, and to the Scientific Advisory Board at Active Against ALS who found our work worthy of sponsoring. I have faith that a cure for ALS will be found, and I have hope that it will be within the lifetime of each person I meet who is fighting this disease.

Events in May for ALS Awareness Month

Twinkie Run – May 4

Help us raise money for ALS research while eating Twinkies®! Join us for a [5K Run/Walk](#) through Gallup Park in Ann Arbor on Sunday, May 4 at 9:00 am. The Twinkie Run has been one of our cornerstone events since 2009.



Participation is limited to the first 250 registrations. In honor of Coach Bob, Kids 12 and under run free. Festive attire and costumes are welcome!

Now through April 14:

Early bird registration is \$25 for adults 13 and over. T-shirts can be ordered as well

through April 14. We will have limited quantities available for purchase on race day so make sure you order yours today!

April 15-May 3: registration is \$35
Race day registration is \$45

[Click here to register or donate](#) to the Twinkie® Run!

If you are interested in [Volunteering](#) at the Twinkie® Run, please [click here!](#)

Libraries and chalking

During the month of May, libraries throughout the area have agreed to create displays with information about ALS, the work of our organization, and books about the disease and the impact it has on family, friends and caregivers. Check out the displays in these participating libraries: Ann Arbor, Milan, Chelsea, Saline, Tecumseh and Ypsilanti. Folks can also find books about ALS in neighborhood Little Free Libraries throughout A2 and surrounding communities. Look for ALS ribbons on Main Street in Ann Arbor as well as informational flyers, bookmarks and table tents in local businesses. And as always, don’t forget to “chalk the walk” – an opportunity for folks of all ages to raise awareness of ALS in fun, colorful and creative ways.



Michigan Madness

April 15 & 17



The Phi Delta Theta Fraternity at the University of Michigan is holding a [Michigan](#)

[Madness](#) 3v3 basketball tournament to raise money for ALS Research! Collegiate teams can register to play in this tournament which will be held across two days: April 15 and April 17, both from 4:00pm-7:00pm at the Coliseum at 721 S. Fifth Ave. in Ann Arbor. Cost of entry is \$120 per team. The more money you raise, the higher seeded you will be in the tournament!

We encourage all of you to support your favorite team with a [donation](#) and come cheer on the players!

Rivalry Ride 2025

September 14



The date has been set for our 14th annual Rivalry Ride. Join the group as we ride from Spartan Stadium in East Lansing to the Big House in Ann Arbor on September 14. Last year, we had over 25 riders complete the 62 mile course. For more information, or to register, [click here](#). Not a bike rider? You can still help out by making a [donation here](#).





TO EVERYONE WHO HAS DONATED, VOLUNTEERED, OR SPONSORED AN EVENT OR PLANNED AN EVENT OF YOUR OWN. WE HAVE NOW DONATED **\$1,036,731** FOR ALS RESEARCH!

Thank You Thank You Thank You

We would like to thank our outgoing Board Members who left our board in November.

Elisabeth Weinstein has been a board member since 2017. She has served as our Scientific Advisory Board liaison, overseeing our process of evaluating grant submissions and selecting awardees. Although Elisabeth is no longer a board member, she will continue to help us with our grant process and awards. Thanks so much for all of your help and dedication to the cause!

Laura Stidham also stepped

off the Board this past November. Laura was a founding board member, serving from 2008 to 2017 in various roles. She rejoined in 2019 and has served as Vice-Chair, sat on our Finance Committee, and was a significant help with our budgeting and financial functions. She was also active in the planning of our summer Youth Swimathons and volunteered at almost every event! Laura will be missed, but we have a feeling we will still continue to see her smiling face at events in the upcoming years.

ALS Research at Risk

You may be hearing about freezes on federal grants and how it is impacting important medical research. The ALS Association has posted a summary of what is happening, including action you can take if you desire. [Click here](#) to read more about possible impacts on ALS research and to make your voice heard.



Grocery donations

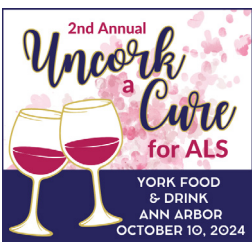
As a reminder, when you shop at Kroger, they donate a portion of your payment to Active Against ALS. Just set up your Kroger Plus Account and within your account, select Active Against ALS as your Community Rewards recipient. Just by shopping at Kroger, Active Against ALS reaps the benefits!



Want to get involved?

We are always looking for energetic and dedicated people to join our board and get more involved. The board meets just once monthly and works on approximately 4-5 events per year. The time commitment to join the board can be flexible with your busy schedule. Please email us at info.activeagainstals@gmail.com if you are interested in joining [our board](#) or simply volunteering for one of our events!

Uncork a Cure 2024 Recap



Our 2nd Annual Uncork a Cure for ALS was held on October 10, 2024. More than 140 people attended the event, where we netted more than \$21,000 for ALS research! Past board member Kathy Siler and her granddaughter Elizabeth Stidham spoke about the impact that ALS has had on their family - they have lost six members to ALS over three generations. Dr. Sami Barmada, an ALS researcher at U-M and a member of our Scientific Advisory Board, then described recent

breakthroughs in ALS research. Participants enjoyed tasting four different wines (and a couple of beers), delicious food from York and Zingerman's and listening and dancing to music by Four Roses.

Many thanks to the event's major sponsors - Bank of Ann Arbor, York and Zingerman's. The online auction, with more than 60 items donated by local businesses and individuals, raised more than \$7,000. For a full list of our sponsors and donors, please [click here](#). As always, the event was the result of an amazing team effort, including committee members and volunteers at the event from Phi Delta Theta, Community High School, and Pioneer High School!

