Fulton ALS Center Newsletter



Gregory W. Fulton ALS Center

Summer 2020

Inside this issue:

Patient Highlight: Scott Keniston

Clinical Research Study Updates

Clinical Research Updates: HEALEY ALS Platform Trial

Research Staff Updates

Nurses Week

ALS Advocacy Update

ALS Basic Science Research During a Pandemic

ALS Association Support Groups

MDS ALS Golf Classic

Find Us on Facebook!

Support the Fulton Center

The Great LiveLikeLou Outdoor Clean-Up for ALS Families



Patient Highlight: Scott Keniston

Meet Scott Keniston

I am 51 years old. I was born in Redwood City, California and have been a San Francisco 49ers fan my whole life. I moved to Paradise Valley, Arizona when I was in grade school. I grew up playing soccer, but started playing football in high school at Phoenix Christian High School. We were State Champions in 1984 and 1985. I have been married to my high school sweetheart, Patti, since 1988. We have two children: Aaron, 30, who lives in Hayward, California with his wife, Caitlin. Kristen, 28, lives here in Phoenix and is married to Cole. She just gave us our first grandchild last December, Ryker Scott. I have always been active and continued to play and coach soccer all my life until recently. I love to bass fish, race cars, and I also used to mountain bike.



ALS Diagnosis

I went to see an orthopedic PA who happened to be a friend of ours. I had a lump on the back of my right foot that was bothering me and decided it needed to be looked at. The bump ended up just being bursitis/tendonitis. While I was there I mentioned I had "tweaked" my left knee a few months before and it was still bothering me. He did x-rays and found signs of arthritis, which is pretty common for an almost 50-year old that had played hard all his life. I used a cloth knee brace for a bit to see if that would help but the pain never went away. A few different MRIs (knee and back) were done to see if there was anything causing the pain and they came out clear. I also tried doing some injections but those didn't work either. At one appointment I made a comment about my flip flop flying off of my foot when I walked. That is when the doctor realized I had foot drop. The doctor immediately sent me to a spine specialist to have him perform an EMG. Patient Highlight: Scott Keniston continued from page 1

When the spine specialist was examining me he saw some other symptoms and felt it would be better to have me examined and EMG done by a neurologist along with a brain MRI. About a month later I saw Dr. Ladha at BNI. He did an examination, blood work, and another MRI scan. On May 10, 2018, Dr Ladha did my EMG. All my other tests had come back negative. Those results along with an abnormal EMG gave me a diagnosis of ALS. I was very blessed to have the right doctors at the right time. My diagnosis, after noticing the foot drop, came within a few months. Some people aren't diagnosed for years.

ALS Research

After Dr Ladha said "You have ALS. Do you have any questions?", my first question was "How can we help?" We, my family members included, were willing to help in assisting to find a cure in any way we could. So far the only clinical research we have been involved with was getting MRIs done of my legs and my wife's legs. They are trying to compare ALS legs to healthy legs in hopes of using the results of a leg MRI as a biomarker for ALS. Ideally, they want to compare legs that are about the same age. My wife and I were born six days apart so we were a good match. The process for both of us took an afternoon. The staff were great. They were friendly and explained everything going on and showed us what they were looking for.

ALS Community and Advocacy Efforts

We have participated in the Walk to Defeat ALS for the last two years. Last year, we along with our friends and family, had a golf tournament that raised over \$18,000 for

the ALS Arizona Chapter (donated through our walk team Keniston's ALStars). We attended Bite Nite last year and my wife and I are on the Bite Nite committee this year. I have attended many activities that the ALS Association has provided including adaptive fishing, golf, water sports etc. I was blessed to be asked to represent the MDA and throw out the first pitch on ALS Awareness Night at the Arizona Diamondbacks' game last summer. So much fun!

I was hoping to go to Washington DC for ALS Advocacy both last year and this year. Last year I had my knee replaced the month before and this year....well. Hopefully we can go next year. My wife and I were blessed to be a part of a group of ALS patients and caregivers that met with Senator Martha McSally in February. We were able to discuss funding, insurance issues, caregiver support, needs and frustrations with her. I feel we made a huge impact on her.

Advice for pALS and cALS

I think the best thing I have done is getting involved! You have no control over this disease. I feel like when we are actively involved in fundraising, meeting other ALS families, and advocating for ALS, we are at least doing SOMETHING. I came back from my first ALS event, adaptive fishing, and told my wife I don't think I had ever met a happier, more friendly group of people. At first you might think you don't want to go to these events. Who really wants to see what is coming? But once you meet these fighters and see how happy and what great attitudes they have, I think you feel like things are going to be okay.

Clinical Research Study Updates

If you have any questions about participating in clinical research at the Fulton Center, please visit our website at:

<u>www.barrowneuro.org/</u> <u>patients-families/find-a-</u> clinical-trial

Biogen 261AS101

Biogen 261AS101 is a Phase 1 study to evaluate the safety and tolerability of BIIB100 in ALS patients. The Fulton Center is currently enrolling patients.

BIO 3

BIO 3 is a biomarker study which aims to look at different features of ALS by using standard outcomes measures, but also utilizing cognitive assessment and biofluid collection during an 18month period. This study will also help expand our biorepository. The Fulton Center is currently enrolling patients.

Biogen 233AS101 Part C

Biogen 233AS101 Part C is the 3rd part of this study, which will be the fixed dose of BIIB067. To be eligible to screen for this study, you must have familial ALS with an SOD-1 mutation. This study is currently enrolling patients at the Fulton Center. *Clinical Research Study Updates continued from page 2*

Speech Analysis

Speech Analysis is an observational study that will be looking at the use of speech and language measures to detect cognitive changes in patients with ALS. This study is currently enrolling at the Fulton Center.

ALS Post-Mortem Tissue Bank

The ALS Post-Mortem Tissue Bank collects postmortem tissue (spinal cord, muscle, and brain tissue) from both people with ALS and people without ALS. This tissue bank will support ALS research efforts by strengthening collaborations in the research community and providing information to better understand the disease. The Fulton Center is currently enrolling patients in this program.

REFINE-ALS

REFINE-ALS is a biomarker study for patients who will be receiving Edaravone. The Fulton Center is currently enrolling patients

Neurosense

Neurosense is a Phase 2 open label study to evaluate the safety and tolerability of the Ciprofloxacin/Celecoxib fixed dose combination. The Fulton Center is currently enrolling patients.

Clinical Research Updates: HEALEY ALS Platform Trial

Barrow to Participate in First Platform Trial for ALS

Every 90 minutes, someone learns they have ALS and another person dies from the disease. People with ALS live about two to five years on average, and existing medications can only moderately slow the progression.

These statistics illustrate the need to accelerate the study of promising ALS drugs. That's why <u>Barrow Neurological</u> <u>Institute and 53 other sites</u> around the United States are collaborating to <u>launch</u> <u>the first-ever platform trial for ALS</u>.

Also known as <u>amyotrophic lateral</u>

sclerosis or Lou Gehrig's disease, ALS is a progressive neurodegenerative disease that affects motor neurons. When these nerve cells deteriorate and die, they are no longer able to initiate and control muscle movements. This causes the muscles in the body to waste away, eventually depriving people of their ability to walk, speak, swallow, and breathe.

The Sean M. Healey Center at Massachusetts General Hospital worked with the Northeast ALS (NEALS) Consortium to assemble a team of experts from around the country to rethink the design of early-phase trials for the disease, accelerate the path to effective treatments, and increase patient access to trials.

Barrow is the only participating site in Arizona and is providing all of the monitoring and clinical outcomes training.

"I believe the start of the platform trial project represents a major step in improving the speed and efficiency of ALS clinical trial conduct, which will hopefully lead to faster drug development and more effective agents to treat ALS coming to patients more quickly," said neurologist <u>Dr. Jeremy</u> <u>Shefner</u>, who specializes in ALS and serves as the chair of neurology for Barrow.

How Platform Trials Work

While <u>traditional clinical trials</u> evaluate only one drug at a time—an expensive process that can take years to complete—platform trials test multiple drugs simultaneously.

A central institutional review board (IRB) approves the overall protocol for the trial, which includes its objectives, design, methodology, and statistical considerations. This infrastructure remains in place for each drug tested and until a drug proves successful in curing ALS.

The trial's flexible design, borrowed from successes in cancer research, allows drugs to be dropped as they are deemed ineffective and new drugs to be added as they become available. This model closes the gap between identifying a promising therapy and actually testing it.

"As the vision of the platform trial is to always be enrolling, there is no downtime that is usually associated with study startup and closeout," said <u>Dr. Shafeeq</u> <u>Ladha</u>, director of the <u>Gregory W. Fulton</u> <u>ALS and Neuromuscular Disease Center</u> <u>at Barrow</u>.

Rapidly testing therapies not only provides answers faster, but it also reduces costs and ensures we continuously advance our understanding of the disease itself.

Also, because platform trials use a shared control group for all drugs, they reduce a participant's chance of being on a placebo instead of an active treatment. This also means fewer patients need to be enrolled—another factor in helping to accelerate the trial.

Research Staff Updates



My name is Cassie Nelson and I had the pleasure of joining the ALS department at Barrow Neurological Institute last October. Although I do not have a background in ALS or research, I've quickly grown a special place in my heart for our ALS patients. I am currently working on a few ALS studies, as well as some Myasthenia Gravis studies. One

reason that I really enjoy my job is because these trials offer hope to my patients and their families.

I am originally from central Wisconsin and completed my BSN at the University of Wisconsin-Madison. Go Badgers! Yes, cheese heads are real and I will gladly have a discussion with you about the best place to find cheese curds, fried or squeaky! After graduating and moving to Arizona for some sunshine, I began my nursing career in a cardiac progressive care unit specializing in congestive heart failure. After that, I spent some time circulating in the operating room in a variety of specialties. In my free time, I enjoy jogging, spending time with friends and family, cooking and trying to keep up with my Springer Spaniel puppy, Stella.



Stephanie Strong

My name is Stephanie Strong and I have been working as a Research Coordinator with Dignity Health since 2017. In January of this year, I was honored to transition to the ALS/Neuromuscular Research Department from Hepatology Research. Throughout my career I have worked in several areas in research, but this is my first

opportunity working with ALS patients. I am excited to be a part of conducting ALS research trials and hope that new medications can be found to help improve the quality of lives of our patients. Originally I am from Kansas and I completed my bachelor's degree in Human Biology from the University of Kansas. Needless to say I will be a Jayhawk fan for life! Currently I am going to nursing school as well as working, so I do not really have much time to enjoy my hobbies. When I do have time I like to cook, hike, camp, and go fishing. Clinical Research Updates continued from page 3

"It greatly increases the throughput of promising drugs," Dr. Ladha said. "This has become a problem as science has presented us with more drugs than we have patients to test in an efficient way."

However, comparing each drug to the same placebo group means all treatment regimens must be similar to each other and have similar inclusion criteria.

Drugs that are ready for <u>Phase III</u> studies are not good candidates for platform trials, as platform trials are best suited for quickly assessing the viability of new drugs before they can move to Phase III.

Additionally, platform trials rely on having a large pipeline of promising drugs so that there are no long gaps.

An initial call for drug proposals yielded an influx of ideas from almost 30 participants in 10 countries. The Healy Center created a committee to select the top five drugs to launch the trial with. The applicants were mainly small biotechnology companies with promising ideas but without the funds to set up clinical trials themselves.

The <u>HEALEY ALS Platform Trial</u> has received funding from the Healey family and friends, AMG Charitable Foundation, TackleALS, and the <u>ALS</u> <u>Association</u>.

The primary outcome of the trial will be whether or not the drugs boosted a functional rating score for ALS after six months. This widely used score is determined through assessments of speech, swallowing, walking, breathing, handling food utensils, dressing, and other daily functions.

"The NEALS platform trial will change the paradigm of how we do ALS clinical research," Dr. Ladha said. "It creates a unique partnership between industry and academics and helps to bring drugs to market in a collaborative way, balancing the business and the science."

Enrollment is expected to open in the spring. For more information about the trial, email <u>Fulton.Research@DignityHeath.org</u>.

Nurses Week

The Gregory W. Fulton ALS and Neuromuscular Disease Center has many wonderful nurses who work with our patients. We were happy to celebrate them earlier this month for Nurses Week!



Gale Kittle

Outcomes Director and Research Nurse Clinician

"I've spent my entire nursing career at Barrow, in many different areas. Several years ago I began working with Dr. Ladha on the first ALS trials at Barrow, and found that I loved working with ALS patients and clinical research. Today my role focuses on training others who work on ALS trials all over the world. Although I miss working directly with patients, I know that I am still impacting lives by helping to find effective treatments through ALS clinical research."



Cassie Nelson

Research Nurse

"The best part of my day is just getting to talk with my patients. I can only imagine what it must feel like to find yourself in a hospital or diagnosed with an illness. I am deeply fulfilled by getting to hear my patient's stories, fears, hopes and dreams. I enjoy getting to walk through the journey with them and their families on the good days and bad days."



Jourdan Milliard

Nurse Practitioner

"My patients and their families inspire me and make being a nurse practitioner such a rewarding profession. A good day for me is knowing that I was able to improve someone's quality of life and address not only their medical but emotional needs. I am grateful to work alongside others that are passionate about patient advocacy, education, and research."



Nancy Rosas

Registered Nurse

"I love being a nurse for many reasons—mostly because it has brought me into the lives of people whom I wouldn't have met if I weren't a nurse. This specialty never stops giving."

ALS Advocacy Update

Last year, hundreds of advocates went with their local ALS Association chapters to Washington DC to meet with their legislators about policies that affect the ALS community. One of these policy asks was to support the removal of non-invasive ventilators from Medicare's competitive bidding program, which would limit access for patients. After a year of campaigns and advocacy efforts, Medicare will not be adding noninvasive ventilators to the competitive bidding program. Thank you to our ALS community and advocates for their hard work!

Link: CMS Removes Non-Invasive Ventilators from Round 2021 of the Competitive Bidding Program

ALS Basic Science Research During a Pandemic

The COVID-19 pandemic has brought new and unprecedented challenges to the research laboratories at the Fulton ALS Center. With stay-at-home orders

and social distancing measurements in place, it becomes difficult to work together in a small lab space while keeping everyone safe. So how did we manage to continue our research endeavors in both the Sattler lab and the Bowser lab? Incorporating social distancing in the laboratories required flex scheduling our staff to reduce the total number of staff members present in the lab at any one time. Research was focused on the most important and most timely projects that required our scientists to still be present in the laboratories. This meant that some research studies have been halted or delayed during COVID-19. However many of our colleagues at other research institutions were completely shut down by COVID-19, so we were

fortunate to keep our research moving forward during this time.

Meanwhile, staff working at home were performing data analysis, which is easily done with just a personal

computer and remote access to the secured BNI data server. Microscopic images were analyzed using imaging software, genetic profiling data sets were analyzed using

big data software packages and data figures were generated for future publications summarizing our research findings. Indeed, several manuscripts were submitted throughout the past two months of this pandemic, including projects on ALS biomarkers and new insights into trafficking of cells into the central nervous system during ALS.

At the same time, junior faculty members Drs. Bakkar and Medina as well as Drs. Sattler and Bowser were submitting several grant applications to federal funding agencies (DOD and NIH), disease foundations (MDA), and local research funding sources (ADRC/ BNF), taking advantage of a time without national or international

travels to conferences and meetings. The latter has all been moved to virtual meetings via Zoom or WebEx or other virtual meeting platforms, allowing the Fulton scientists to keep up with the latest discoveries and exchanging their research findings with their peers.

12

Tina Kovalik and Justin Saul from Bowser's Lab



Stephen Moore from Sattler's Lab



ALS Association Support Groups

ALSA support groups are currently being held virtually. If you have any questions, please call (602) 297-3800 or email info@alsaz.org.

Join the meeting from your computer, tablet or smartphone.

https://global.gotomeeting.com/ join/951959885

You can also dial in using your phone. United States: +1 (408) 650-3123 Access Code: 951-959-885

West Valley Patients, Caregivers, Family and Friends

When: 2nd Thursday of each month 1:00pm-3:00pm

East Valley Patients, Caregivers, Family and Friends

When: 2nd Tuesday of each month 1:00pm-3:00pm

Phoenix Caregivers

When: 3rd Tuesday of each month 5:30pm-7:30pm

Phoenix Survivors

When: 3rd Wednesday of each month 5:30pm-7:30pm

Tucson Patients, Caregivers, Family and Friends

When: 1st Thursday of each month 12:00pm-2:00pm

Tucson Caregivers

When: 3rd Thursday of each month 1:00pm-3:00pm

Tucson Survivors

When: 4th Tuesday of each month 10:00am-12:00pm

Northern Arizona Patients, Caregivers, Family and Friends

When: 4th Monday each month 12:00pm-2:00pm



MDA ALS Golf Classic Thursday, September 3rd, 2020

https://mda.donordrive.com/event/mdaalsgolfclassic



DUTCH BROS

Find Us On Facebook!

The Gregory W. Fulton ALS and Neuromuscular Disease Center is now on Facebook.

Find us at: Gregory W Fulton ALS Center

Support the Fulton Center

If you would like to support the Fulton Center's Clinic or ALS Research efforts, please see the links below.

Clinic Donations: https://give.supportbarrow.org/als

Research Donations: <u>https://give.supportbarrow.org/alsresearch</u>

The Great LiveLikeLou Outdoor Clean-Up for ALS Families

The LiveLikeLou Foundation has created a volunteer program that supports families impacted by ALS.

"The Great LiveLikeLou Outdoor Clean-Up for ALS Families" matches ALS families with a Phi Delta Theta volunteer who can assist with yard work, repairs, and other outside odd jobs. If you are interested in getting matched with a volunteer, please visit the link below.

The Great LiveLikeLou Outdoor Clean-Up for ALS Families Inquiry



Matching ALS Families with Members of Phi Delta Theta in a Time of Need



Gregory W. Fulton ALS Center