Amyotrophic lateral sclerosis, also known as ALS or Lou Gehrig’s disease, is one of the most complicated diseases to unravel and currently has no cure. Approximately 6,000 people are diagnosed with this neurodegenerative disease every year, and at least 90 percent of them have no family history of the disease. Most individuals with ALS die within three to five years of the onset of symptoms.

The overall goal of ALS research at Barrow Neurological Institute is to identify cellular and molecular mechanisms of the disease and to translate those findings to improved treatments for ALS patients, both within Arizona and throughout the world. Drs. Robert Bowser and Rita Sattler lead ALS research at the Barrow, with Drs. Nadine Bakkar and David Medina working in the Bowser Laboratory as junior faculty members. The ALS research team also consists of postdoctoral fellows, graduate students, research technicians, and interns.

Robert Bowser, PhD

Dr. Bowser is the chief scientific officer, John and Betty VanDenburgh Chair of Neuromuscular Disease, and professor and chair of the Department of Neurobiology at Barrow Neurological Institute and St. Joseph’s Hospital and Medical Center. He received his PhD from Yale University and completed postdoctoral fellowship training at the Albert Einstein College of Medicine. His research focuses on the discovery and use of biomarkers to uncover mechanisms of neurodegeneration associated with ALS, translating those findings toward new therapies, and the use of biomarkers in drug development and clinical trials. He works extensively with the patient population. His lab developed the procedures used for collection, processing, and storing of human biofluids (blood, cerebrospinal fluid) in ALS research studies and clinical trials throughout the world. He has received numerous awards based on his research, including the 2015 Sheila Essey Award for ALS Research, which is considered the top international award for ALS research. He co-chairs the Northeast ALS Consortium (NEALS) Biofluid Repository and is a member of the Target ALS Post-Mortem Tissue Core, overseeing the largest national biofluid and tissue repository used by scientific investigators throughout the world.

Dr. Bowser and his team have pioneered ALS biomarker discovery and the use of biomarkers in ALS clinical trials. His work has led to identifying new mechanisms of motor neuron degeneration and new avenues to deliver drugs into the central nervous system using nanoparticles. In collaboration with Dr. Rita Sattler, Dr. Bowser created the first-ever precision medicine program for an individual ALS patient, in which the investigators screened all FDA-approved drugs to identify those that protect patient-derived motor

Continued on back
neurons and then introduced two drugs to the ALS patient. His lab was also the first to use and publish artificial intelligence-based approaches to uncover new pathways and genes contributing to ALS. Dr. Bowser has also started a local biotechnology company (Iron Horse Diagnostics, Inc.) to commercialize biomarkers for ALS and other neurologic diseases, including traumatic brain injury, and will soon be launching the first diagnostic test for ALS.

**Rita Sattler, PhD**

Dr. Sattler is an associate professor of neurobiology at Barrow Neurological Institute and St. Joseph’s Hospital and Medical Center. She obtained her PhD from the University of Toronto and was a postdoctoral fellow at Johns Hopkins University. Dr. Sattler’s laboratory reprograms blood and skin cells from patients into neurons and other cell types of the brain and spinal cord to create models of their disease in a dish. She then uses these patient-derived stem cells and high-end microscopy-based approaches to study cellular and molecular mechanisms contributing to motor neuron degeneration. This work contributes to the search for neuroprotective drugs and new therapies for ALS and other neurodegenerative diseases. Dr. Sattler also leads our PhD graduate training program and summer undergraduate training program. These programs are vital to the growth and development of the next generation of scientific leaders, and they promote educational opportunities for Arizona students.

Both Dr. Bowser and Dr. Sattler receive research funding support from National Institutes of Health (NIH) and the Department of Defense. Drs. Bowser and Sattler also have leadership positions in national and international ALS collaborative projects, foundations, and funding organizations.

**ALS Clinical Activities**

The Gregory W. Fulton ALS and Neuromuscular Disease Center at Barrow Neurological Institute is led by Drs. Jeremy Shefner and Shafeeq Ladha. It is the largest ALS clinic west of the Mississippi River. We have more active clinical trials and clinical research studies for ALS patients than almost any clinic in the United States. Dr. Shefner helped create a national collaborative network of ALS clinics to perform clinical trials, and it is used by almost all ALS clinical trials. His group helps monitor most ALS clinical trials in the U.S. and throughout the world. Dr. Shefner earned the Sheila Essey Award in 2014, making Barrow the only institution in the world with two Essey Award winners. Dr. Ladha is an outstanding ALS clinician, who is devoted to patient care and leads efforts to develop new imaging-based biomarkers in ALS patients. The ALS Center at Barrow Neurological Institute is an international leader in ALS patient care, clinical trial management, and translational research efforts to develop new and improved therapies for ALS patients.