

# Gregory W. Fulton ALS & Neuromuscular Disease Center



## IMPACT OF PHILANTHROPY



**1,472**  
patients treated  
in 2024



**14+**  
research studies



**20**  
patients enrolled in  
Expanded Access  
Program

A diagnosis of amyotrophic lateral sclerosis (ALS), or Lou Gehrig's disease, can change the trajectory of life in the blink of an eye. This frightening and devastating disease gradually robs patients of their ability to move, talk, swallow, and even breathe on their own.

Led by Shafeeq Ladha, MD, the Gregory W. Fulton ALS & Neuromuscular Disease Center provides patients with an expert diagnosis and holistic, personalized, and integrated care to improve their quality of life. At the same time, Barrow ALS scientists are conducting cutting-edge research to more effectively treat, and one day cure, the disease. The Fulton ALS Center is further distinguished as an ALS Association Certified Treatment Center of Excellence.

## You contribute to increased access to ALS therapies.

Although there is no cure for ALS, patients still want to fight the disease while they can, as well as help future patients. They can do this by participating in clinical trials. However, less than 50% of ALS patients qualify for traditional clinical trials due to rigid enrollment criteria.

Thanks to the generosity of Autumn and Bobby Henderson, Barrow launched the Henderson-Liebman ALS Expanded Access Program to allow more patients with ALS to participate in trials testing promising new drugs. In its first year, the program provided 20 patients with access to experimental

medications that would normally not be available to them through traditional clinical trials.

## On the Horizon

In 2023, Barrow was awarded a \$16.7 million grant from the National Institutes of Health (NIH) to create and lead the Access for All in ALS Consortium (ALL ALS), which aims to collect blood, spinal fluid, and other clinical information from patients across the country. To date, the team has activated eight of the 34 clinical sites and has enrolled 20 patients. In the next year, they plan to activate the rest of the sites, continue enrolling patients, and develop the clinical research registry to enter the data collected.

### YOUR SUPPORT ADVANCES INNOVATIVE ALS RESEARCH.

#### Eye Movements as Biomarkers

Dr. Ladha is investigating whether high-resolution cameras can detect very small eye movement changes in patients in the earliest stages of ALS. He will then track those changes over time to see if they can serve as biomarkers of disease progression.

#### Predicting Falls in ALS

Dr. Ladha is working with researchers at Arizona State University to investigate the causes of falls in ALS by conducting a series of sophisticated balance tests. This could help predict which patients are at the highest risk for falls so preventative measures can be implemented.

#### Gene Mutations in ALS

Robert Bowser, PhD, Chief Scientific Officer at Barrow, has developed novel preclinical models of different mutations in the MATR3 gene, which is linked to ALS. He has identified specific mutations that could cause ALS and others that could increase the risk of developing it.

#### TDP-43 Dysfunction in ALS

Rita Sattler, PhD, is studying how dysfunction in the TDP-43 protein contributes to ALS, paving the way for new treatments that can be applied to the 97% of patients whom this affects.

#### Cryptic Exons in ALS and FTD

Dr. Sattler and Lauren Gittings, PhD, collaborated with the Translational Genomics Research Institute to confirm the presence of a cryptic exon (a specific error in a genetic sequence) in cells in ALS and frontotemporal dementia (FTD).