Barrow Neurological Foundation

FISCAL YEAR 2023 STEWARDSHIP REPORT

Gregory W. Fulton ALS and Neuromuscular Disease Center

AMYOTROPHIC LATERAL SCLEROSIS (ALS) is a terrifying diagnosis for patients and a heartbreaking one for their friends and family. The disease gradually robs patients of their ability to walk, talk, eat, and even breathe on their own. Meanwhile, all of their cognitive functions remain intact, so they are fully aware of what is happening. Currently, there is no cure for ALS, and treatment options are extremely limited.

The Gregory W. Fulton ALS and Neuromuscular Disease Center at Barrow Neurological Institute, led by Shafeeq Ladha, MD, is a national model for offering comprehensive care within a single center while increasing access to clinical trials. Barrow's renowned ALS scientists also work to conduct innovative research into new treatments for the disease. The Fulton ALS Center is an ALS Association and Muscular Dystrophy Association Certified Treatment Center of Excellence.

3,215
outpatient clinic visits

15
active research studies

5
external research grants awarded to Barrow ALS scientists

BARROW NEUROLOGICAL INSTITUTE BY THE NUMBERS

CLINICAL IMPACT

117,700+
total number of patient visits

5,800+
brain and spine surgeries

14
Centers of Excellence

GLOBAL IMPACT

59
research fellows and visiting scholars: Argentina, Austria, Brazil, Chile, China, Colombia, Czech Republic, Denmark, Finland, Germany, India, Italy, Jordan, Mexico, Pakistan, Peru, Portugal, South Korea, Spain, Taiwan, Turkey, United Kingdom

EXPANDING CLINICAL TRIAL ACCESS

Although there is no cure for ALS, patients still want to be proactive and fight the disease in any way they can. This includes participating in clinical research trials. Unfortunately, less than 50% of ALS patients qualify for traditional clinical trials based on rigid participation criteria. Barrow aims to change that with the Henderson-Liebman ALS Expanded Access Program, which was made possible by a generous $2.5 million gift from Autumn and Bobby Henderson.

Expanded access programs (EAPs) are designed to allow broader access to experimental therapies. As such, the Henderson-Liebman ALS Expanded Access Program will offer ALS patients the opportunity to participate in trials testing promising new drugs, even if they don't qualify for traditional clinical trials. This has the potential to shift the paradigm for ALS clinical trial participation dramatically, bringing hope to patients who feel stuck in terms of accessing new therapies.

> **"My childhood friend's father was diagnosed with ALS in 2020. I had grown up with her family, so it was devastating watching him suffer through this terrible disease. I made this gift to honor my dear friend's father and the work of Dr. Shafeeq Ladha, who provided him with exceptional care."**
>
> Autumn Henderson

ADVANCING ALS RESEARCH

New Medication Approval: Fulton ALS Center Director Shafeeq Ladha, MD, and his team played a critical role in both the early phase 1 and pivotal phase 3 trials that led to FDA approval of a new drug to treat a genetic form of ALS. The drug, tofersen, is the first treatment that targets a familial cause of ALS and is one of the few treatments available to slow disease progression. This marks a major milestone for the ALS community and has the potential to pave the way for developing more treatment options for patients.

Gene Therapy for ALS: Barrow scientist David Medina, PhD, has been working to protect motor neurons in ALS by activating the retinoid signaling pathway. At first, he tried a medication-based approach to delivering retinoids to this pathway, but he encountered significant problems. With philanthropic support from Barrow Neurological Foundation, Dr. Medina was able to collaborate with Fredric Manfredsson, PhD, and switch to a gene therapy approach. Initial results were promising and the scientists

received a grant from the United States Department of Defense (DoD) to continue their work.

Repurposing Existing Drugs: Brad A. Racette, MD, FAAN, Chair of Neurology at Barrow, and his team utilized de-identified U.S. Medicare data to discover links between existing medications and a lower risk of ALS. In preclinical models, lovastatin, a drug commonly used to lower cholesterol, showed the most promise in preserving motor neurons and delaying symptom onset. The Racette Laboratory, which utilizes start-up funding from Barrow Neurological Foundation, was awarded a two-year grant from the DoD to further validate its findings.

Neuroimaging Biomarkers: Barrow ALS scientist Nadine Bakkar, PhD, partnered with Barrow neuroimaging scientist Ashley Stokes, PhD, to evaluate blood vessel changes as a possible indicator of disease progression, and as a potential therapeutic target, by comparing advanced neuroimaging scans, bio-fluid specimens, and clinical measures of people with and without ALS over time.

Genetic Mutations: In some cases, the same genetic mutations occur in both ALS and frontotemporal dementia (FTD). One of those mutations is a repetitive expansion of a gene called C9orf72. Barrow scientist Rita Sattler, PhD, has identified, for the first time ever, a dysfunctional genetic sequence in specific brain cells of patients with the C9orf72 mutation. The study, which received initial funding from Barrow Neurological Foundation, has the potential to discern which cells are most vulnerable to the genetic changes that cause these diseases.

ON THE HORIZON

The Fulton ALS Center plans to create a collaborative research platform that leverages the expertise of engineers, data scientists, and Barrow ALS specialists to make breakthrough treatment discoveries. The platform would also allow for the comprehensive study of the relationship between ALS, Parkinson's disease, and Alzheimer's disease.

The ALS team plans to create monthly ALS/neuromuscular disease clinics in rural and underserved areas of Arizona, allowing patients who can't travel long distances to receive expert care. In addition, the team plans to expand treatment and care for other neuromuscular diseases such as muscular dystrophy, myositis, myopathies, and primary lateral sclerosis by recruiting a chair in neuromuscular diseases.

BARROW NEUROLOGICAL INSTITUTE BY THE NUMBERS

RESEARCH

327
active research studies

200+
peer-reviewed journal publications

$12 MILLION
in new federal research grant support

DONOR IMPACT

3,898
total donors

$44 MILLION
distributed to Barrow Neurological Institute, including:

$25.4 MILLION
designated to the Ivy Brain Tumor Center

$5.3 MILLION
designated to specific centers/programs

$11.9 MILLION
for basic, clinical, and translational research

$1 MILLION
in endowments

The Gregory W. Fulton ALS and Neuromuscular Disease Center at Barrow Neurological Institute is the largest center of its kind west of the Mississippi.

THANK YOU FOR YOUR SUPPORT

ALS is one of the most devastating and debilitating diseases in the world. With support from donors like you, the Fulton ALS Center is able to provide patients with the best care possible while conducting innovative research aimed at developing new and more effective treatments for this disease. In the past year alone, Barrow ALS scientists have received three significant grants from the DoD and have played a critical role in studies that led to FDA approval of a new drug for a genetic form of ALS. Our scientists also were the first to discover a dysfunctional genetic sequence in specific brain cells of ALS and FTD patients with the C9orf72 genetic mutation. These milestones would not have been possible without your generosity.

On behalf of the entire Fulton ALS Center team, thank you for your support.

With gratitude,

Shafeeq Ladha, MD
Ira A. and Mary Lou Fulton Chair in Motor Neuron Diseases
Director, Gregory W. Fulton ALS and Neuromuscular Disease Center

The mission of Barrow Neurological Foundation is simple: to be the catalyst of our donors' passion for transformation by providing the resources for Barrow Neurological Institute to achieve its mission of saving human lives through innovative treatment, groundbreaking research, and educating the next generation of the world's leading neuroclinicians.

Barrow
Neurological Foundation

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