

Study of Radicava Effects in ALS

+Amyotrophic Lateral Sclerosis

Full Trial Name: Radicava/(Edaravone)

Enroll & participate in study from your home!

Findings in Biomarkers in ALS (REFINE-ALS) Radicava has been shown to slow the loss of physical function in ALS and was approved by the FDA as a treatment for ALS in 2017. The purpose of this observational study is to provide a deeper understanding of the biological effects of Radicava in participants with ALS. REFINE-ALS will measure the levels of distinct biomarkers involved in oxidative stress and in inflammatory response, neuronal injury or death, and muscle injury.

All participants must make the clinical decision to be prescribed Radicava prior to enrolling and screening for the study. Participants will be followed over six cycles of Radicava as an intravenous (IV) infusion over 24 weeks, with blood and urine samples collected at each visit for analyses. Biomarker levels and ALS progression will be assessed before initiating treatment, at the start of treatment, and at specific times throughout the study. The study requires 8 study visits to MGH over approximately 6 months.

Principal Investigator: Suma Babu, MD

Sponsor: MT Pharma

Enrollment Contact: Austin Lewis, 617-724-7928, alewis29@mgh.harvard.edu; Mackenzie Keegan, 617-643-6252, mkeegan@mgh.harvard.edu

Study of LAB PALS

+ALS, +Healthy Volunteers, +Asymptomatic ALS gene carriers

Full Trial Name: A Longitudinal Analysis of Biomarkers in Patients with ALS

This study is collecting biomarker samples such as blood, urine, and cerebrospinal fluid from patients with ALS, asymptomatic ALS gene carriers, and healthy volunteers. The purpose of this study is to provide a longitudinal sample set which can be used to further uncover ALS pathophysiology, discover disease biomarkers, and identify new therapeutic targets. The samples we collect will be used to compare and analyze changes in immune cells and other changes in plasma and gene expression. ALS patients and asymptomatic ALS gene carriers participate in up to 7 visits every 3 months over 2 ½ years. Healthy volunteers participate in a one-time visit. Visits include a collection of medical information and ALS history, blood draw, Slow Vital Capacity test, and ALSFRS-R questionnaire. Participants must be at least 18 years of age and have no other preexisting neurological conditions.

Principal Investigator: James Berry, MD, MPH

Sponsor: Winthrop Fund and Barvin Fund

Enrollment Contacts: Lisa Ramdas, 617-724-9196, lramdas@mgh.harvard.edu



MASSACHUSETTS
GENERAL HOSPITAL

NEUROLOGICAL CLINICAL RESEARCH INSTITUTE



Healey Center

Sean M. Healey & AMG Center
for ALS at Mass General

Currently Enrolling

Biofluid Biomarker & Observational Studies



Updated: June 2022

Study of DIALS

+ *Asymptomatic first-degree adult relatives of people with familial ALS*

Full Trial Name: Dominant Inherited ALS (DIALS) Network

This study is recruiting participants who do not have any neurological symptoms, but who have a first-degree relative with ALS caused by a mutation. The purpose of the research study is to study a population at risk for developing ALS. The information collected in this study will further our understanding of underlying early disease changes to allow for development of novel therapeutics that target the earliest changes in ALS and allow for possible disease prevention.

Through this study you will be offered genetic counseling, and genetic testing for all currently known genes that may cause ALS. In addition, the study will be performing regular, longitudinal evaluations (e.g. blood samples, questionnaire completion; pulmonary and strength testing etc..) for a period of several years. Study visits will be completed at the Neurological Clinical Research Institute at Massachusetts General Hospital.

Principal Investigator: Katharine Nicholson, MD

Sponsor: ALS Finding a Cure, Target ALS, ALS Association, American Academy of Neurology/Muscular Dystrophy Association

Enrollment Contact: Kathleen Diana, 617-724-6346, kdiana@mgh.harvard.edu; Allison Carey, 617-726-1559, abcarey@mgh.harvard.edu

Study of Longitudinal Microbiome in ALS

+ *Amyotrophic Lateral Sclerosis*

+ *Asymptomatic ALS Gene Carriers*

+ *Healthy Volunteers*

Enroll and participate from home!

Full Trial Name: Longitudinal Assessment of the Gut Microbiome in People with ALS

This study is recruiting participants with ALS, asymptomatic patients who carry an ALS causing gene, and healthy volunteers. The purpose of the

research study is to look at the stool of people with ALS to observe the relationship between the gut microbiome and the progression of ALS over time. Information collected in this study through questionnaires regarding medical history and diet, as well as stool and blood samples will further our understanding of ALS and contribute towards the development of novel therapeutics. ALS and healthy volunteers will have visits every 3 months, while asymptomatic ALS gene carriers will have visits every 6 months. Visits may be attended in-person or remotely, over 5 years.

Principal Investigator: James Berry, MD, MPH

Sponsor: National Institutes of Health and Brigham and Women's Hospital

Enrollment Contact: Kelly Fisher, 617-726-9094, kefisher@mgh.harvard.edu

Connect to ALS Research at the Healey Center!

Sign up for the MGH ALS Link:



<https://lp.constantcontactpages.com/su/saTzwIp/ALSLink>

View currently enrolling ALS trials:



<https://www.massgeneral.org/neurology/als/research/als-clinical-trials>

Study of ALS Sample Repository (Living Library)

+ *Amyotrophic Lateral Sclerosis*

+ *Healthy Volunteers*

+ *Non-ALS Neuro Disease Volunteers*

+ *Motor Neuron Disease Volunteers*

One In-Person Blood Collection

Full Trial Name: ALS Sample Repository

We are developing a diverse living library of biofluid samples (blood, spinal fluid, urine) from people of different ages, ethnicities, and sexes, from healthy volunteers, people with amyotrophic lateral sclerosis (ALS), and motor neuron disease (MND), as well as other neurological diseases that may mimic motor neuron diseases. Samples collected will be stored and used for ALS research conducted globally to answer questions related to cause, prevention, treatment, and heritability of ALS. Participants must be at least 20 years old and be able to answer brief questions about their medical and family history, as well as be willing to have blood and/or CSF drawn for the study.

Principal Investigator: James Berry, MD, MPH

Sponsor: Hollister Lindley Fund

Enrollment Contact: Alison, Clark, 617-726-4284, aclark51@mgh.harvard.edu, or, Kelly Fisher, 617-726-9094,

kefisher@mgh.harvard.edu

For more information about these trials:

Contact the research coordinator listed for trial(s) you are interested in OR Judi Carey, Research Access Nurse, at

jcarey8@partners.org or 617-724-8995