

Inosine

Researchers are currently enrolling people with ALS in a research study which is evaluating the safety of Inosine, a supplement that is used to raise blood levels of uric acid. Your participation in this study would be at least 27 weeks. The purpose of the research study is to determine whether inosine is safe and tolerable for patients with Amyotrophic Lateral Sclerosis (ALS). While taking the study drug, participants will be asked to complete several tests and utilize a mobile application to collect information on tasks. From this study, the researchers hope to learn more about ALS and its treatment. The study will take participants 27 weeks to complete. Participants must be at least 18 years of age.

Principal Investigator: Katharine Nicholson, MD

Contact:

Jianing Liu
617-726-1880
jliu45@partners.org

Brainstorm

The purpose of the study is to evaluate the safety and efficacy of transplanting self-derived mesenchymal stem cells secreting neurotrophic factors (MSC-NTF) into the cerebrospinal fluid in patients with ALS. The cells are derived from your own bone marrow and administered into your cerebrospinal fluid three times (once every 8 weeks for 16 weeks). Participation in this study will last approximately 11 months.

Principal Investigator: James Berry, MD, MPH

Contact:

Taylor Mezoian
617-643-0312
tmezoian@mg.harvard.edu



MASSACHUSETTS
GENERAL HOSPITAL

NEUROLOGICAL CLINICAL RESEARCH INSTITUTE

NCRI

Interventional Studies Currently Recruiting

For more information, visit:

<http://www.massgeneral.org/als/>

<http://www.alsconsortium.org>

For more information about any of these trials,
please contact the listed study coordinator or
one of our research nurses:

Judi Carey, R.N., (617) 724-8995

jcarey8@partners.org

Sarah Luppino, R.N., (617) 724-3380

Katie Campbell, R.N., (617) 643-6249

Jen Scalia, R.N., (617) 726-0316

Danica Sanders, R.N., (617) 643-6249

Taylor Stirrat, R.N., (617) 726-5059

Updated: September 2018

Orion (Refals)

This Phase III research study is being done to evaluate if Levosimendan (ODM-109) can help with respiratory function in people with ALS. We also want to find out if levosimendan is safe to take without causing too many side effects in ALS. Orion Corporation Orion Pharma is the sponsor of this study. Levosimendan is contained in a capsule, which is taken orally.

Study participation will last approximately 12 months. Please contact the study team to obtain additional information about the study visits and procedures. The study team will review with the inclusion and exclusion criteria during the study, which is also available on clinicaltrials.gov.

Principal Investigator: Suma Babu, MD, MPH

Contact:

Olivia Pijanowski
617-643-2522
opijanowski@mgh.harvard.edu

RNS60-ALS

We are doing this trial to find out the effects of RNS60 on biomarkers in people with amyotrophic lateral sclerosis (ALS). Biomarkers are substances in the body that can be measured and evaluated as indicators of the body's biological processes. We will study components in blood that may be affected by RNS60 and how it might be related to ALS.

People who enroll in this trial will receive either RNS60 or placebo for 24 weeks. During this time, you will be asked to make weekly in-person study visits to MGH. You will also receive a supply of RNS60 or placebo to take at home once per day using a nebulizer.

Principal Investigator: Sabrina Paganoni, MD

Contact:

Michael Doyle
617-724-7398
mdoyle@mgh.harvard.edu

Genentech

The purpose of the research study is to determine whether the drug GDC-0134 is safe and tolerable for patients with ALS. This study will also look at the amount of study drug in your blood after different doses are taken. Participation in this study will last approximately three to five months. Participants may have the opportunity to participate in more than one dosing period. Each dosing period will require one four-night stay in the hospital during GDC-0134 dosing, and a follow up visit at one week and two weeks post-dosing. Participants must be at least 18 years of age, and able to comply with study procedures. We will review a complete list of the study's inclusion and exclusion criteria with you during a screening visit, to ensure it will be safe for you to enroll.

Principal Investigator: Nazem Atassi, MD, MMSc

Contact:

Brixhilda Dedi
617-726-4284
bdedi@mgh.harvard.edu

Biogen SOD1

We are doing this research study to find out about the safety and tolerability of the study drug BIIB067. The study is funded by Biogen MA Inc. This study is recruiting patients with SOD1-Amyotrophic Lateral Sclerosis (SOD1-ALS) with a forced vital capacity greater than or equal to 50% of predicted value. Participation in the study will last for approximately 31 weeks and will include an overnight stay at MGH in addition to in person visits. The study team can provide additional information on the number of required visits during your initial visit. There are additional inclusion/exclusion criteria that the study team will review with you in more detail.

Principal Investigator: Nazem Atassi, MD, MMSc

Contact:

Michael Doyle
617-724-7398
mdoyle16@mgh.harvard.edu

Centaur (Combination of Phenylbutyrate (PB) and Tauroursodeoxycholic Acid (TUDCA))

This research study is being done to find out if AMX0035 can help with Amyotrophic Lateral Sclerosis (ALS). We also want to find out if AMX 0035 is safe to take without causing too many side effects.

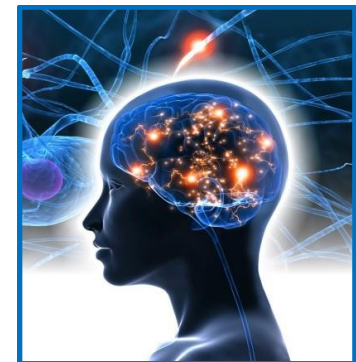
AMX0035, a combination of Phenylbutyrate (PB) and Tauroursodeoxycholic Acid (TUDCA), is not approved by the US Food and Drug Association (FDA). This means that AMX0035 can only be used in research studies. This is the first use of the combination drug AMX0035 in humans. However, the individual components of the drug, PB and TUDCA, have been studied in small clinical trials of patients with ALS.

This research study will compare AMX0035 to placebo. Participants will receive study drug for a total of 24 weeks, followed by a telephone call about 30 days from the final study visit. During this time, we will ask participants to make up to seven study visits to the clinic and four telephone calls. Overall participants may be in the research study for about 8 months.

Principal Investigator: James Berry, MD, MPH

Contact:

Aileen Shaughnessy
617-724-9196
anshaughnessy@mgh.harvard.edu



Speech Motor Impairment in ALS

The Speech and Feeding Disorders Lab at MGH Institute of Health Professions is interested in studying the movements the face and mouth during speech, chewing and swallowing in persons diagnosed with ALS and healthy volunteers. You will be asked to fill out a health questionnaire as well as to repeat various sounds and sentences while the movements of your face and mouth are recorded. This research aims to help improve the diagnosis and treatments of ALS, and to help develop new technologies that will help improve communication for people with speech impairments.

Contact:

Speech and Feeding Disorders Lab
617-724-6347
speechfeedinglab@mghihp.edu



NCRI

For more information about any of these trials,
please contact the listed study coordinator or
one of our research nurses:

Judi Carey, R.N., (617) 724-8995

jcarey8@partners.org

Sarah Luppino, R.N., (617) 724-3380

Katie Campbell, R.N., (617) 643-6249

Jen Scalia, R.N., (617) 726-0316

Danica Sanders, R.N., (617) 643-6249

Taylor Stirrat, R.N., (617) 726-5059



Biofluid Biomarker & Observational Studies Currently Recruiting

For more information, visit:

<http://www.massgeneral.org/als/>

<http://www.alsconsortium.org>

Updated: September 2018

The purpose of this study is to find out how long the SOD1 protein stays in cerebrospinal fluid (CSF). The SOD1 protein is known to cause some forms of familial Amyotrophic Lateral Sclerosis (ALS). This study is recruiting adults with SOD1-confirmed Amyotrophic Lateral Sclerosis (ALS), Sporadic ALS (not caused by SOD1 gene), SOD1-positive asymptomatic gene carriers, and healthy adults with no medical history of neurological disease.

This study involves drinking a special leucine-labeled beverage for 10 days, which will be provided by the study team. Participation in this study will last approximately 4 months and requires 6 visits to MGH. Four of these visits will involve a lumbar puncture (LP).

Participants must be 18 years of age, able to comply with study procedures, and be medically safe to undergo a lumbar puncture (LP).

Principal Investigator: Katharine Nicholson, MD

Contact:
Michael Doyle
617-724-7398
mdoyle16@mgh.harvard.edu



Smartphone App for ALS

This study is recruiting adults with ALS who are smartphone users and are able to download and use a smartphone application. The study asks each participant to use the smartphone application for a few minutes every day by answering a questionnaire/survey, recording your voice and/or performing an on-screen exercise. The purpose of the research study is to determine how helpful a smartphone application would be in collecting research data and to learn more about disease progression.

Individuals will be in the study for about 3 months and will have the option to extend their participation by another 3 months. Participants must be at least 18 years of age.

Principal Investigator: James Berry, MD, MPH

Contact:
Harli Weber
617-643-5376
hweber1@mgh.harvard.edu

Skin Biopsy/Stem Cells for Research in MND

+Healthy Volunteers
+Primary Lateral Sclerosis

Neurodegenerative diseases are diseases in which nerve cells of the brain and spinal cord die. There is a need to understand the cause of these diseases and to develop treatments. Recent advancements in stem cell technology have allowed us to create a person's own nerve cells by taking a skin biopsy or blood sample. This study wants to use this new technology to make models for neurodegenerative diseases. We hope this will give us a better understanding of the diseases, enable us to use the cells for drug screening, and in the future, develop treatments.

Principal Investigator: James Berry, MD, MPH

Contact:
Tiina Xu
617-643-7428
tjxu@mgh.harvard.edu

Answer ALS +Primary Lateral Sclerosis

Answer ALS is a research study that is helping to create the largest-ever collection of stem cell lines derived from the blood of people with ALS. The collection of cells will be linked to detailed clinical information and a repository of biospecimens. The cell lines and clinical data will be studied in laboratories from across the country that have partnered for this project. Data from these labs will be analyzed individually and together using "big data" analysis techniques to demonstrate why and how motor neurons are affected by ALS, to identify biologically unique subgroups of people with ALS, and to search for new targets for drug therapy.

Participants must be at least 18 years of age, and able to follow study tasks. Participants will be asked to come to MGH approximately every 3 months for 1 year, and will be followed by telephone thereafter for as long as they are willing.

Principal Investigator: James Berry, MD, MPH

Contact:
Brixhilda Dedi
617-726-4284
bdedi@mgh.harvard.edu



Neuroinflammation (PBR28) Imaging Study +Healthy Volunteers

+Primary Lateral Sclerosis

The purpose of the study is to learn more about inflammation in the brains of people with Motor Neuron Disease (MND) using combined Magnetic Resonance Imaging (MRI) and Positron Emission Tomography (PET). Our study will examine whether particular cells, called microglia, are hyperactive in the nervous system of people with MND, such as those individuals with ALS.

This study aims to enroll the following cohorts of individuals:

- ALS, Primary Lateral Sclerosis (PLS), Hereditary Spastic Paraplegia (HSP)
- Healthy individuals who are known carriers of an ALS gene such as chromosome 9 open reading frame 72 (C9orf72) or superoxide dismutase 1 (SOD1)
- Individuals with a diagnosis of Frontotemporal Dementia (FTD)
- Healthy individuals without any neurodegenerative disease and no family history of MND

Study participation involves two visits to MGH over a maximum of three months. Participants must be between the ages of 18 and 80, be medically safe to undergo an MRI scan and be able to safely lie flat for at least 90 minutes. Additionally, participants cannot be taking any immunosuppressive medications or have a diaphragm pacing system and cannot have a diagnosis of Parkinson's disease, Alzheimer's disease, unstable psychiatric disease, or renal failure. All participants will be reimbursed for parking and receive compensation of \$150 upon completion of each MR-PET scan. There will be additional compensation of \$100 for each lumbar puncture completed by individuals with MND.

Principal Investigator: Nazem Atassi, MD, MMSc

Contact:

Catherine Cebulla
617-643-6252
ccebulla@mg.harvard.edu



For more information about any of these trials, please contact the listed study coordinator or one of our research nurses:

Judi Carey, R.N., (617) 724-8995

jc Carey8@partners.org

Sarah Luppino, R.N., (617) 724-3380

Katie Campbell, R.N., (617) 643-6249

Jen Scalia, R.N., (617) 726-0316

Danica Sanders, R.N., (617) 643-6249

Taylor Stirrat, R.N., (617) 726-5059

Updated: September 2018



MASSACHUSETTS
GENERAL HOSPITAL

NEUROLOGICAL CLINICAL RESEARCH INSTITUTE

NCRI

Imaging Biomarker Studies Currently Recruiting

For more information visit:

<http://www.massgeneral.org/als/>

<http://www.alsconsortium.org>

SPINE-ALS

We are doing this research to learn more about changes in the spinal cord and brain in ALS. “Microglia” are a type of immune cell that we are particularly interested in. We would like to find out if microglia are activated in the spinal cord and brain of individuals with ALS. Special imaging techniques are now available to test for changes in microglia. Magnetic Resonance Imaging (MRI) and Positron Emission Tomography (PET) are two tests that allow us to take pictures and “look inside” the body without surgery. MR-PET scanners use both MRI and PET tests at the same time. The MR-PET scanner may give clearer images and more information about the inside of the body.

If you choose to take part in this study you may have 2 visits at MGH, up to 3 months apart. We will pay you \$150 for completion of the spinal cord MR-PET scan. If you choose to participate in the optional brain MR-PET scan you will be paid an additional \$50 for completion.

Principal Investigator: Nazem Atassi, MD, MMSc

Contact:
Jianing Liu
617-726-1180
Jliu45@mgh.harvard.edu

Catherine Cebulla
617-643-6252
ccebulla@mgh.harvard.edu

TRACK-ALS

+Healthy Volunteers

TRACK-ALS is a multicenter, longitudinal study which aims to identify imaging and biofluid biomarkers in people with ALS to expand the understanding of disease pathology and progression. By identifying changes that occur in the blood, brain, and cerebrospinal fluid (CSF) of individuals with ALS, this pilot project has the potential to inform both diagnostic measures and drug development. Participation in the study for those with ALS involves approximately six onsite visits to MGH every three months for up to 18 months. Healthy volunteers are asked to make up to two onsite visits to MGH over the course of approximately two months. At these visits, participants will undergo an MRI scan to enable researchers to look at structural changes in the brain. Participants will also have blood drawn for analysis of inflammatory markers and generation of stem cells for further research. Other outcome assessments for ALS participants include breathing tests, muscle tests, and questionnaires, as well as an optional lumbar puncture to enable researchers to analyze biomarkers in the CSF. Finally, a subset of participants will undergo PET scans, which allow researchers to identify regions of inflammation in the brain.

Participants must be between the ages of 18 and 80, be medically safe to undergo an MRI scan (i.e., no metallic particles in the body), and be able to safely lie flat for at least 90 minutes. Additionally, participants cannot be taking any immunosuppressive medications or have a diaphragm pacing system and cannot have a diagnosis of Parkinson’s disease, Alzheimer’s disease, unstable psychiatric disease, cognitive impairment, or renal failure.

Principal Investigator: Nazem Atassi, MD, MMSc

Contact:
Jianing Liu
617-726-1180
Jliu45@mgh.harvard.edu

Catherine Cebulla
617-643-6252
ccebulla@mgh.harvard.edu



GE-179

+Healthy Volunteers

This study is recruiting participants with Amyotrophic Lateral Sclerosis (ALS), as well as people without a history of neurological disease, using MRI and PET scans to look at the brain activity of certain receptors involved in neurotransmission. Criteria include, but are not limited to, the following:

- 1) Age 30 – 80
- 2) No electrically powered devices in their body
- 3) Able to lie flat comfortably for at least 90 minutes

The purpose of this research study is to determine whether there are differences between people with and without ALS in brain NMDA receptor activity (a channel present in nerve cells of brain and spinal cord that regulates neurotransmission). Participation in this study will involve up to 3 visits (a screening and one to two imaging sessions). The MRI and PET scans are compensated at \$100 each and parking fees are waived.

Principal Investigator: Nazem Atassi, MD, MMSc

Contact:
Jianing Liu
617-726-1180
jliu45@mgh.harvard.edu

