Trial of BIIB100 for ALS

**Full Trial Name:** Double-Blind, Placebo-Controlled, Single Ascending Dose Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of BIIB100 Administered Orally to Adult Participants With ALS

**Trial Phase:** 1

We are doing this Phase I research study to find out about the safety and tolerability of the study drug BIIB100. BIIB100 is taken orally. This study is placebo-controlled, which means that some participants will receive study medication that contain no BIIB100.

The study is recruiting for patients with a diagnosis of Amyotrophic Lateral Sclerosis who have a slow vital capacity of \( \geq 65\% \).

Participation in the study will last for 6 weeks and will include a 2-night overnight stay at MGH in addition to 2-3 in-person visits. During the overnight stay, you will receive a single dose of the study medication.

There are additional inclusion/exclusion criteria that the study team will review with you if you are interested in participating. For more information regarding this trial, please contact one of the study coordinators. **Principal Investigator:** Dr. Katharine Nicholson. **Sponsor:** Biogen MA Inc. **Enrollment Contact:** Isabel Anez, 617-643-2499, ianezbruzual@mgh.harvard.edu; Yuriko Fukumura, 617-643-2522, yafukumura@mgh.harvard.edu

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Trial of BIIB078 for C9ORF72-ALS

**Full Trial Name:** Phase 1 Multiple-Ascending-Dose Study to Assess the Safety, Tolerability & Pharmacokinetics of BIIB078 Administered Intrathecally to Adults with C9ORF72-Associated ALS

**Trial Phase:** 1

We are doing this research study to find out about the safety and tolerability of the study drug BIIB078. This study is recruiting patients with C9ORF72-Associated ALS with a slow vital capacity greater than or equal to 50\% of predicted value. Participation in the study will last for approximately 52 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 if you are interested in participating. For information or to discuss how to participate, please contact study coordinator: **Principal Investigator:** Suma Babu, MD, MPH. **Sponsor:** Biogen MA Inc. **Enrollment Contact:** Stuti Shah, 617-643-5376, sshah58@mgh.harvard.edu or Yuriko Fukumura, 617-643-2522, yafukumura@mgh.harvard.edu

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Visit the MGH Healey Center Website: [http://www.massgeneral.org/als/](http://www.massgeneral.org/als/)

Then select **Research Opportunities** from the dropdown menu to read information about ALS trials and studies.

To receive eblasts about the Platform Trial, other NEALS ALS trials, and, clinical care at MGH, sign up for the Link:

[Sign up for ALS Link](http://www.massgeneral.org/als/)

To read updates about the Healey Platform Trial, select **Platform Trial** from the dropdown menu on opening screen.

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**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

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**Updated: August 2020**
Trial of BLZ945 for ALS

Full Trial Name: Open-label, adaptive design study in patients with ALS to characterize safety, tolerability and brain microglia response, as measured by TSPO binding, following multiple doses of BLZ945 using positron emission tomography (PET) with radioligand [11C]-PBR28

Trial Phase: 2

This Phase II research study is being done to study the safety and tolerability of a molecule called BLZ945 in patients with ALS. We also want to find out if BLZ945 is safe to take without causing too many side effects in ALS. Novartis is the sponsor of this study, and BLZ945 is taken orally. This research study will use an imaging method known as Positron Emission Tomography or PET to measure the effect of BLZ945 on a specific inflammatory cell type in the brain called microglia, which are activated in ALS. The study aims at providing important information on whether BLZ945 could be a potential treatment for patients with ALS and to help to select the most appropriate doses for the planning of future research in patients with ALS. This study is open label, which means that all participants receive study drug. Study participation will last at most, 70 days and include up to 5-7 hospital admission days at MGH. 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
Sponsor: Novartis
Enrollment Contact: Austin Lewis, 617-724-7928, alewis29@mgh.harvard.edu;

Trial of Ravulizumab for ALS

Full Trial Name: Phase 3, Double-Blind, Randomized, Placebo-Controlled, Parallel Group, Multicenter Study with an Open-Label Extension to Evaluate the Efficacy and Safety of Ravulizumab in Patients with Amyotrophic Lateral Sclerosis (ALS)

Trial Phase: 3

The purpose of this trial is to evaluate whether Ravulizumab (Ultomiris®) can slow ALS progression. It is administered intravenously about every 8 weeks. Participation in this trial will last up to 36 months, including a 1 year placebo-controlled trial followed by a 2 year open label extension. Please contact the study team to obtain additional information about the study visits and procedures. Some inclusion criteria include: over age 18, ALS diagnosis, onset of weakness within 3 years of screening, slow vital capacity (SVC) ≥ 65% of predicted value. Some exclusion criteria include: history of N. meningitidis infection, (Meningitis), active infection within 14 days, fever within 7 days. If you are interested in participating and think you might be eligible, please contact the trial team to discuss a screening visit.

Principal Investigator: James Berry, MD, MPH
Sponsor: Alexion Pharmaceuticals, Inc.
Enrollment Contact:
Reagan Church, 617-726-4284, rmchurch@mgh.harvard.edu;
Anthony Kostov, akostov@mgh.harvard.edu

Trial of BIIB067 for SOD1-ALS

Full Trial Name: A Study to Evaluate the Efficacy, Safety, Tolerability, Pharmacokinetics & Pharmacodynamics of BIIB067 Administered to Adult Subjects with Amyotrophic Lateral Sclerosis and Confirmed Superoxide Dismutase 1 Mutation

Trial Phase: 3

We are doing this research study to evaluate the clinical efficacy of the study drug BIIB067. The study is funded by Biogen MA Inc. The Principal Investigator at MGH is Dr. Suma Babu. This study is recruiting patients with SOD1-Amyotrophic Lateral Sclerosis (SOD1-ALS). Participation in the study will last for approx 32 weeks and will include 11-12 in person visits at MGH. 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 if you are interested in participating. For more information regarding this trial, please contact the study coordinator:

Principal Investigator: Suma Babu, MD, MPH
Sponsor: Biogen MA Inc.
Enrollment Contacts: Jenna Doherty, 617-643-0312, jchoderty30@mgh.harvard.edu, or, Yuriko Fukumura, 617-643-2522, yafukumura@mgh.harvard.edu
HEALEY ALS Platform
Trial Details

• Approximately 60 sites nationwide are conducting this trial.

• Approximately 480 participants will be enrolled into the first three regimens of the Platform Trial. As new study medications are added, additional participants will be enrolled.

• Participation in the trial will last for approximately 6 months and will include about 7 in-person visits.

• If eligible for the Platform Trial, each participant will provide informed consent to the Master Protocol and then be randomly assigned to one of the regimens.

• The study medication to placebo ratio is 3:1 across all regimens.

• Every participant who completes a regimen may have the option to receive the study medication in an open label extension.

• There are inclusion/exclusion criteria that the study team will review with you in more detail if you are interested in participating.

Information About
Future Regimens

More study medications are anticipated to be added to the HEALEY ALS Platform Trial, supported by pharma, foundation partners, philanthropy, federal and other fundraising initiatives.

To learn more about current and future regimens, please visit the website below:
www.massgeneral.org/neurology/als/research/first-platform-trial-treatments

To stay connected to the Healey Center at Mass General for ALS research and clinical care, sign up for the ALS Link:
https://lp.constantcontactpages.com/su/saTzwIp/ALSLink

Stay Connected to ALS Research Worldwide

To learn about ALS investigational drug trials and observational studies, please visit:
www.clinicaltrials.gov
www.neals.org
www.alsuntangled.com

Register for the National ALS Registry:
www.cdc.gov/als/ALSJoinALSRegistry.html

HEALEY ALS Platform Trial
Currently Enrolling Regimens

Updated August 2020
What is a Platform Trial?
A platform trial is a trial in which multiple study medications are tested at the same time in different participants using a master protocol and specialized statistical tools. This results in a more efficient and expedited trial. New regimens (study medications) can be added as they become available, which decreases or eliminates the gap in time between identification of a potential therapy and beginning a trial in humans.

Why Platform Trial?
Faster answers, More access, Less placebo, More learning about ALS.

Platform trials are designed to decrease the amount of time it will take to find successful therapies; improve study medication to placebo ratio; and increase access to participants by conducting the same trial at multiple research centers. The platform trial is perpetually open until successful treatments are discovered.

How does the Platform Trial work?
Participants in the Platform Trial will be randomly assigned to one of the trial regimens (study medications) that are available at the time of their enrollment. All current regimens in the Platform Trial will have a 3:1 study medication to placebo ratio for participants.

What is a Regimen?
A regimen is a study medication treatment that specifies the dosage, the schedule, and the duration of treatment. After informed consent to the master protocol, each participant will be randomly assigned to one regimen. Each regimen will follow the Master Protocol but may include additional activities and inclusion/exclusion criteria.

Initial Platform Regimens

Regimen A: Trial of Zilucoplan
This regimen is being conducted to see if zilucoplan is safe and effective in people diagnosed with ALS. This medication works by inhibiting tissue damage caused by the immune system.
Developed By: Ra Pharmaceuticals

Regimen B: Trial of Verdiperstat
This regimen is being conducted to see if verdiperstat is safe and effective in people diagnosed with ALS. This medication works by reducing neural inflammation.
Developed By: Biohaven Pharmaceuticals

Regimen C: Trial of CNM-Au8
This regimen is being conducted to see if CNM-Au8 is safe and effective in people diagnosed with ALS. This medication provides an energetic assist to impaired motor neurons and helps improve their ability to function normally.
Developed By: Clene Nanomedicine

How do I participate in the Platform Trial?
If you are interested in participating, please read the instructions in the box displayed in the previous column.
Study of Skin Biopsy/Stem Cells for Research in MND
+Amyotrophic Lateral Sclerosis
+Primary Lateral Sclerosis
+Healthy Volunteers

Full Trial Name: Stem Cells for Research in Motor Neuron Diseases (MND)
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
Sponsor: Harvard Stem Cell Institute

Enrollment Contact:
Ross Cimagala, 617-643-7828, rcimagala@mgh.harvard.edu

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Sign up for ALS Link

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For more information about these trials:

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Updated: August 2020
Study of SOD1 Kinetics
+ Amyotrophic Lateral Sclerosis
+ Asymptomatic SOD1-positive gene carriers

Full Trial Name: SOD1 Kinetics
Measurements in SOD1 Positive ALS Patients
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) and SOD1-positive asymptomatic gene carriers.
This study involves a 16-hour leucine infusion at Washington University in St. Louis, Missouri. Participation in this study will last approximately 4 months and requires 6 visits to MGH. Five 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
Sponsor: ALS Finding a Cure
Enrollment Contact: Isabel Anez, 617-643-2499, ianezbruzual@mgh.harvard.edu or Ross Cimagala, 617-643-7828, rcimagala@mgh.harvard.edu

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: Isabel Anez, 617-643-2499, ianezbruzual@mgh.harvard.edu; Austin Lewis, 617-724-7928, alewiss29@mgh.harvard.edu

Study of ALS Sample Repository (Living Library)
+ Amyotrophic Lateral Sclerosis
+ Healthy Volunteers
+ Non-ALS Neuro Disease Volunteers
+ Motor Neuron Disease Volunteers

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 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 hereditability 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: Ross Cimagala, 617-643-7828, rcimagala@mgh.harvard.edu, or, Katie Holmberg, 617-724-9196, kholmberg@mgh.harvard.edu
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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

Updated: August 2020
Study of SPINE-ALS
+Amyotrophic Lateral Sclerosis
+Healthy Volunteers

**Full Trial Name:** Positron Emission Tomography to Characterize in vivo Neuroinflammation in the Spinal Cord in People with 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 5 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 Investigators:** Suma Babu, MD, MPH
**Sponsors:** ALS Finding a Cure Foundation, Voyager Therapeutics

**Enrollment Contacts:**
Austin Lewis, 617-724-7928, alewis29@mgh.harvard.edu, or Dario Gelevski, 617-726-0563 dgelevski@mgh.harvard.edu

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Neuroinflammation (PBR28) Imaging Study
+Amyotrophic Lateral Sclerosis
+Primary Lateral Sclerosis
+Hereditary Spastic Paraplegia
+Healthy Volunteers (known carriers of ALS gene)
+Frontotemporal Dementia

**Full Trial Name:** Glial Activation Measured by PBR28-PET in People with Neurodegenerative Diseases

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.

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:** Suma Babu, MD, MPH
**Sponsors:** Neurodegenerative Disease Pilot Study Grant, K23 NS 083715, Evan and Arlene Yegelwel Endowed Fund for Primary Lateral Sclerosis Research and Care, PET Imaging of inflammation and epigenetics in ALS (ALS ONE), Muscular Dystrophy Association, Sundry

**Enrollment Contacts:**
Austin Lewis, 617-724-7928, alewis29@mgh.harvard.edu, or Dario Gelevski, 617-726-0563 dgelevski@mgh.harvard.edu
Study of Nutrition using mHealth App + Amyotrophic Lateral Sclerosis

Full Trial Name: The E-health Application To Modify Oral Energy Intake & Measure

Enroll in this study from your home!

Outcomes Remotely in ALS (EAT MORE 2)
Researchers at MGH have designed a mobile health app to help people with ALS calculate and track their ideal calories. The app is based upon prior MGH research which demonstrated that maintaining weight appeared to improve ALS disease progression and quality of life. The new ALS Nutrition app provides nutritional counseling tailored to individual needs, monitors weight, provides recommendations about how much and what foods to eat, and contains over 100 recipes created by the Registered Dietitians at MGH. People who download the app can use the app for free and they also have an option to share their data with MGH researchers.

To access the app, download the CareEvolution platform & search for the ALS Nutrition study:
- **Apple iPhone**: https://apps.apple.com/app/mydatahelps/id1286789190

Principal Investigator: Anne-Marie Wills MD, MPH
Sponsor: ALS Association
Enrollment Contact: Mansi Sharma, 617-643-2400, mghALsapp@partners.org
Or visit us at:
https://projects.iq.harvard.edu/alsnutrition

Visit the MGH Healey Center Website:
http://www.massgeneral.org/als/

Then select **Research Opportunities** from the dropdown menu to read information about ALS trials and studies.

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![Sign up for ALS Link](image)

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For more information about these trials:

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Updated: August 2020
**Study of Smartphone App for ALS**
+Amyotrophic Lateral Sclerosis

**New! Enroll in the study from your home. Email or call the coordinator listed below.**

**Full Trial Name:** Feasibility and Sensitivity of a Symptom Monitoring Application in Real Time (SMART) for Amyotrophic Lateral Sclerosis

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 with ALS will be in the study for about 12 months. The study is currently recruiting participants who meet the following guideline: Adults with Amyotrophic Lateral Sclerosis (ALS) to download and use the smartphone application using their smartphone device running iOS 8 or higher, or Android 4.1 or higher.

**Principal Investigator:** James Berry MD, MPH

**Sponsor:** ALS Finding a Cure

**Enrollment Contact:**
Ella Collins, 617-726-0981, ecollins15@mgh.harvard.edu or Minhaj Rahman, 617-726-1880, smrahman@mgh.harvard.edu

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**Study of Speech Motor Impairment in ALS**
+Amyotrophic Lateral Sclerosis, +Primary Lateral Sclerosis, +Healthy Volunteers

**Full Trial Name:** Speech Motor Impairments: Coordination of tongue, lips, and Jaw

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 and 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.

**Principal Investigator:** Jordan Green, MD

**Sponsors:** National Institutes of Health and the American Speech-Language-Hearing Foundation

**Enrollment Contact:**
Speech and Feeding Disorders Lab 617-724-6347
speechfeedinglab@mghihp.edu

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**Study of Typing in ALS**
+Amyotrophic Lateral Sclerosis
+Healthy Volunteers

**New! Enroll in the study from your home. Email or call one of the coordinators listed below.**

The purpose of this study is to see if a smartphone keyboard can identify unique typing patterns in ALS and serve as a quantifiable, digital biomarker of fine motor change over time in people with ALS. This is a 9-month long study, with visits every 3 months. The visits include standard questionnaires and outcome measures, including muscle and breathing tests. The study team will help you download and install the nQ software on your smartphone. We will ask you to keep the software on your smartphone throughout the duration of the study. Ownership of a smartphone and ~15 minutes of daily use is required.

**Principal Investigator:** James Berry, MD, MPH

**Sponsor:** nQ Medical

**Enrollment Contacts:** Ella Collins, 617-726-0981, ecollins15@mgh.harvard.edu or Minhaj Rahman, 617-726-1880, smrahman@mgh.harvard.edu

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**Study of Digital Quantitative Measurements**
+Amyotrophic Lateral Sclerosis
+Healthy Volunteers

The purpose of this study is to test the feasibility and utility of digital quantitative measurements for people with ALS. Specifically, we aim to investigate the utility of digital tools to quantify the neurological exam in clinic. We are also investigating the utility of digital tools to quantify behavior outside of clinic. This study is a week-long investigation looking at digital biomarkers. There is an in-clinic visit where we will obtain your consent to participate in the study and ask you to complete a number of tasks, including a digital neurological exam. The study team will give you an Apple Watch and an iPhone to collect digital information about you for one week. At the end of the week, you will be asked to return to the clinic for another digital neurological exam. Please contact us if you are interested in hearing more details and/or if you would like to know if you are eligible to participate in this study.

Stipend for completion of study: $150

**Principal Investigator:** James Berry, MD, MPH

**Enrollment Contacts:**
Ella Collins, 617-726-0981, ecollins15@mgh.harvard.edu or Minhaj Rahman, 617-726-1880, smrahman@mgh.harvard.edu