Healey & AMG Center
Sean M. Healey & AMG Center for ALS
at Massachusetts General Hospital

Massachusetts General Hospital
Founding Member, Mass General Brigham

Currently Enrolling Investigational Products Trials

UPDATED JUNE 2023

Trial of BIIB105 for ALS and polyQ-ALS

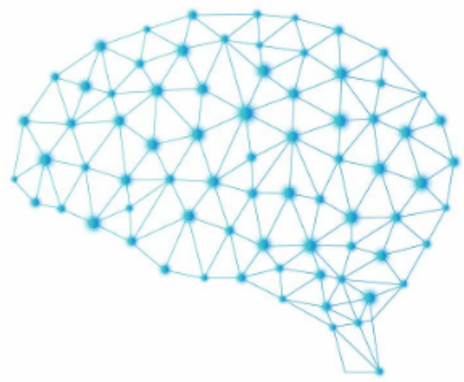
Sponsor: Biogen MA Inc.
Full Trial Name: A Phase 1 Multiple-Ascending-Dose Study to Assess the Safety, Tolerability, and Pharmacokinetics of BIIB105 Administered Intrathecally to Adults with Amyotrophic Lateral Sclerosis With or Without Poly-CAG Expansion in the Ataxin-2 Gene
Trial Phase: 1
Trial Length: 6-7 months (13 in-person visits)
Drug to Placebo Ratio: 2:1 or 3:1, open label extension (OLE) for 2 years
Target: ATXN2 protein
Science: BIIB105 is an antisense oligonucleotide (ASO) medication that may reduce the amount of ATXN2 protein. By decreasing ATXN2, this may prevent the accumulation of TDP-43 protein, which is responsible for the death of motor neurons.
Administration: Lumbar punctures (needle inserted into spinal fluid in the lower spine to administer dose)
Purpose: To learn about the safety and tolerability of BIIB105 in adults with a diagnosis of Amyotrophic Lateral Sclerosis (ALS) and to look at the level and action of the study drug in the body and what happens to this level over time.

Principal Investigator: Dr. Suma Babu
Enrollment Contacts: Erica Scirocco, escirocco@mgh.harvard.edu, 617-726-1363; Munaf Hatem, mhatem@mgh.harvard.edu, 617-643-3530

Trial of BrainGate

Full Trial Name: BrainGate: Feasibility Study of an Intracortical Neural Interface System for Persons with Tetraplegia
Trial Length: 13 months
Patients who have weakness due to motor neuron disease such as amyotrophic lateral sclerosis (ALS) and have no or limited use of their hands are needed for an FDA regulated research study to evaluate a new technology which may allow an individual with quadriplegia to control a computer cursor and assistive devices, like a robotic arm, by thought. This study is invasive and requires surgery. Research sessions are run at participants' residences, so to be eligible, participants must live within 3 hours drive of Boston, MA or Providence, RI.

Principal Investigator: Leigh Hochberg, MD, PhD
Enrollment Contacts:
clinicaltrials@braingate.org,
neurotechnology@mgh.harvard.edu



For more information:
Contact the research coordinator listed for studies you are interested in OR Judi Carey, Research Access Nurse, mghalsresearch@mgh.harvard.edu or 617-724-8995

Trial of Baricitinib for NADALS

Sponsor: Mark Albers, MD, PhD
Full Trial Name: Neurodegenerative Alzheimer's Disease and Amyotrophic Lateral Sclerosis (NADALS) Basket Proof of Concept Trial including Asymptomatic Individuals using Baricitinib
Trial Phase: Phase 1-2
Trial Length: Up to 28 weeks (Up to 7 in-person visits)
Drug to Placebo Ratio: No Placebo
Target: Type I interferon signaling
Science: Baricitinib aims to block type I interferon signaling, which is robustly active within the central nervous system of subsets of patients with Amyotrophic Lateral Sclerosis and Alzheimer's Disease. Type 1 interferon signaling is an immune response that promotes inflammation which can lead to motor neurons dying and the progression of ALS symptoms.
Administration: One 2 mg tablet once per day for the first 8 weeks of the trial, two 2 mg tablets once per day for the remaining 16 weeks. Tablets can be taken orally or crushed and administered through a G-tube
Purpose: In this study, the levels of baricitinib present in blood and cerebrospinal fluid (CSF) will be measured to determine safety and its effect on biomarkers related to ALS and AD. We hope these findings will help better evaluate the efficacy of baricitinib for the treatment of ALS.
Principal Investigator: Doreen Ho, MD
Sponsor: Mark Albers, MD, PhD
Enrollment Contacts: Kylie Graves, klgraves@mgh.harvard.edu, 617-643-7912; Chloe Noll, cnoll@mgh.harvard.edu, 617-724-7113

Things to Think About When Considering Participation in Clinical Trials

- **What phase is the trial?**
- **Why is this medication being tested in ALS?**
- **Is there a specific genetic target?**
- **How do I take the medication and how often?**
- **Does the trial have placebo?**
- **Does the trial have an open label extension?**
- **Am I allowed to take standard of care mediations while in this trial?**
- **What are the eligibility criteria of the trial?**
- **How long will I be in the trial?**
- **How many visits and how often will I have to come to the research center?**
- **How long are the visits and what happens at these visits?**
- **Can I participate in the trial remotely or at a research center closer to home?**
- **Are there any tests or procedures done during the trial?**
- **What are the potential benefits and risks of being in this clinical trial?**
- **How will participation in the trial affect my clinical care?**
- **Are there any reimbursements for participating in this trial?**

Trial of RAPA-501 Cell Therapy

Sponsor: Rapa Therapeutics, LLC
Full Trial Name: Phase I Trial of Autologous Hybrid TREG/Th2 Cell Therapy (RAPA-501) for Amyotrophic Lateral Sclerosis
Trial Phase: 1
Trial Length: Up to 1 year (10-30 in-person visits)
Drug to Placebo Ratio: Open Label (no placebo)
Target: T-cells
Science: In people with ALS, the body's immune system becomes imbalanced and appears to hasten the loss of motor neurons in the brain and spinal cord. Regulatory T-cells help reduce inflammation and could lead to a more balanced immune system in people with ALS. The goal of this study is to reduce neuroinflammation, potentially slowing ALS progression, using specially prepared regulatory T-cells, called RAPA-501 cells.

RAPA-501 cells are created through a series of steps by first taking the participant's own blood though a specialized IV (apheresis), then isolating regulatory T-cells from the blood. Next, these regulatory T-cells are grown under special conditions in a petri dish, becoming RAPA-501 cells. The RAPA-501 cells are then returned to the participant through an intravenous infusion. Prior to the IV infusion of RAPA-501 cells, a low dose of chemotherapy is given to reduce the body's immune response and potentially heighten the effects of the RAPA-501 cells.
Administration:
(1) Apheresis (blood separation) to collect T-cells
(2) Intravenous (IV) infusion of low-dose chemotherapy drugs (Cyclophosphamide and Pentostatin)
(3) Intravenous (IV) infusion of the specialized RAPA-501 cells
Purpose: To find out if RAPA-501 cell therapy is safe in people living with ALS. Two doses of RAPA-501 cells will be investigated for safety.
Principal Investigator: Dr. James Berry, MD, MPH
Enrollment Contact: Jacqueline Topping, jtopping@mgh.harvard.edu, 617-643-6036; Chloe Noll, cnoll@mgh.harvard.edu, 617-724-7113

Trial of SAR443820

Sponsor: Sanofi US Services Inc
Full Trial Name: A Phase 2, multicenter, randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of SAR443820 in adult participants with amyotrophic lateral sclerosis (ALS), followed by an open-label extension
Trial Phase: 2
Trial Length: 108 weeks
Drug to Placebo: 2:1, open label extension (OLE) for last 82 weeks
Target: RIPK1 receptor
Science: SAR44380 inhibits a receptor in your nervous system called RIPK1. When RIPK1 is activated, it results in inflammation and damage to your cells. Because SAR443820 works by blocking RIPK1, it may help reduce inflammation and damage to cells in your nervous system and interfere with the pathway causing ALS.
Administration: Oral pill taken twice daily
Purpose: To learn about the safety and efficacy of SAR443820 in adults with a diagnosis of Amyotrophic Lateral Sclerosis (ALS) and to look at the level and action of the study drug in the body and what happens to this level over time.
Principal Investigator: Dr. Doreen Ho
Enrollment Contacts: Amrita Iyer, aiyer2@mgh.harvard.edu, 617-643-9550; Chrysthie Moline, cmoline@bwh.harvard.edu, 617-643-2499

Trial of BLZ945 for ALS

Sponsor: Novartis
Full Trial Name: An 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
Trial Length: 6 months
Drug to Placebo Ratio: Open label (no placebo)
Target: Inhibitor of CSF-1R
Science: In people with ALS, immune cells in the brain called microglia are activated and cause neuroinflammation. The goal of this study is to reduce neuroinflammation through treatment of BLZ945. This will be evaluated using PBR28 PET imaging to measure microglial activation, as well as through collection of biomarkers of neuroinflammation and disease activity in participants with ALS.
Administration: Oral pill taken once per week or for the first four days of a two-week period
Purpose: To learn about the safety and tolerability of BLZ945 in adults with a diagnosis of Amyotrophic Lateral Sclerosis (ALS) and to help select the most appropriate doses for the planning of future research in patients with ALS.
Principal Investigator: Dr. Suma Babu
Enrollment Contacts: Henry Rutherford, hrutherford@bwh.harvard.edu, 617-643-6252; Jingqi Zhu, jzhu34@mgh.harvard.edu, 617-643-2522

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