Mass General Brigham

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

# Currently Enrolling Investigational Products Trials

*UPDATED FEBRUARY 2024*

## 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

## 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; Sean Morgan smorgan18@mgb.org 617-724-9196

**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 ION363 for FUS-ALS

**Sponsor:** Ionis Pharmaceuticals
**Full Trial Name:** A Phase 1-3 Study to Evaluate the Efficacy, Safety, Pharmacokinetics and Pharmacodynamics of Intrathecally Administered ION363 in Amyotrophic Lateral Sclerosis Patients with Fused in Sarcoma Mutations (FUS-ALS)
**Trial Phase:** 1-3
**Trial Length:** Up to 3 years and 11 months (up to 20 in- person visits)
**Participants:** People with FUS ALS
**Drug to Placebo Ratio:** 2:1 for 14 months, open label extension (OLE) for 20 months
**Target:** FUS RNA
**Science:** ION363 is an investigational antisense medicine targeting the FUS gene to reduce production
of the FUS protein. There is evidence that mutations in the FUS gene can lead to rapid, progressive loss of motor neurons in patients with FUS-ALS, so this drug may reduce or prevent disease progression in FUS-ALS patients.
**Administration:** Lumbar puncture (needle inserted into spinal fluid in the lower spine to administer dose)
**Purpose:** To evaluate the efficacy of the study drug in functioning and survival in ALS patients with FUS mutations.
**Principal Investigator:** Dr. Suma Babu
**Enrollment Contacts:** Munaf Hatem, mhatem@mgh.harvard.edu, 617-643-3530; Alison Wheeler, awheeler7@mgh.harvard.edu, 617-643-8449

## Trial of RAPA-501 Cell Therapy

**Sponsor:** Rapa Therapeutics, LLC

**Full Trial Name:** Phase 2/3 Trial of Autologous Hybrid TREG/Th2 Cell Therapy (RAPA-501) for Amyotrophic Lateral Sclerosis

**Trial Phase:** 2/3

**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.

**Administration:**
(1) Apheresis (blood separation) to collect T-cells
(2) Intravenous (IV) infusion of the specialized RAPA-501 cells

**Purpose**: To learn more about the efficacy and safety of RAPA-501 cell therapy in people living with ALS.

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

**Enrollment Contact:** Megan Okoro, mokoro@mgh.harvard.edu, 617-643-6252 or Timothy Royse, troyse@mgh.harvard.edu 617-643-4968

# 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?**

# Your Notes About Our Trials

# Stay Connected to ALS Research

Sign up for the MGH ALS Link to Stay Connected to Research:

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

View Currently Enrolling ALS Trials at the Healey Center:

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