Mass General Brigham

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

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Currently Enrolling Trials of Investigational Products

Trial of AMX0114

Sponsor: Amylyx Pharmaceuticals
Trial Name: A Phase 1, Randomized, Doubleblind, Placebo-controlled, Multiple Ascending Dose Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of the Antisense Oligonucleotide AMX0114
Phase: 1
Trial Length: 25 weeks
Participants: People with ALS aged 18+
Drug to Placebo Ratio: 3:1
Target: CAPN2 RNA
Science: AMX0114 is an investigational antisense oligonucleotide (ASO) medicine targeting the CAPN2 gene to reduce production of the Calpain-2 protein. There is evidence that calpain-2 is associated with processes known to cause neuronal injury and loss of axons which are attached to the motor neurons. By reducing Calpain-2 protein this drug may slow ALS progression.
Administration: Lumbar puncture (needle inserted into spinal fluid in the lower spine to administer dose); 4 doses every 4 weeks with an additional lumbar puncture for collection at the end of study
Purpose: To evaluate the safety and tolerability of the study drug in ALS patients
Principal Investigator: Dr. Sabrina Paganoni
Enrollment Contacts:
amx0114healey@mgb.org;
Mia Fleischer, 617-724-5659;
Grace Addy, 617-726-4282

Trial of VHB937

Sponsor: Novartis
Trial Name: A phase 2, randomized, double-blind, placebo-controlled parallel group study of VHB937 in Amyotrophic Lateral Sclerosis (ALS) over 40 weeks followed by an Open-label Extension (ASTRALS)
Phase: 2
Trial Length: 8 months followed by optional open label extension
Participants: Adults diagnosed with ALS
Drug to Placebo Ratio: 2:1
Target: Direct activation of microglia cells by TREM2
Science: This drug is expected to stabilize and activate TREM2, a protein in microglia cells. Microglia cells are affected by inflammatory diseases, such as ALS. Activation of TREM2 is thought to rebalance these microglia cells to a neuroprotective state, which would prevent further damage to neurons.
Administration: Intravenous infusion into a vein in your arm
Purpose: To study the tolerability, efficacy, and safety of VHB937 in participants with ALS.
Principal Investigator: Dr. James Berry, MD, MPH
Contact Information:
astralshealey@mgb.org
Caitlin Thomas, 617-643-7912
Shannon Chan, 617-643-4968

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:
Mia Fleischer
mfleischer@mgh.harvard.edu
617-724-5659

For more information:
Contact the clinical research coordinator(s) for studies of interest to you or Judi Carey, Research Access Nurse, 617-724-8995 or Michelle Redenz, ACE Nurse, 617-726-0034, or their email address: mghalsresearch@mgh.harvard.edu

Trial of RAPA-501

Sponsor: Rapa Therapeutics, LLC
Full Trial Name: Phase 2/3 Trial of Autologous Hybrid TREG/Th2 Cell Therapy (RAPA-501) for ALS
Trial Phase: 2/3
Trial Length: Up to 1 year in-person visits (5 – 8), 2 years remote follow-up visits (8)
Participants: Adults with ALS
Drug to Placebo Ratio: Open Label (no placebo)
Target: T-cells
Science: In people with ALS, the body's immune system becomes imbalanced, which may contribute to the loss of motor neurons in the brain and spinal cord. Regulatory T-cells, a specific type of immune cell, reduce inflammation. Scientists believe these cells may help to balance the immune system of people with ALS. The study utilizes a modified Regulatory T-cells, called RAPA-501 cells, to reduce neuroinflammation and potentially slow ALS progression. This process involves: (1) harvesting T-cells from the participants own blood through a process called apheresis, (2) reprogramming the harvested T-cells in special cell culture conditions to become RAPA-501 cells, (3) infusing the specialized RAPA-501 cells back into the participants bloodstream through an IV.
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 Contacts:
Megan Okoro 617-643-6252
mokoro@mgh.harvard.edu;

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

Your Notes About Our Trials

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 ALS 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?
- Do I have to become a clinic patient to participate in a trial at your center?
- 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?

View Currently Enrolling ALS Research at the Healey Center:

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

Register for Healey Community Webinars for Updates:

https://partners.zoom.us/webinar/register/WN_JW9rQBhTRFW5uoUIDtJguw#/registration

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

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