Mass General Brigham

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

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Currently Enrolling Investigational Products Trials

Trial of Prosetin

Company Name: ProJenX
Phase: 1
What is it?: A molecule designed to stop or slow the function of a protein known as a MAP4K.
Administration: Liquid in a syringe taken by mouth followed by drinking water
Drug to Placebo Ratio: There is a 3:1 ratio of active to placebo, meaning 75% of participants receive active treatment
Trial Length: Placebo-portion is approximately 28 days followed by ~13 months Open-Label Extension Treatment
Compensation: Participants will receive $556 total if they complete the study. If they do not complete the study, they will be compensated for their time for each visit that is completed.
Slice of Science: Prosetin is a MAP4Kinase inhibitor that has anti-inflammatory properties and can cross the blood-brain barrier. Prosetin blocks MAP4K4, a protein involved in ALS progression. By blocking MAP4K4, motor neurons may survive longer.
# of Visits, In-Person & Remote: Part C (if not entering the OLE) – 9 in-person visits; Part C (if entering the OLE) – 6 in-person visits; Part D – 19 in-person visits
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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:
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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:
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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:
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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