Currently Enrolling Investigational Products Trials

**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

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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:** Munaf Hatem, mhatem@mgh.harvard.edu, 617-643-3530; Alison Wheeler, awheeler7@mgh.harvard.edu, 617-643-8449

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
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 medications 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 Trials at the Healey Center:
https://www.massgeneral.org/neurology/als/research/als-clinical-trials

Register for Platform Trial Webinars for Updates:
https://partners.zoom.us/webinar/register/WN_JW9rOBhTRFW5uoUlDtjguw#/registration

Sign up for the MGH ALS Link to Stay Connected to Research:
https://lp.constantcontactpages.com/su/saTzwlp/ALSLink