Healey Center
Sean M. Healey & AMG Center for ALS at Mass General

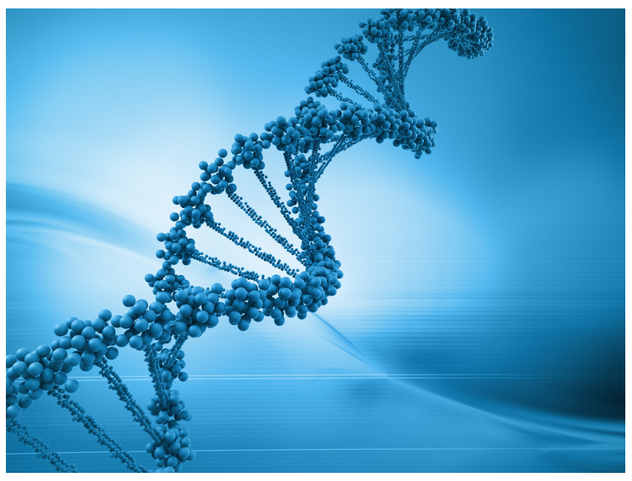
Massachusetts General Hospital
Founding Member, Mass General Brigham

Currently Enrolling Genetic ALS Clinical Research

UPDATED NOVEMBER 2022

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: Gabriel Jacobs, 617-726-3015, gjacobs@mgh.harvard.edu; Kush Mehta, 617-643-5376, kmehta9@mgh.harvard.edu



Study of DIALS

Full Study Name: Dominant Inherited ALS (DIALS) Network
Study Length: At least 5 years (annual visits with optional 6-month visits)
Participants: People who do not have any neurological symptoms, but have a first-degree relative with ALS caused by a mutation
Biomarkers: Blood, urine, and optional cerebrospinal fluid
Purpose: To study people at risk for developing ALS to further our understanding of underlying early disease changes. The information collected in this study may lead to development of treatments that target the earliest changes in ALS and allow for possible disease prevention.
Principal Investigator: James Berry, MD, MPH
Sponsor: ALS Finding a Cure, ALS Association, Philanthropy
Enrollment Contacts: Kathleen Diana, 617-724-6346, kdiana@mgh.harvard.edu; Allison Carey, 617-726-1559, abcarey@mgh.harvard.edu

For more information:
Contact the research coordinator listed for trial(s) you are interested in OR Judi Carey, Research Access Nurse, at jcarey8@partners.org or 617-724-8995

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 104 weeks
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: Gabriel Jacobs, 617-726-3015, gjacobs@mgh.harvard.edu; Kush Mehta, 617-643-5376, kmehta9@mgh.harvard.edu

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?**

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