Trial of ION363 for FUS-ALS

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
The purpose of this Phase 1-3 research study is to evaluate the clinical efficacy and safety of the drug ION363 in patients that have FUS-ALS. ION363 is administered intrathecally, which means that the drug will be administered through a lumbar puncture in the lower spine. The study consists of two parts: Part 1which is placebo-controlled (meaning that some participants will receive placebo, which looks like the study drug but does not contain any active ingredients), and part 2, which is an open-label extension period where all participants will receive the active drug.
This study is recruiting patients who have a confirmed diagnosis of Fused in Sarcoma Amyotrophic Lateral Sclerosis (FUS-ALS) with a slow vital capacity ≥ 50%.
Participation in this study will last approximately 2 years and 11 months. In Part 1 we will ask you to visit MGH **10** times during an 8-month time period. During this time, one visit will require an overnight stay and visits when the study medication is being administered will require you to stay at the site for at least 6 hours. In part 2, we will ask you to visit MGH 15 or 23 times, depending on which group you are randomly assigned to. Part 2 will be approx 2 years plus 3 months.
For more information, please contact the following study coordinators:
Principal Investigator: Dr. Suma Babu
Sponsor: Ionis Pharmaceuticals
Enrollment Contact: Gabriel Jacobs, 617-726-3015, gjacobs@mgh.harvard.edu Kush Mehta, 617-643-5376, kmehta9@mgh.harvard.edu

Connect to ALS Research at the Healey Center!

Sign up for the MGH ALS Link:

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

View currently enrolling ALS trials:

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

For more information about these trials:

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

MASSACHUSETTS GENERAL HOSPITAL
NEUROLOGICAL CLINICAL RESEARCH INSTITUTE

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

Currently Enrolling
Genetic ALS Clinical Research

Updated: June 2022

Trial of BIIB105 for ALS and polyQ-ALS

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 WithoutPoly-CAG Expansion in the *Ataxin-2* Gene **Trial Phase:** 1

The purpose of this Phase 1 research study is to learn about the safety and tolerability of the study drug BIIB105 in adults with a diagnosis of Amyotrophic Lateral Sclerosis who have a slow vital capacity of ≥60%. This study will also look at the level and action of the study drug in your body, and what happens to this level over time. BIIB105 is administered intrathecally (via lumbar puncture into the lower spine). This study is placebo-controlled, which means that some participants will receive placebo, which looks like the study drug but does not contain any active ingredients.

Participation in the study will last for approximately 29 weeks, including a 4-week screening period, 13-week treatment period and a 12-week follow-up period. During the treatment period, participants will receive 3 loading doses of BIIB105 every 2 weeks, followed by 2 doses administered once every 4 weeks, for a total of 5 doses. Please contact one of the coordinators.

Principal Investigator: Dr. Suma Babu
Sponsor: Biogen MA Inc.
Enrollment Contact: Mackenzie Keegan, 617-643-6252, mkeegan@mgh.harvard.edu; or Gabriel Jacobs, 617-726-3015, gjacobs@mgh.harvard.edu

Study of DIALS

+ Asymptomatic first-degree adult relatives of people with familial ALS

Full Trial Name: Dominant Inherited ALS (DIALS) Network

This study is recruiting participants who do not have any neurological symptoms, but who havea first-degree relative with ALS caused by a mutation.

Principal Investigator: Katharine Nicholson MD
Sponsor: ALS Finding a Cure, Target ALS, ALS Association, American Academy of Neurology/Muscular Dystrophy Association
Enrollment Contact: Kathleen Diana, 617-724-6346, kdiana@mgh.harvard.edu; Allison Carey, 617-726-1559, abcarey@mgh.harvard.edu

Trial of TPN-101 for Patients with C9orf72

Full Trial Name: A Phase 2a Study of TPN-101 in Patients with C9ORF72 ALS/FTD (Amyotrophic Lateral Sclerosis and/or Frontotemporal Dementia)
Trial Phase: 2

The purpose of this study is to assess the safety and tolerability of the drug TPN-101 in patients with *C9orf72* ALS and/or FTD. This study will also measure the levels and efficacy of the drug in your body over time. TPN-101 is a gel capsule that is taken orally. This study is randomized and placebo-controlled, meaning that some patients may receive an inactive drug that looks identical to TPN-101. Patients will beasked to participate in this study for 58 weeks. Including a 6-week screening period, 6-month treatment, 6-month Open-Label, and a follow-up visit 4 weeks after Open-Label. During the treatment period, patients will take either TPN-101 or placebo daily. For Open-Label, all participants will receive the investigational drug, TPN-101. For more information, please contact the study team.

Principal Investigator: Dr. Doreen Ho
Sponsor: Transposon Therapeutics, Inc.
Enrollment Contact: Isabel Cepeda, 617-726-1880, icepeda@mgh.harvard.edu

Trial of LAM-002A for C9orf72-Associated ALS

Full Trial Name: A Phase 2a Trial to Evaluate the Safety, Tolerability, and Biological Activity of LAM-002A (apilimod dimesylate capsules) in C9ORF72-Associated ALS
Trial Phase: 2a

The aim of this Phase 2 research study is to find out if LAM-002A is a safe treatment option for patients with C9ORF72-associated ALS (C9ALS). During the study, participants will receive the LAM-002A drug orally in pill form. This is an open label extension trial, meaning that for the first 12 weeks, some participants will receive a placebo (which looks like the study drug, but has no active ingredients) instead of LAM-002A. Following the completion of weeks 1-12, all participants will be eligible to receive LAM-002A for the next 12 weeks of the trial. Participation in this study will involve blood draws, lumbar punctures, and several clinical measures of ALS, as research staff seek to understand how LAM-002A is processed by the body and if it impacts ALS progression. Our team is looking for patients with C9ALS and a slow vital capacity of ≥50% who are willing to complete 10 study visits and several phone calls over 28 weeks. Please reach out to the enrollment contact listed below:

Principal Investigator: Suma Babu, MD, MPH
Sponsor: AI Therapeutics, Inc.
Enrollment Contact: Madeline Zarro, 617-726-1363, mzarro@mgh.harvard.edu