

## Trial of RAPA-501 Cell Therapy for ALS

**Full Trial Name:** Phase I Trial of Autologous Hybrid TREG/Th2 Cell Therapy (RAPA-501) for Amyotrophic Lateral Sclerosis

### Trial Phase: 1

We are doing this Phase I, three-cohort research study to find out if RAPA-501 cell therapy is safe in patients with ALS. This is an open label study, which means that there is no placebo, and all participants will receive actual RAPA-501 cell infusions. Two doses of RAPA-501 cells will be investigated for safety. In addition, if RAPA-501 cells are found to be safe, additional patients will receive RAPA-501 cells in combination with a low dose of two chemotherapy agents. The study is looking for patients with a diagnosis of Amyotrophic Lateral Sclerosis who have a forced vital capacity of  $\geq 50\%$ . In this study, participants' white blood cells (T-cells) will be removed during a procedure called Apheresis (a type of blood filtering). The T cells will be made into the specialized RAPA-501 cells and re-infused through an I.V. Total study participation will last about 1 year; 6 months of treatment (up to 19 visits to MGH) and 6 months of follow-up (up to 3 visits to MGH). Please contact the study team to obtain additional information about study visits and procedures and to review specific criteria.

**Principal Investigator:** Dr. James Berry

**Sponsor:** Rapa Therapeutics, LLC

**Enrollment Contact:** Kelly Fisher, 617-726-9094, [kefisher@mgh.harvard.edu](mailto:kefisher@mgh.harvard.edu)



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<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 listed for trial(s) you are interested in OR Judi Carey, Research Access Nurse, at [jcarey8@partners.org](mailto:jcarey8@partners.org) or 617-724-8995



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## Currently Enrolling Trials of Investigational Products



**Healey Center**

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



Updated: June 2022

### **Trial of Reldesemtiv**

**Full Trial Name:** A Phase 3, Multi-Center, Double-Blind, Randomized, Placebo-Controlled Trial to Evaluate the Efficacy and Safety of Reldesemtiv in Patients with Amyotrophic Lateral Sclerosis (ALS)

#### **Trial Phase: 3**

The purpose of this study is to assess the effect of the investigational drug, reldesemtiv, on disease progression and muscle weakness in people living with ALS. Reldesemtiv is a tablet that is taken by mouth. This study is randomized and placebo-controlled, meaning that some participants may receive an inactive drug (or placebo) that looks identical to reldesemtiv. Participants will be asked to participate in this study for up to 54 weeks; this study has up to 8 in-person visits and up to 9 remote visits via phone or video call. The trial includes a 2-week screening period, 24-week placebo-controlled period, 24-week active drug treatment period, and a follow-up visit 4 weeks after the active drug treatment period. During the placebo-controlled period, participants will take either reldesemtiv or placebo twice daily. For the active drug treatment period, all participants will receive investigational drug, reldesemtiv. Please contact the study team listed below.

**Principal Investigator:** Sabrina Paganoni, MD, PhD

**Sponsor:** Cytokinetics, Inc.

**Enrollment Contacts:** Isabel Cepeda, 617-726-1880, [icepeda@mgh.harvard.edu](mailto:icepeda@mgh.harvard.edu); Max Higgins, 617-643-2522, [mphiggins@mgh.harvard.edu](mailto:mphiggins@mgh.harvard.edu)

### **Trial of BLZ945 for ALS**

**Full Trial Name:** Open-label, adaptive design study in patients with ALS to characterize safety, tolerability and brain microglia response, as measured by TSPO binding, following multiple doses of BLZ945 using positron emission tomography (PET) with radioligand [11C]-PBR28

#### **Trial Phase: 2**

This research study is being done to study the safety and tolerability of a molecule called BLZ945 in patients with ALS. We also want to find out if BLZ945 is safe to take without causing too many side effects in ALS. Novartis is the sponsor of this study, and BLZ945 is taken orally. This research study will use an imaging method known as Positron Emission Tomography or PET to measure the effect of BLZ945 on a specific inflammatory cell type in the brain called microglia, which are activated in

ALS. The study aims at providing important information on whether BLZ945 could be a potential treatment for patients with ALS and to help to select the most appropriate doses for the planning of future research in patients with ALS. This study is open label, which means that all participants receive study drug. Study participation will last, at most, 70 days and include up to 5-7 hospital admission days at MGH. Please contact the study team to obtain additional information.

**Principal Investigator:** Suma Babu, MD, MPH

**Sponsor:** Novartis

**Enrollment Contact:** Austin Lewis, 617-724-7928, [alewis29@mgh.harvard.edu](mailto:alewis29@mgh.harvard.edu); Mackenzie Keegan, [mkeegan@mgh.harvard.edu](mailto:mkeegan@mgh.harvard.edu), 617-643-6252



### **Trial of BrainGate**

+Amyotrophic Lateral Sclerosis

**Full Trial Name:** BrainGate: Feasibility Study of an Intracortical Neural Interface System for Persons With Tetraplegia

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. The clinical trial requires a commitment of 13 months.

**Principal Investigator:** Leigh Hochberg, MD PhD

**Enrollment Contact:** [clinicaltrials@braingate.org](mailto:clinicaltrials@braingate.org), [neurotechnology@mgh.harvard.edu](mailto:neurotechnology@mgh.harvard.edu)

### **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 Without Poly-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  $\geq 60\%$ . 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. For more information, 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](mailto:mkeegan@mgh.harvard.edu); or Gabriel Jacobs, 617-726-3015, [gjacobs@mgh.harvard.edu](mailto:gjacobs@mgh.harvard.edu)

