UC San Diego

ALS Clinical Trials & Research

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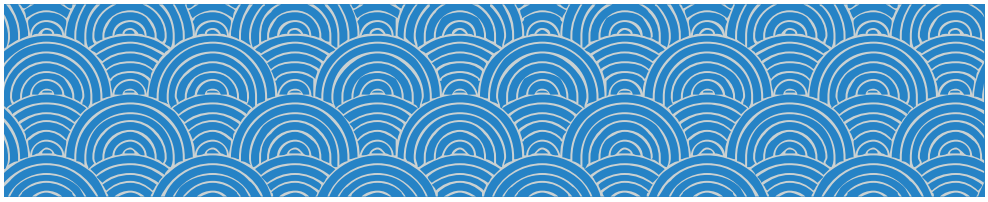

Sponsor: Biogen

An Efficacy, Safety, Tolerability, Pharmacokinetics and Pharmacodynamics Study of BIIB067 in Adults With Inherited Amyotrophic Lateral Sclerosis (ALS)(VALOR (Part C))

Description

The primary objective of this study is to evaluate the clinical efficacy of BIIB067 administered to adult participants with ALS and confirmed superoxide dismutase 1 (SOD1) mutation. The secondary objectives are to evaluate the safety, tolerability, and pharmacodynamic (PD) effects of BIIB067 administered to adult participants with ALS and confirmed SOD1 mutation. For further information please search Clinical Trials.gov Identifier: NCTO2623699.

Key Inclusion Criteria

- Diagnosis of ALS with Confirmed SOD1 Mutation (Familial ALS)
- SVC ≥ 65 %
- No implanted ports or catheters
- If taking Riluzole, must be on stable dose for ≥ 30 days
- If taking Edaravone, must be on stable dose for ≥ 60 days
- Deemed as Fast-Progressor by SOD1 specific mutation or ALSFRS slope (determined by study parameters after review)
- Able to undergo Lumbar Puncture (drug route intrathecal)

Please contact study team for further eligibility criteria, recruitment status and clinical trial information.

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Sponsor: Biogen

A Study to Assess the Safety, Tolerability, and Pharmacokinetics of BIIB078 in Adults with C9ORF72-Associated Amyotrophic Lateral Sclerosis

Description

The primary objective of this study is to evaluate the safety and tolerability of BIIB078 in adults with C9ORF72-Amyotrophic Lateral Sclerosis (ALS). The secondary objectives of this study are to evaluate the pharmacokinetic profile of BIIB078 and to evaluate the effects of BIIB078 on clinical function. As the first-in-human study, the study enrolls a small number of participants in each cohort. Every participant in a cohort is treated with the same dose or placebo. The study is designed to evaluate and confirm the safety of each dose before enrolling and exposing new participants to a higher dose on the next cohort. Therefore, proceeding from cohort to cohort, the recruitment status of "Active, not recruiting" may change. For further information please search ClinicalTrials.gov Identifier: NCT03626012.

Key Inclusion Criteria

- Diagnosis of ALS and confirmed C9ORF72 Mutation (Familial ALS)
- SVC ≥ 50 %
- If taking Riluzole, must be on stable dose for ≥ 30 days
- If taking Edaravone, must be on stable dose for ≥ 60 days
- No Implanted Port or Catheter
- Informant/Caregiver Able to Attend Study Visits
- Fast/Moderate Progressors – Determined by ALSFRS Slope
- Must Pass ALS Cognitive Behavioral Screen at Screening

Please contact study team for further eligibility criteria, recruitment status and clinical trial information.

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Sponsor: Biogen

A Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of BIIB100 Administered Orally to Adults with Amyotrophic Lateral Sclerosis

Description

The primary objective of this study is to evaluate the safety, tolerability of single-ascending doses of BIIB100 in adults with amyotrophic lateral sclerosis (ALS). The secondary objective of the study is to characterize the pharmacokinetic profile of BIIB100. For further information please search ClinicalTrials.gov Identifier: NCT03945279.

Key Inclusion Criteria

- Diagnosis of ALS
- If taking Riluzole, must be on stable dose for ≥ 30 days
- If taking Edaravone, must be on stable dose for ≥ 60 days
- No Implanted Port or Catheter
- Significant Cognitive Impairment
- Weight ≥ 132.2 lbs
- SVC ≥ 65 %
- Treatment with drugs that are transported by Breast Cancer Protein (BCRP) and P-glycoprotein (P-gp) including, but not limited to, rosuvastatin, sulfasalazine, dabigatran, digoxin and fexofenadine

Please contact study team for further eligibility criteria, recruitment status and clinical trial information.

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Target ALS

Bio-Fluid Biomarker – Blood Biorepository

Description

The purpose of this study is to test what changes happen in participants with ALS that can be seen in blood. These changes are called biomarkers. Biomarkers may help us diagnose and monitor ALS more easily and could potentially help advance ALS drug development.

The primary long-term goals of the repository are to learn how to better understand, prevent, diagnose or treat ALS and other diseases.

Inclusion Criteria

- Patients with Diagnosis of ALS
- Patients without Diagnosis of ALS as Controls

Key Study Procedures

This study will include a one-time blood draw of about 5 tablespoons of blood.

Note: Study Procedures can be done at home visit.

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UCSD – John Ravits, MD, FAAN

Neurodegenerative Disorders Blood Biorepository

Description

The purpose of this study is to create a repository (bank) of blood samples from people with neurodegenerative diseases, so that we can study these diseases. The cause of the various neurodegenerative diseases is unknown even though we have studied them for several decades. While science has the ability to study these diseases, direct access to human samples with these diseases is vitally important and in short supply. Since often important research questions and opportunities cannot be known ahead of time, it is important that samples are collected and stored in a way that preserves them so they can then be drawn upon when the research questions or opportunities arise. This will allow samples to be used over and over again and compared with previous studies, and shared by other researchers to get as much research value out of them as possible.

Inclusion Criteria

- Patients with Diagnosis of ALS
- Patients without Diagnosis of ALS as Controls

Key Study Procedures

This study will include a one-time blood draw of 60ml -250 ml.

Note: Study procedures can be done at home visit.

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UCSD – John Ravits, MD, FAAN

Biorepository of Neurodegenerative Diseases Central Nervous Systems

Description

The cause of the various neurodegenerative diseases is unknown despite several decades of scientific research. The general purpose of this study is to investigate them directly in the human central nervous system (CNS), which is the brain and spinal cord. The specific purpose of this study is to create a repository (or bank) of them from people with neurodegenerative diseases so that this can be done. While science has the ability to study these diseases, direct access to human samples with these diseases is vitally important and in short supply. Since often important research questions and opportunities cannot be known ahead of time, it is important that samples are collected and stored in a way that preserves them so they can then be drawn upon when the research questions or opportunities arise. This will allow samples to be used over and over again and compared with previous studies, and shared by other researchers to get as much research value out of them as possible.

The purpose of this study is to create a central nervous system (brain & spinal cord) biorepository – after death. All transport arrangements pre and post donation will be completed by study coordinator.

Inclusion Criteria

- Patients with Diagnosis of ALS
- Patients without Diagnosis of ALS as Controls

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