MS CENTER CLINICAL RESEARCH

The UCSF MS Center is an internationally recognized leader in multiple sclerosis clinical research. We conduct clinical trials involving the use of experimental treatments, as well as observational studies that help better understand the natural history of the disease. We are trying to understand what role genes, the environment and the immune system have in multiple sclerosis and other neuroimmunological diseases. Although our primary clinical research focus is on MS, we also study other diseases such as neuromyelitis optica (NMO), optic neuritis, transverse myelitis, encephalitis and sarcoidosis.

Clinical research studies include only individuals who choose to participate in them. If you are interested in research conducted at our Center, please inform your neurologist or nurse. If you are currently a patient of the UCSF MS Center, some of these studies may be completed during your clinic visits. Some research studies are open for enrollment even if you do not receive your neurological care at UCSF. Should you be interested in a research study, you can be given an unsigned copy of the study consent form to think about and discuss with anyone before making your decision about entering the study. Please take your time to decide whether you would like to participate, and ask the study doctor or study staff to explain any words or information in the consent form that you do not clearly understand.

As of August 2014, the UCSF MS Research Center is conducting the following experimental studies:

Clinical Trials of Disease Modifying Treatments

Open to enrollment for patients with relapsing multiple sclerosis

Vitamin D - RCT Study

A randomized controlled trial of vitamin D in multiple sclerosis

This research is being done to see if giving a high dose of vitamin D to people with multiple sclerosis (MS) makes the disease better. Studies have suggested that people with MS who have lower vitamin D levels have more attacks of MS. It is not known if giving extra vitamin D to people with MS helps make the disease better or not.

Some doctors of people with MS give them vitamin D already, although they don't know if it is helpful or not. The Institute of Medicine recommends that every person take 600 international units (IU) of vitamin D each day. The study doctors want to know if giving a higher dose of vitamin D, 5,000 IU each day can help lower the risk of MS attacks or MS worsening.

All participants will receive an approved treatment for MS called glatiramer acetate (Copaxone). Half of the people will receive standard-dose vitamin D (600 IU), and half will receive high-dose vitamin D (5,000 IU).

Participation in this study will last for two years.

If you have been diagnosed with a relapsing form of MS, are between the ages of 18-50 and are able to come to UCSF for study visits you may be eligible to participate.

For more information, please contact Nisha Raj Revirajan at RevirajanN@neurology.ucsf.edu (Subject: 'Vitamin D RCT'), or call 415-502-7220.

ReBUILD

A Randomized, Double-Blind, Parallel-Group, Placebo Controlled Crossover Trial to Assess the Efficacy, Safety, Tolerability, and Pharmacokinetics of an Identified Small Molecule as a Remyelinating Agent in Multiple Sclerosis

This Phase II clinical trial will assess the efficacy, safety, and tolerability of an identified compound in the promotion of remyelination in patients with relapsing-remitting multiple sclerosis. This investigational drug was approved for the treatment of allergic rhinitis by the Food and Drug Administration (FDA) in past years. Since then, the medication has been identified at UCSF as a compound that potentially enhances oligodendrocyte differentiation and remyelination. However, it is not approved as a therapy for multiple sclerosis.

Study participants will be asked to come to the UCSF MS Center at least 5 times over approximately 5-6 months. Participation will include an initial screening visit to determine eligibility, a baseline visit, and 3 follow-up visits (at 1, 3, and 5 months after the baseline visit). All study visits will occur at the UCSF campus. Most visits will take about 3 hours.

Study participation will involve taking the study drug by mouth, twice each day, or the equivalent amount of placebo. Participants will be randomized into one of two groups: Group A and Group B. Group A will receive 3 months of active drug followed by 2 months of placebo while Group B will receive 3 months of placebo followed by 2 months of the active drug. Patients in this study can remain on their standard disease modifying treatment during the course of the study. However, patients cannot participate in any other investigational new drug research study for which drug effects on remyelination are unknown.

For more information, please contact study coordinator Sam Arnow at Samuel.Arnow@ucsf.edu, or call 415-353-2707.

RADIANCE Study

A Phase 2/3, Multi-Center, Randomized, Double-blind, Placebo-controlled (Part A) and Double-blind, Double-dummy, Active-controlled (Part B), Parallel group study to evaluate the efficacy and safety of RPC1063 administered orally to relapsing Multiple Sclerosis patients.

The purpose of this research study is to determine if the experimental drug, RPC1063, is safe and effective in the treatment of relapsing multiple sclerosis (MS), compared to an FDA approved standard treatment of IFN β-1a (Avonex®) 30 µg.

UCSF will participate in Part B of the study only.

Two different doses of RPC1063 will be tested against Avonex®. Patients will be assigned to receive one of the following three study drugs and the corresponding placebo:

0.5mg RPC1063 (oral capsule) – and placebo injection

1 mg RPC1063 (oral capsule) – and placebo injection

IFN β-1a (Avonex® - 30 µg injection) and placebo capsules

Patients will have a 67% (2 out of 3) chance of being assigned to one of the active doses of RPC1063 and 33% (1 out of 3) chance of being assigned to Avonex®.

In addition, because this study is double blinded, you will also be "treated" with a placebo. A placebo is one that contains no active substance but may resemble a real medication. Since Avonex® is administered by injection, and RPC1063 is administered orally (capsule), patients enrolled in this study will be required to receive both types of delivery (orally and injection) so that no one will be able to tell which active medication the patient is truly being given. This study is completely blinded, meaning, you, your doctor, and the Sponsor, Receptos, Inc. will not know if you"re getting RPC1063 or Avonex® until the entire study is concluded for all subjects.

Participation in this study will last for approximately 28 months and include a 2 year treatment period.

If you have been diagnosed with a relapsing form of MS, are between the ages of 18-55, have not been treated with natalizumab, fingolimod or other SIP1R agonists and are able to come to UCSF for study visits you may be eligible to participate.

For more information, please contact Nancy Shum at Nancy.Shum@ucsf.edu (Subject: 'Radiance Inquiry'), or call 415-502-7219.

Observational Studies

Open to enrollment for MS patients

EPIC Study

Multiple Sclerosis Genetics- Expression, Proteomics, Imaging, Clinical

The purpose of this study is to identify the genes that influence a person's susceptibility to MS and the genes that influence a person's response to treatments and medicines prescribed for MS. This study involves over 500 participants at UCSF. MS has a complex pattern of inheritance. In order to which genes may be involved, large numbers of individuals are being studied. DNA extracted from blood cells is analyzed to find genes that may be markers for the presence of MS or markers of treatment response. It is important to screen unaffected family members as well as individuals without MS to determine how these genes are distributed. Another purpose of this study is to determine whether recent improvements in magnetic resonance imaging (MRI) techniques can provide doctors with more information addressing the question of why the manifestations of MS are different in different individuals. By comparing neuroimaging with clinical course and genetic variables it will be possible to make correlations between different phenotypes (clinical characteristics that can be seen) and genotypes (internal genetic "make-up") of MS.

For more information, please visit our study website or contact Cuquita Gomez at Refujia.Gomez@ucsf.edu (Subject: 'EPIC Study'), or call 415-502-7197.

Visual Evoked Potentials (VEP) in the Evaluation of Neurological Disease

The purpose of this study is to determine how visual evoked potential testing, a non-invasive way to measure nerve function, correlates with neurological disease. Participation in the study will take a total of about 1 hour or less. People with neurological disease as well as healthy controls may be eligible to participate.

We are particularly interested in evaluating people with progressive multiple sclerosis (primary or secondary).

For more information, please contact Christopher Songster at Christopher.Songster@ucsf.edu, or call 415-353-2273.

Neuroimmunology Banking Studies

Banking of Cerebrospinal Fluid and/or Peripheral Blood from patients with Multiple Sclerosis, Neuromyelitis Optica and other neurological diseases or blood from healthy subjects

The purpose of this study is to collect samples that may aid in better understanding disease mechanisms of inflammation of the brain and spinal cord. In addition, these samples may help identifying markers relevant to such diseases. Blood from patients or healthy donors will be collected. CSF will be strictly obtained during procedures for the diagnostic workup of patients in the Department of Neurology at UCSF. Blood will be mainly obtained during diagnostic workup but may also be obtained for research purposes only. A portion of the samples that are collected as a part of this study may be used now or in the future for research purposes with the goal to have a constant flow of materials from human subjects required for basic laboratory research.

Participation in this study may include: drawing a blood sample by a UCSF staff member and 5 to 6 tablespoons of blood will be taken. You may donate blood up to 6 times in 3 years. If you consent to donate cerebrospinal fluid (CSF), we will ask to take up to a total of 30 ml (6 teaspoons) of your CSF including the portion required for diagnostics and the portion to be used for research. Your primary care-taker will inform you about the lumbar puncture (LP) procedure. This procedure does not apply to healthy control subjects. You may donate CSF at each diagnostic LP.

If you have been diagnosed with multiple sclerosis, neuromyelitis optica or another neurological disorder and you are a patient being seen at the UCSF MS Center, you may be able to participate. If you do not have a neurological disorder and live in the Bay area, you may be able to participate as a control.

For more information, please contact Max Kazer at Max.Kazer@ucsfmedctr.org (Subject: 'Banking Study Inquiry'), or call 415-502-7204.

Genetic Susceptibility to Develop MS

Multiple Sclerosis (MS) is the most common acquired disease of the central nervous system in young adults. Genes, the fundamental units of heredity, likely play a role in determining who is at risk for developing MS, how the disease progresses, and how someone responds to therapy. The goal of this study is to identify and characterize the repertoire of genes that may affect the risk of a person developing MS.

Recent technological advances together with a better understanding of the human genome are opening new and promising opportunities to unravel the genetic basis of MS. Our strategy for fueling gene discovery in MS relies on the meticulous scanning of the entire genome of patients, their relatives, and unrelated controls unaffected by the disease. Due to the complex nature of MS, a large number of participants are needed to accelerate discovery.

If you have been diagnosed with MS, have a family member diagnosed with MS, or are healthy and could serve as a control, you may be eligible to participate.

Participation in this study will take approximately three hours and will require a blood draw.

This is a nationwide study; participation does not require that you live near San Francisco, CA (except for healthy unrelated controls).

To participate in this study or to request additional information, please complete this brief survey.

Observational Studies

Open to enrollment for patients with neuromyelitis optica or neurosarcoidosis

Neuromyelitis Optica

T Cell Recognition of CNS Autoantigens in Multiple Sclerosis and Neuromyelitis Optica

Multiple sclerosis (MS) and neuromyelitis optica (NMO) are believed to be autoimmune conditions in which the immune system (which normally helps fight infections) causes damage to the nervous system. Recent discoveries have shed new light onto how this may be occurring, but the process is still not completely understood. Newer studies have also suggested that intestinal bacteria may also be involved in these conditions. This study is being conducted to get a better understanding of how certain types of white blood cells (T cells) and intestinal bacteria may be involved in MS and NMO.

If you have been diagnosed with MS, NMO, or if you are healthy, and you are above the age of 13, you may be eligible to participate in this study. If you are eligible, and if you agree to take part in this research study, we would like to collect a blood sample, and an optional stool sample. By studying these specimens, we hope to learn more about what causes MS and NMO, to expand our knowledge of what happens during the course of these diseases, and hopefully to find better treatments in the future. Participation in the study should take less than two hours.

If you would like more information, please contact Dr. Scott Zamvil (Principal Investigator) at zamvil@ucsf.neuroimmunol.org or 415-502-7395, or Collin Spencer (Study Coordinator) at cspencer@ucsf.neuroimmunol.org or 415-502-7268.

You may also visit the Zamvil Laboratory website.

Neuroimmunology Banking Studies

Banking of Cerebrospinal Fluid and/or Peripheral Blood from patients with Multiple Sclerosis, Neuromyelitis Optica and other neurological diseases or blood from healthy subjects

The purpose of this study is to collect samples that may aid in better understanding disease mechanisms of inflammation of the brain and spinal cord. In addition, these samples may help identifying markers relevant to such diseases. Blood from patients or healthy donors will be collected. CSF will be strictly obtained during procedures for the diagnostic workup of patients in the Department of Neurology at UCSF. Blood will be mainly obtained during diagnostic workup but may also be obtained for research purposes only. A portion of the samples that are collected as a part of this study may be used now or in the future for research purposes with the goal to have a constant flow of materials from human subjects required for basic laboratory research.

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If you have been diagnosed with multiple sclerosis, neuromyelitis optica or another neurological disorder and you are a patient being seen at the UCSF MS Center, you may be able to participate. If you do not have a neurological disorder and live in the Bay area, you may be able to participate as a control.

For more information, please contact Max Kazer at Max.Kazer@ucsfmedctr.org (Subject: 'Banking Study Inquiry') or call 415-502-7204.

Sarcoid

Neurological Manifestations of Sarcoidosis and Ways to Distinguish Sarcoidosis from other Causes of Nervous System Inflammation

The purpose of this study is to determine how sarcoidosis and neurosarcoidosis affect the nervous system. The study is observational and may involve some or all of the following: medical record review, an in-person evaluation, brain MRI, retinal scans, a blood draw and review of surplus pathological (biopsy) specimens.

People with biopsy-proven sarcoidosis or suspected neurosarcoidosis may be eligible to participate and are encouraged to contact our team for more information.

For more information, please contact Dr. Jeffrey M. Gelfand, MD at Jeffrey.Gelfand@ucsf.edu or 415-502-7215.

Additional ongoing studies that are *closed to enrollment*

OPERA - Study To Evaluate The Efficacy And Safety Of Ocrelizumab In Comparison To Interferon Beta-1a (Rebif®) In Patients With Relapsing Multiple Sclerosis

ORATORIO - Study to evaluate the efficacy and safety of ocrelizumab in adults with Primary Progressive Multiple Sclerosis.

PREFER MS Study - A randomized, Open-label Study to Evaluate the Patient Retention of Fingolimod.

Riluzole - Study to see if riluzole is safe and effective in treating patients with early Multiple Sclerosis (MS) and Clinically Isolated Syndrome (CIS).

STRATIFY - To better understand whether antibodies to JCV may be used to predict whether a patient is at higher or lower risk for developing PML.

Vitamin D - Pilot Study - Pharmacodynamic and immunologic effects of Vitamin D supplementation in patients with multiple sclerosis and healthy controls.