

Neurology Clinical Trials Unit

Dementia

Contact: Everlyne Gomez (949) 824-8116

- Imaging Dementia - Evidence for Amyloid Scanning: A Coverage with Evidence Development Longitudinal Cohort Study
- A Phase 2b, double-blind, randomized, placebo-controlled study of RVT-101 in subjects with dementia with Lewy bodies (DLB)
- A Long-Term Extension Study of the Safety and Tolerability of RVT-101 in Subjects with Dementia with Lewy Bodies (DLB)
- *A Phase III, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, Efficacy and Safety Study of Crenezumab in Patients with Prodromal to Mild Alzheimer's Disease*
- A Phase 3, multicenter, randomized, double-blind, placebo-controlled study to assess the efficacy, safety, and tolerability of AVP-786 (deuterated [d6]-dextromethorphan hydrobromide [d6-DM]/quinidine sulfate [Q]) for the treatment of agitation in patients with dementia of the Alzheimer's type.
- A Phase IIA, Multi-Center, Randomized, Single-Blind, Placebo-Controlled, Crossover Study To Assess The Safety, Tolerability, And Preliminary Efficacy Of A Single Intravenous Dose of Allogeneic Human Mesenchymal Stem Cells To Subjects With Mild to Moderate Dementia Due to Alzheimer's Disease

Epilepsy

Contact: Breana Chew (949) 824-7524 or Veronica Martin (714) 456-7760

- A Double-Blind, Randomized, Placebo-Controlled, Parallel-Group, Multicenter Study To Evaluate The Efficacy and Safety of Lacosamide As Adjunctive Therapy for Uncontrolled Primary Generalized Tonic-Chronic Seizures in Subjects With Idiopathic Generalized Epilepsy
- An open-label, multicenter extension study to evaluate the long-term safety and efficacy of lacosamide as adjunctive therapy for uncontrolled primary generalized tonic-clonic seizures in subjects with idiopathic generalized epilepsy
- *RNS[®] System Post-Approval Study in Epilepsy*

Movement Disorders

Contact: Everlyne Gomez (949) 824-8116 or Breana Chew (949) 824-7524

- Enroll-HD: A Prospective Registry Study in a Global Huntington's Disease Cohort A CHDI Foundation Project
- A Phase 3, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Examine the Efficacy, Safety and Tolerability of APL-130277 in Levodopa Responsive Patients with Parkinson's Disease Complicated by Motor Fluctuations ("OFF" Episodes)
- An Open-Label, Phase 3 Study Examining the Long-Term Safety, Tolerability and Efficacy of APL-130277 in Levodopa Responsive Patients with Parkinson's Disease Complicated by Motor Fluctuations ("OFF" Episodes)

- An Observational Study of Personal KinetiGraph™ (PKG™) Movement Recording System Use in Routine Clinical Care of Patients with Parkinson's Disease
- A phase IIa, randomize, double-blind, placebo-controlled study of the safety and efficacy of fenofibrate as a treatment of Huntington's disease
- A Phase 3 Double-Blind, Placebo Controlled, Parallel Group Study of Isradipine as a Disease Modifying Agent in Subjects with Early Parkinson Disease
- Characterization of Movement Disorders and Its Biomarkers

Multiple Sclerosis

Contact: Everlyne Gomez (949) 824-8116 or Breana Chew (949) 824-7524

- Plegridy™ (peginterferon β -1a) Real World Effectiveness and Safety Observational Program (POP)
- An Open-Label, Single-Arm Study to Evaluate the Effectiveness and Safety of Ocrelizumab in Patients with Early Stage Relapsing Remitting Multiple Sclerosis

Neuromuscular

Contact Veena Mathew (714) 456-2864; Marie Wencel (714) 456-2525 or Veronica Martin (714) 456-7760

- Fluid Biomarkers with Deep Phenotyping in Patients with ALS
- A Phase 3, Randomized, Double-Blind, Placebo-Controlled Multicenter Study to Evaluate Efficacy and Safety of Repeated Administrations of Nurown® (Autologous Mesenchymal Stem Cells Secreting Neurotrophic Factors) in Participants with Amyotrophic Lateral Sclerosis (ALS)
- A Phase 3, Multi-National, Double-Blind, Randomized, Placebo-Controlled, Stratified, Parallel Group, Study to Evaluate the Safety, Tolerability and Efficacy of Tirasemtiv in Patients with Amyotrophic Lateral Sclerosis (ALS)
- A Phase 3, Open-Label Extension Study of Tirasemtiv for Patients with Amyotrophic Lateral Sclerosis (ALS) Who Completed VITALITY-ALS (CY 4031)
- A PHASE 2, MULTI-CENTER, DOUBLE-BLIND, RANDOMIZED, DOSE-RANGING, PLACEBO-CONTROLLED STUDY TO EVALUATE THE EFFICACY, SAFETY, AND TOLERABILITY OF CK-2127107 IN PATIENTS WITH AMYOTROPHIC LATERAL SCLEROSIS (ALS)
- International GBS Outcome Study (IGOS): A prospective Inflammatory Neuropathy Consortium (INC) study on clinical and biological predictors of disease course and outcome in Guillain-Barré syndrome (GBS)
- A Multicenter, Double Blind, Placebo Controlled Study to Assess the Efficacy and Safety of H.P. Acthar Gel in the Treatment of Subjects with Amyotrophic Lateral Sclerosis
- Evaluation of the safety, tolerability, efficacy and activity of AMX0035, a fixed combination of Phenylbutyrate (PB) and Tauroursodeoxycholic Acid (TUDCA), for treatment of amyotrophic lateral sclerosis (ALS)
- Effect of Mexiletine on Cortical Hyperexcitability in Sporadic Amyotrophic Lateral Sclerosis (SALS)

- A Phase 2 Pharmacodynamic Study of Ezogabine on Neuronal Excitability in Amyotrophic Lateral Sclerosis
- A Phase 2 Randomized, Double-Blind, Placebo-Controlled, Multicenter Study of NP001 in Subjects with Amyotrophic Lateral Sclerosis (ALS) and Evidence of Elevated Systemic Inflammation
- Prospective, Double-blind, Randomized, Placebo-Controlled Phase III Study Evaluating Efficacy and Safety of Octagam 10% in Patients With Dermatomyositis (“ProDERM study”)
- A Phase 2 Randomized, Double-Blind, Placebo-Controlled Study of ACE-083 in Patients with Charcot-Marie-Tooth Disease Types 1 and X
- A PHASE III, OPEN-LABEL, EXTENSION TRIAL OF ECU-MG-301 TO EVALUATE THE SAFETY AND EFFICACY OF ECULIZUMAB IN SUBJECTS WITH REFRACTORY GENERALIZED MYASTHENIA GRAVIS (gMG)
- An Open-label, Ascending-Dose, First-in-Human Study to Assess the Safety, Tolerability, and Pharmacokinetics of Intravenous Infusions of ATB200 Alone and ATB200 Co-administered with Oral AT2221 in Adult Subjects with Pompe Disease who were Previously Treated with Alglucosidase alfa
- A Retrospective Characterization of Response to Enzyme Replacement Therapy in Late onset Pompe Disease
- A Randomized, Double Blind, Placebo Controlled Phase II Study to Evaluate the Safety, Efficacy, and Pharmacokinetics of ARGX-113 in Patients with Myasthenia Gravis who have Generalized Muscle Weakness
- A Phase 3, Randomized, Double-Blind Clinical Trial to Evaluate the Efficacy and Safety of Abatacept SC with Standard Treatment Compared to Standard Treatment Alone in Improving Disease Activity in Adults with Active Idiopathic Inflammatory Myopathy (IIM)
- A phase 3 randomized, multicenter, multinational, double-blinded study comparing the efficacy and safety of repeated biweekly infusions of neoGAA (GZ402666) and alglucosidase alfa in treatment-naïve patients with late-onset Pompe disease
- GT11306: A Multi-center, Randomized, Double-blind, Placebo-controlled Study to Evaluate the Efficacy and Safety of Immune Globulin (Human), 10% Caprylate/Chromatography Purified (IGIV-C) in Symptomatic Subjects with Generalized Myasthenia Gravis
- GT11408: A Multi-Center, Randomized, Double-blind, Placebo-controlled Study to Evaluate the Efficacy and Safety of Immune Globulin (Human), 10% Caprylate/Chromatography Purified (IGIV-C) in Symptomatic Subjects with Generalized Myasthenia Gravis
- Using Next Generation Sequencing to Unravel the Pathogenesis of Sporadic Inclusion Body Myositis (IBM) – The International IBM Consortium Genetic Study
- A Phase 2, Randomized, Double-Blind, Placebo-Controlled Trial of IMO-8400 in Patients with Dermatomyositis
- Investigating Pompe Prevalence in NEuromuscular Medicine Academic Practices (IPANEMA Study)

- Belimumab for Maintenance Therapy in Idiopathic Inflammatory Myositis
- A Multicenter, Randomized, Investigator-and-Subject-Blind, Placebo-Controlled, Treatment Sequence Study Evaluating the Safety, Tolerability, and Efficacy of UCB7665 in Subjects with Moderate to Severe Myasthenia Gravis
- A Phase 2 Open-label study to Evaluate the Safety of Aceneuramic Acid Extended Release (Ace-ER) Tablets in GNE Myopathy (GNEM) (also known as Hereditary Inclusion Body Myopathy (HIBM)) patients with Severe Ambulatory Impairment
- A Phase 3 Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy and Safety of Sialic Acid-Extended Release Tablets in Patients with GNE Myopathy (GNEM) or Hereditary Inclusion Body Myopathy (HIBM)
- A Phase 3b Open-label Extension Study to Evaluate the Safety and Efficacy of Aceneuramic Acid Extended-Release (Ace-ER) Tablets in Patients with GNE Myopathy (GNEM) or Hereditary Inclusion Body Myopathy (HIBM)
- Hereditary Inclusion Body Myopathy-Patient Monitoring Program (HIBM-PMP): A Registry and Prospective Observational Natural History Study to Assess HIBM Disease
- A Three-month, Open-Label, Randomized, Parallel Active Control, Single and Repeat Dose, Dose-escalation Study of the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Preliminary Efficacy of VAL-1221 Delivered Intravenously (IV) in Ambulatory and Ventilator-free Patients with Late-Onset GSD-II (Pompe Disease)

Stroke/TBI

Contact: Veronica Martin (714) 456-7760

- A Double-Blind, Controlled Phase 2B Study of the Safety and Efficacy of Modified Stem Cells (SB623) in Patients with Chronic Motor Deficit from Ischemic Stroke
- A Double-Blind, Controlled Phase 2 Study of the Safety and Efficacy of Modified Stem Cells (SB623) in Patients with Chronic Motor Deficit from Traumatic Brain Injury (TBI)
- The Surpass IntraCranial Aneurysm Embolization System Pivotal Trial to treat large OR giant wide neck aneurysms