Neurology Clinical Trials Unit 2023	
Title	**PI Name**
A Phase 2, Randomized, Double-Blind, Placebo-Controlled, 48-Week, Parallel-Group Study of the Efficacy and Safety of Losmapimod in Treating Subjects with Facioscapulohumeral Muscular Dystrophy (FSHD) with Open-Label Extension (OLE)	Goyal, Namita
An Open-label Extension to the Phase 2 Randomized, Double-blind, Placebo-controlled, Crossover Multicenter Study to Evaluate the Safety and Efficacy of KZR-616 in the Treatment of Patients With Active Polymyositis or Dermatomyositis	Goyal, Namita
A Phase III, Randomized, Double-Blind, Placebo-Controlled, Multicenter Trial to Evaluate the Safety and Efficacy of AMX0035 Versus Placebo for 48-week Treatment of Adult Patients with Amyotrophic Lateral Sclerosis (ALS)	Goyal, Namita
A Phase 3b, Multicenter, Randomized, Double-blind Study to Evaluate Efficacy and Safety of Oral Edaravone Administered for a Period of 48 Weeks in Subjects with Amyotrophic Lateral Sclerosis (ALS)	Goyal, Namita
A Randomized, Double-Blind, Placebo-Controlled Study to Assess Safety, Tolerability, and Pharmacokinetics Following Multiple Doses of ABBV-CLS-7262 in Subjects with Amyotrophic Lateral Sclerosis Followed by an Active Treatment Extension	Goyal, Namita
A Phase 2a Study of TPN-101 in Patients with C9ORF72 ALS/FTD (Amyotrophic Lateral Sclerosis and/or Frontotemporal Dementia)	Goyal, Namita
Phase 2a Safety, Tolerability, Pharmacokinetic, and Pharmacodynamic Study of Intravenous ANX005 in Subjects with Amyotrophic Lateral Sclerosis	Goyal, Namita
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)	Goyal, Namita
A Phase 3, Open-Label Extension of COURAGE-ALS (CY 5031)	Goyal, Namita
A Phase 2/3, Double-Blind, Randomized, Placebo-Controlled, Parallel Group, Multicenter Study to Evaluate the Efficacy and Safety of Ravulizumab in Adult Participants with Dermatomyositis	Goyal, Namita

A Phase 3b, Multicenter, Randomized, Double-blind Extension Study to Evaluate the Continued Efficacy and Safety of Oral Edaravone Administered for an Additional Period of up to 48 Weeks Following Study MT-1186-A02 in Subjects with Amyotrophic Lateral Sclerosis (ALS)	Goyal, Namita
A phase 3, Global, Randomized, double-blind, Placebo controlled 72 week, Parallel design study of the efficiency and safety of Losmapimod in treating Patients with Facioscapulohumeral Muscular Dystrophy(FHSD) (Reach)	Goyal, Namita
A Phase 2, Multicenter, Randomized, Double-blind, Placebo-controlled Study to Evaluate the Efficacy and Safety of SAR443820 in Adult Participants With Amyotrophic Lateral Sclerosis, Followed by an Open-label Extension	Goyal, Namita
A Phase 2b/3, Multi-Center, Randomized, Double-Blind, Placebo-Controlled, 12 Month Clinical Trial to Evaluate the Efficacy and Safety of MN-166 (Ibudilast) Followed by an Open-Label Extension Phase in Subjects With Amyotrophic Lateral Sclerosis	Goyal, Namita
Influence of NT5c1A antibodies on disease progression, clinical phenotype and blood and muscle biomarkers in sporadic Inclusion Body Myositis A prospective evaluation (INSPIRE-IBM)	Goyal, Namita
A Phase 2, Randomized, Double-Blind, Placebo-Controlled, Parallel Study to Assess the Efficacy, Safety, Tolerability, PK, and Biomarker Effects of PTC857 in Adult Subjects With Amyotrophic Lateral Sclerosis (CARDINALS)	Goyal, Namita
A Phase 2, Multicenter, Randomized, Double-blind, Placebo-controlled, Parallel-group study to Evaluate the Efficacy and Safety of Nipocalimab in Participants with Active Idiopathic Inflammatory Myopathies	Goyal, Namita
Clinical Procedures to Support Research in ALS (CAPTURE-ALS)	Goyal, Namita
A Phase II/III Randomized, Double-blind, Placebo-controlled, Multicenter Study to Determine the Efficacy and Safety of ABC008 in the Treatment of Subjects with Inclusion Body Myositis	Goyal, Namita
A Phase II, Multicenter, Randomized, Placebo-Controlled, Double-Blind Study to Evaluate the Pharmacodynamics, Safety, Tolerability, Pharmacokinetics, and Efficacy of RO7204239 in Participants With Facioscapulohumeral Muscular Dystrophy	Goyal, Namita
A Second Intermediate Expanded Access Protocol for Amyotrophic Lateral Sclerosis with CNM-Au8	Goyal, Namita
This expanded access protocol is to provide access to the investigational product, SLS-005, to participants with ALS who are not eligible to participate in clinical trials.	Goyal, Namita

Retrospective Analysis of Survival Outcomes Among Patients with Amyotrophic Lateral Sclerosis Who Received an Investigational Treatment, NP001, in Phase 2 Clinical Trials	Goyal, Namita
HEALEY ALS Platform Trial	Goyal, Namita
An Open-label, Adaptive Design Study in Patients With Amyotrophic Lateral Sclerosis (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 the Radioligand [11C]-PBR28	Goyal, Namita
A Phase 3, Randomized, Double-Blind, Placebo-Controlled, Multicenter Study to Evaluate the Safety and Efficacy of Ravulizumab in Complement-Inhibitor-Naïve Adult Patients With Generalized Myasthenia Gravis	Habib, Ali A
A Phase 3, Multicenter, Open-Label Extension Study of Zilucoplan in Subjects with Generalized Myasthenia Gravis	Habib, Ali A
ARGX-113-2003: A Phase 3b, Randomized, Open-label, Parallel-Group Study to Evaluate Different Dosing Regimens of Intravenous Efgartigimod to Maximize and Maintain Clinical Benefit in Patients with Generalized Myasthenia Gravis	Habib, Ali A
Intermuscular Coherence: A Biomarker for Early Diagnosis and Follow-up of ALS	Habib, Ali A
A Phase III, Randomized, Double-Blind, Placebo-Controlled, Multicenter Study To Evaluate Efficacy, Safety, Pharmacokinetics, And Pharmacodynamics Of Satralizumab In Patients With Generalized Myasthenia Gravis	Habib, Ali A
Long-Term, Observational, Registry of Patients With Generalized Myasthenia Gravis Who Have Received Treatment With Complement C5 Inhibition Therapies	Habib, Ali A
A Randomized, Double-blind, Multicenter, Placebo-controlled Phase 3 Study with Open-label Period to Evaluate the Efficacy and Safety of Inebilizumab in Adults with Myasthenia Gravis	Habib, Ali A
PLS Natural History Study (PNHS)	Habib, Ali A
A Phase 3, Multi-center, Randomized, Quadruple-blind, Placebo-controlled Study to Assess the Efficacy and Safety of Batoclimab as Induction and Maintenance Therapy in Adult Participants with Generalized Myasthenia Gravis (gMG)	Habib, Ali A

NIMBLE Network Biorepository including MGNet (Neuroimmunology Biosample; Myathenia Gravis Network)	Habib, Ali A
A Phase 3, Randomized, Double-blind, Placebo-controlled, Parallel, Multicenter Study to Evaluate the Safety and Efficacy of ALXN1720 in Adults with Generalized Myasthenia Gravis	Habib, Ali A
A Phase 3b, Multicenter, Open-Label, Single-Arm Study to Evaluate the Safety, Tolerability, and Efficacy of Zilucoplan in Participants With Generalized Myasthenia Gravis Switching From Intravenous Complement Component 5 Inhibitors to Subcutaneous Zilucoplan	Habib, Ali A
An Open-Label, Crossover Study to Evaluate Rozanolixizumab Self-Administration By Study Participants With Generalized Myasthenia Gravis	Habib, Ali A
A Phase-2b, Double-Blind, Randomized Controlled Trial to Evaluate the Activity and Safety of Inebilizumab in Anti-Nmda Receptor Encephalitis and Assess Markers of Disease	Kong, Xiao-Tang
Long-Term Follow-Up Study of Patients With Spinal Muscular Atrophy Receiving Risdiplam Treatment	Korb, Manisha
TTR Amyloidosis Prevalence and Phenotype (TTRAPP Study)	Korb, Manisha
A Double-Blind, Randomized, Placebo-Controlled, Multicenter, Outpatient, Parallel-Group Study to Assess the Efficacy and Safety of Staccato Alprazolam in Study Participants 12 Years of Age and Older with Stereotypical Prolonged Seizures	Mnatsakanyan, Lilit
RNS® System Post-Approval Study in Epilepsy	Mnatsakanyan, Lilit
An Open-Label, Multicenter, Outpatient Extension Study to Evaluate the Safety and Tolerability of Staccato Alprazolam in Study Participants 12 Years of Age and Older With Stereotypical Prolonged Seizures	Mnatsakanyan, Lilit
Multicenter, Randomized, Double-blind, Placebo-controlled Study to Evaluate the Efficacy, Safety, and Tolerability of 36 Weeks of Treatment With NLY01 in Early-stage Parkinson's Disease	Morenkova, Anna E
A randomized, double blind, placebo-controlled, 2-period crossover, phase 2 study to evaluate the efficacy, safety, tolerability, pharmacokinetics, and pharmacodynamics of oral TAK-071 in Parkinson's patients with cognitive impairment and an elevated risk of falls.	Morenkova, Anna E

Enroll-HD: A Prospective Registry Study in a Global Huntington's Disease Cohort A CHDI Foundation Project	Morenkova, Anna E
A Randomized, Double-Blind, Placebo-Controlled, Two Part Study in Parkinson's Disease Patients With Dyskinesia to Assess the Efficacy and Safety/Tolerability of Fixed Dose Combinations of JM-010 and its Individual Components	Morenkova, Anna E
A Randomized, Double-blind, Placebo-controlled Multicenter Study to Assess the Efficacy and Safety of Lenrispodun as Adjunctive Therapy in the Treatment of Patients with Motor Fluctuations due to Parkinson's Disease	Morenkova, Anna E
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.	Mozaffar, Tahseen
A Phase 3 Open-label Extension Study to Assess the Long-term Safety and Efficacy of Intravenous ATB200 Co-administered With Oral AT2221 in Adult Subjects With Late Onset Pompe Disease.	Mozaffar, Tahseen
An International Clinical Outcome Study of Dysferlinopathy	Mozaffar, Tahseen
A Phase 2 Trial to Investigate the Efficacy, Safety, and Tolerability of Efgartigimod PH20 SC in Adult Patients with Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) (ADHERE)	Mozaffar, Tahseen
A Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Safety and Efficacy of Pitolisant on Excessive Daytime Sleepiness and Other Non-Muscular Symptoms in Patients with Myotonic Dystrophy Type 1, Followed by an Open-Label Extension	Mozaffar, Tahseen
Open-label Extension of the ARGX-113-1802 Trial to Investigate the Long-term Safety, Tolerability, and Efficacy of Efgartigimod PH20 SC in Patients with Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)	Mozaffar, Tahseen
GRASP-LGMD RESEARCH PROTOCOL; Project 1a Defining Clinical Endpoints in LGMD	Mozaffar, Tahseen
A Phase 2, multicenter, open label, proof-of-concept study evaluating the efficacy, safety, and tolerability of BIVV020 in adults with chronic inflammatory demyelinating polyneuropathy (CIDP)	Mozaffar, Tahseen
A Phase 3, Randomized, Double-blind, Placebo-controlled and Extension Study to Investigate the Efficacy and Safety of Oral Brepocitinib in Adults with Dermatomyositis in Comparison to Standard of Care	Mozaffar, Tahseen

A Phase 2/3, Randomized, Double-Blinded, Placebo-Controlled, Parallel-Group, 2-Arm, Multicenter, Operationally Seamless Study to Evaluate the Efficacy, Safety, Tolerability, Pharmacodynamics, Pharmacokinetics, and Immunogenicity of Efgartigimod PH20 SC in Participants Aged 18 Years and Older With Active Idiopathic Inflammatory Myopathy	Mozaffar, Tahseen
Defining Endpoints in Becker Muscular Dystrophy	Mozaffar, Tahseen
A Phase 1/2a, Randomized, Double-Blind, Placebo-Controlled, First-In-Patient Study Of AJ201 To Evaluate Safety, Tolerability, Pharmacokinetics, And Pharmacodynamics In Adults With Spinal And Bulbar Muscular Atrophy (SMBA)	Mozaffar, Tahseen
A Phase 3 Randomized, Placebo-controlled, Double-blind Study to Evaluate the Efficacy and Safety of BBP-418 (Ribitol) in Patients With Limb Girdle Muscular Dystrophy 2I (LGMD2I)	Mozaffar, Tahseen
Advarra Reliance #26: Neuromuscular Observational Research (MOVR) Data Hub Protocol Pro00034893	Mozaffar, Tahseen
A Randomized Clinical Trial of Andexanet Alfa in Acute Intracranial Hemorrhage in Patients Receiving an Oral Factor Xa Inhibitor	Nagamine, Masaki
Sleep for Stroke Management And Recovery Trial (Sleep SMART)	Nagamine, Masaki
New IDEAS: Imaging Dementia Evidence for Amyloid Scanning Study A Study to Improve Precision in Amyloid PET Coverage and Patient Care	Sajjadi, Seyed Ahmad
A Randomized, Double-blind, Placebo-Controlled, Multicenter Phase 3 Study to Evaluate the Safety, Tolerability, and Efficacy of XEN1101 as Adjunctive Therapy in Focal-Onset Seizures	Sazgar, Mona
A Multicenter, Open-label, Long-term, Safety, Tolerability, and Efficacy Study of XEN1101 in Adults Diagnosed With Epilepsy	Sazgar, Mona
Multi-arm Optimization of Stroke Thrombolysis (MOST): a Single Blinded, Randomized Controlled Adaptive, Multi-arm, Adjunctive-thrombolysis Efficacy Trial in Ischemic Stroke	Shafie, Mohammad
Statins Use in intRacereberal hemorrhage patieNts (SATURN)	Shafie, Mohammad

A multicenter, international, randomized, placebo controlled, double-blind, parallel group and event driven Phase 3 study of the oral FXIa inhibitor asundexian (BAY 2433334) for the prevention of ischemic stroke in male and female participants aged 18 years and older after an acute non-cardioembolic ischemic stroke or high-risk TIA	Shafie, Mohammad
A multicenter, randomized, placebo-controlled, double-blinded, Phase 3 study to evaluate the efficacy and safety of 3K3A-APC, a recombinant variant of human activated protein C, in combination with tissue plasminogen activator, mechanical thrombectomy, or both in subjects with moderate to severe acute ischemic stroke	Shafie, Mohammad
Recombinant Factor VIIa (rFVIIa) for Acute Hemorrhagic Stroke Administered at Earliest Time (FASTEST) Trial	Shah, Jay
Carotid Revascularization and Medical Management for Asymptomatic Carotid Stenosis Trial	Suzuki, Shuichi
An Open-Label, Single-Arm Study to Evaluate the Effectiveness and Safety of Ocrelizumab in Patients with Early Stage Relapsing Remitting Multiple Sclerosis	Sy, Michael
A Phase IIIB Multicenter, Randomized, Double-Blind, Controlled Study to Evaluate the Efficacy, Safety and Pharmacokinetics of a Higher Dose of Ocrelizumab in Adults With Primary Progressive Multiple Sclerosis	Sy, Michael
A Phase III Multicenter Randomized, Double-Blind, Double-Dummy, Parallel-Group Study To Evaluate The Efficacy And Safety Of Fenebrutinib Compared With Teriflunomide In Adult Patients With Relapsing Multiple Sclerosis	Sy, Michael
CorEvitas SPHERES (Synergy of Prospective Health & Experimental Research for Emerging Solutions) Registry for Neuromyelitis Optica Spectrum Disorder (NMOSD)	Sy, Michael
Anticoagulation in Intracerebral Hemorrhage (ICH) Survivors for Stroke Prevention and Recovery (ASPIRE)	Yu, Wengui
Comparison of Anti-coagulation and anti-Platelet Therapies for Intracranial Vascular Atherostenosis CAPTIVA	Yu, Wengui
A Phase 3, Randomized, Double-Blind, Parallel-Group, Placebo-Controlled Study to Demonstrate the Efficacy and Safety of Milvexian, an Oral Factor XIa Inhibitor, for Stroke Prevention After an Acute Ischemic Stroke or High-Risk Transient Ischemic Attack	Yu, Wengui