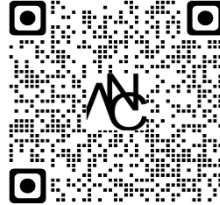




## Currently Enrolling Studies:



### Amyloidosis

- **ALN-TTR02-014 EAP** – Expanded Access Protocol to Provide Patisiran to Patients with Transthyretin-mediated Amyloidosis (ATTR Amyloidosis) With Cardiomyopathy. [NCT05505838](#)
- **wtATTR amyloidosis** – Patients with peripheral and autonomic neuropathies. [NCT04561518](#)

### Amyotrophic Lateral Sclerosis (ALS)

- **MT-1186-A02** – 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). [NCT04569084](#)
- **233AS303** – A Phase 3 Randomized, Placebo-Controlled Trial with a Longitudinal Natural History Run-In and Open-Label Extension to Evaluate BIIB067 Initiated in Clinically Presymptomatic Adults with a Confirmed Superoxide Dismutase 1 Mutation. [NCT04856982](#)
- **PrecisionMed Protocol 4800** – A single or multiple visit protocol for collection of DNA/RNA/SERUM/PLASMA/CSF in Amyotrophic Lateral Sclerosis and related disorders. [NCT05349721](#)
- **PTC857-CNS-001-ALS** – 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). [NCT05349721](#)

### Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)

- **ARGX-113-1802/1902** – Phase 2 Trial to Investigate the Efficacy, Safety, and Tolerability of Efgartigimod PH20 SC in Adult Patients with Chronic Inflammatory Demyelinating Polyneuropathy (CIDP). [NCT04280718](#)
- **80202135CDP3001** – Phase 2/3, Multistage, Multicenter, Randomized, Double-Blind, Placebo-Controlled Parallel Group Withdrawal Study to Evaluate the Efficacy and Safety of Nipocalimab Administered to Adults with Chronic Inflammatory Demyelinating Polyneuropathy (CIDP). [NCT05327114](#)
- **IMVT-1401-2401** – A Phase 3 Study to Assess the Efficacy and Safety of Batoclimab as Induction and Maintenance Therapy in Adult Participants with active CIDP. [NCT05581199](#)

### **Dermatomyositis**

- **PVT-2201-301** – A Phase 3 Study to Investigate the Efficacy and Safety of Brepocitinib in Adults with Dermatomyositis (VALOR). [NCT05437263](#)
- **MS200569\_0041** - A Phase IIa, Randomized, Parallel, Double-Blind, Placebo Controlled Study to Evaluate the Efficacy and Safety of Enpatoran in Dermatomyositis and Polymyositis Participants receiving Standard of Care. [NCT05650567](#)

### **Idiopathic Inflammatory Myopathy**

- **ARGX-113-2007** – 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. [NCT04812925](#)

### **Inclusion Body Myositis**

- **ABC008-IBM-201** - A Phase 2/3, Randomized, Double-blind, Placebo-controlled, Multicenter Study to Determine the Efficacy and Safety of ABC008 in the Treatment of Subjects with Inclusion Body Myositis. [NCT05271409](#)

### **Multifocal Motor Neuropathy**

- **ARGX 117-2002** – A Phase 2, Randomized, Double-Blinded, Placebo-Controlled, Parallel-Group, Multi-Center Trial to Evaluate the Efficacy, Safety and Tolerability, Pharmacokinetics, Pharmacodynamics, and Immunogenicity of 2 Dose Regimens of ARGX-117 in Adults with Multifocal Motor Neuropathy (MMN). [NCT04812925](#)

### **Myasthenia Gravis**

- **VIB0551.P3.S1** – 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. [NCT04524273](#)
- **R3918-MG-2018** – Efficacy And Safety Of Pozelimab And Cemdisiran Combination Therapy In Patients With Symptomatic Generalized Myasthenia Gravis. [NCT05070858](#)
- **IMVT – 1401-3101** – A Phase 3 Study to Assess the Efficacy and Safety of Batoclimab as Induction and Maintenance Therapy in Adult Participants with Generalized Myasthenia Gravis. [NCT05403541](#)

### **Primary Mitochondrial Disease**

- **SPIMD-301** – A Phase 3 Randomized, Double-Blind, Parallel-Group, Placebo-Controlled Trial to Evaluate the Efficacy and Safety of Daily Subcutaneous Injections of Elamipretide in Subjects with Primary Mitochondrial Disease Resulting from Pathogenic Nuclear DNA Mutations (Npmd). [NCT05162768](#)

### **Transthyretin (TTR) – Mediated Amyloidosis**

- **ALN-TTR02-013-ConTTRibute** – A Global Observational Multicenter Long-Term. Study of Patients with Transthyretin (TTR)-Mediated Amyloidosis (ATTR amyloidosis). [NCT04561518](#)