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Currently Enrolling Studies

Amyotrophic Lateral Sclerosis (ALS)

- **ATLAS- 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](https://clinicaltrials.gov/ct2/show/study/NCT04856982)
- **PRECISION MED- Protocol 4800** – A single or multiple visit protocol for collection of DNA/RNA/SERUM/PLASMA/CSF in Amyotrophic Lateral Sclerosis and related disorders.

Becker Muscular Dystrophy

- **GRAND CANYON- EDG- 5506-201** - A Phase 2 Randomized, Double-blind, Placebo-controlled Study to Evaluate the Effect of EDG-5506 on Safety, Biomarkers, Pharmacokinetics, and Functional Measures in Adults and Adolescents with Becker Muscular Dystrophy.” [NCT05291091](https://clinicaltrials.gov/ct2/show/study/NCT05291091)

Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)

- **CIDP- 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](https://clinicaltrials.gov/ct2/show/study/NCT05327114)
- **IMMUNOVANT CIDP- IMVT-1401-2401** –A Phase 2b, Multi-center, Randomized, Quadruple-blind, Placebo-controlled Study of Batoclimab Treatment in Adult Participants with Active Chronic Inflammatory Demyelinating Polyneuropathy (CIDP). [NCT05581199](https://clinicaltrials.gov/ct2/show/study/NCT05581199)



Dermatomyositis

- **NEPTUNIA- 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 (NEPTUNIA). [NCT05650567](#)

Idiopathic Inflammatory Myopathy

- **ALKIVIA- 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. [NCT05523167](#)

Myasthenia Gravis

- **NIMBLE - R3918-MG-2018** – Efficacy and Safety Of Pozelimab And Cemdisiran Combination Therapy In Patients With Symptomatic Generalized Myasthenia Gravis. [NCT05070858](#)
- **ADAPT SERON- ARGX-113-2308**- A Randomized, Double-Blinded, Placebo-Controlled, Phase 3, Parallel-Group Design Study Evaluating the Efficacy and Safety of Efgartigimod IV in Adult Participants with Acetylcholine Receptor Binding Antibody Seronegative Generalized Myasthenia Gravis. [NCT06298552](#)
- **ADAPT OCULUS- ARGX-113-2315** - A Randomized, Double-Blinded, Placebo-Controlled, Phase 3, Parallel-Group Design Study Evaluating the Efficacy and Safety of Efgartigimod PH20 SC Administered by Prefilled Syringe in Adult Participants with Ocular Myasthenia Gravis. [NCT06558279](#)

Postural orthostatic tachycardia syndrome (POTS)

- **RECOVER-AUTONOMIC**- A Platform Protocol for Evaluation of Interventions for Autonomic Dysfunction in Post-Acute Sequelae of SARS-CoV-2 Infection (PASC). [NCT06305780](#)

Inclusion Body Myositis (IBM)

- **IBM- R3918-OT-2383**- Efficacy and safety of Pozelimab and Cemdisiran combination therapy in patients with sporadic inclusion body myositis-Investigator Initiative Study. [NCT06479863](#)



Charcot-Marie-Tooth disorder (CMT Type 1 and Type 2)

- **SYNAPSE-CMT- NMD670-02-0003-A** Phase 2a, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy, Safety, and Tolerability of NMD670 Over 21 Days in Ambulatory Adult Patients with Type 1 and Type 2 Charcot-Marie-Tooth Disease. [NCT06482437](https://clinicaltrials.gov/ct2/show/study/NCT06482437)

UPCOMING STUDIES

Amyotrophic Lateral Sclerosis (ALS)

- **EAP 2 -PL101-ALS501** - Second Intermediate-Size Expanded Access Protocol (EAP) for Pridopidine in People with Amyotrophic Lateral Sclerosis (Pridopidine EAP 2). [NCT06069934](https://clinicaltrials.gov/ct2/show/study/NCT06069934)

Amyloidosis

- **Eidos-AG10-501**- A phase 3 Randomized, Multicenter, Double-Blind, Placebo Controlled Study of Acoramidis for Transthyretin Amyloidosis Prevention in the Young (ACT-EARLY Trial). [NCT06563895](https://clinicaltrials.gov/ct2/show/study/NCT06563895)