

Active Studies

MG0003/MG0004 – Rozanolixizumab Phase 3 Clinical Trial

The purpose of the MG0003 clinical research study is to evaluate the safety and efficacy of Rozanolixizumab in patients with generalized Myasthenia Gravis. Primary endpoint is the score on MG-ADL.

Eligibility:

- Adult ≥18 years old with documented diagnosis of gMG at Visit 1
- Confirmed positive record of autoantibodies against AChR or MuSK prior to Visit 1.
- Moderate to severe gMG (MGFA Class II to IVa at Visit 1; MG-ADL score of ≥ 3 and QMG score ≥ 11 at Visit 1 and Baseline)
- Considering treatment with IVIg or PEX

Neuromuscular Observational Research (MOVR) Data Hub

MOVR Data Hub aggregates clinical, genetic and patient reported data for multiple neuromuscular diseases. The combination of data collected through MOVR Data Hub will provide a comprehensive understanding of health and patient experiences in a single data repository. The recruitment goal 10,000 patients.

Eligibility:

- Male and female patients of any age with ALS, DMD, BMD, and SMA
- No exclusion criteria

Predictors of Prognosis – Biomarker Study

This is a simple blood draw study; patients and healthy controls are asked to give a blood sample that can be done the same day they meet with the ALS team in clinic. Blood will be processed into DNA to determine if there are genes that influence the number and/or rate of motor nerve loss in motor neuron disease. The purpose of the biomarker study is to examine the connection between cell-free DNA and ALS disease progression in hopes of providing earlier diagnosis of ALS and/or better predict prognosis.

Eligibility:

- Age ≥ 20 years old
- ALS diagnosis; or healthy, age-matched control with no history of neurological disease

Pending Studies

REFINE-ALS – Radicava®/Edaravone Biomarker Study

The purpose of REFINE-ALS is to clarify the mechanism of action of Radicava and understand why it seems to work in slowing disease progression in some patients.

Eligibility:

- Adult ≥18 years old with ALS diagnosis
- Decision to begin Radicava prior to screening
- Either naïve to Radicava or have not received a dose 1 month prior to screening
- Cannot be in a clinical trial

ADVANCE-CIDP – HYQVIA Phase 3 Clinical Trial

The purpose of the ADVANCE-CIDP study is to provide evidence for the use of HYQVIA as a maintenance therapy option that enables self-infusion of a full therapeutic dose every 2 to 4 weeks in patients with CIDP.

Eligibility:

- Diagnosis of definite or probable CIDP (focal atypical CIDP and pure sensory atypical CIDP exclusionary)
- Subject responded to IgG treatment in the past and must currently be on a stable dose of IVIG for at least 12 weeks prior to screening
- Subject cannot have received treatment with any corticosteroids within 8 weeks prior to screening
- Subject cannot have undergone plasma exchange within 3 months prior to screening

ALXN1210-ALS-308 – Phase 3 ALS Clinical Trial

A Phase 3 study to evaluate the efficacy and safety of Ravulizumab in patients with ALS.

Eligibility:

- ALS symptom onset \leq 36 months prior to screening
- ALSFRS-R progression of -0.3 points per month or worse from symptom onset to screening
- Upright SVC \geq 65% at Screening
- Cannot be dependent on non-invasive or invasive mechanical ventilation. Dependence on mechanical ventilation is defined as being unable to lie flat (supine) without it, unable to sleep without it, or daytime use $>$ 6 hours per day for $>$ 3 days per week. Non-invasive ventilation for sleep apnea is allowed subject to discussion with Medical Monitor
- Prior stem cell treatment is not exclusionary

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