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Safety, Efficacy, And Pharmacokinetics of Argx-117 in Adults With Multifocal Motor Neuropathy: A Global, Multicenter, Placebo Controlled Phase 2 Study (Arda)

Jeffrey Allen, Eduardo Nobile-Orazio, Stojan Peric, Hans Katzberg, Stephanie Cadour, Inge Van de Walle, Iris Van Hoomissen, Bert Jacquemyn, Olivier Van de Steen, W. Ludo van der Pol

Objective

To report the study design of a Phase 2, multicenter, randomized, placebo controlled, parallel-group study (ARDA, NCT05225675) will assess the safety, efficacy, PK, and PD of ARGX-117 in adults with MMN.

Background

Multifocal motor neuropathy (MMN) is a chronic, immune-mediated neuropathy characterized by progressive asymmetric weakness predominantly affecting the distal upper limbs. MMN is often associated with anti-GM1 IgM antibodies targeting the axolemma at the node of Ranvier, leading to activation of the classical complement pathway which drives subsequent damage to the axon. IVIg is the only proven effective therapy. ARGX-117 is a novel monoclonal antibody that inhibits complement factor 2 (C2). Using an in vitro model for MMN, ARGX-117 was shown to block IgM-mediated classical pathway complement activation on both motor neurons and Schwann cells. ARGX-117 is being studied in adults with MMN in a Phase 2, multicenter, randomized, placebo controlled, parallel-group study.

Design/Methods

Forty-eight participants will be recruited and assigned to one of two dosing cohorts (24 participants each) and randomized 2:1 within each cohort to receive either ARGX-117 or placebo. Key inclusion criteria include: diagnosis of probable or definite MMN per 2010 EFNS/PNS guidelines, stable IVIg regimen, and IVIg treatment dependency. The primary objective is safety based on adverse event monitoring and other safety assessments. Secondary objectives include assessment of efficacy measured as strength and functional disability, pharmacokinetics, pharmacodynamics (C2 and CH50), and immunogenicity of ARGX-117. After completing the 16-week treatment period, participants may enroll in an open-label extension study, or enter the safety follow-up period.

Results

This ongoing phase 2 study will assess the safety and efficacy of ARGX-117 in participants with MMN.

Conclusions

The results of this study will inform and direct future studies of ARGX-117 in this patient population.

Disclosure: Dr. Allen has received personal compensation in the range of $500-$4,999 for serving as a Consultant for Argenyx. Dr. Allen has received personal compensation in the range of $500-$4,999 for serving as a Consultant for csl behring. Dr. Allen has received personal compensation in the range of $500-$4,999 for serving as a Consultant for Takeda. Dr. Allen has received personal compensation in the range of $500-$4,999 for serving as a Consultant for Grifols. Dr. Allen has received personal compensation in the range of $500-$4,999 for serving as a Consultant for Argenx. Dr. Allen has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for grifols. Dr. Allen has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Alexion. Dr. Allen has received personal compensation in the range of $10,000-$49,999 for serving on a Speakers Bureau for CSL behring. Dr. Nobile-Orazio has received personal compensation in the range of $500-$4,999

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Vibrance-mg: Clinical Trial of Nipocalimab in Pediatric Myasthenia Gravis

Sindhu Ramchandren, Shawn Black, Hong Sun

Objective

We describe an open-label study of nipocalimab to determine the effect of nipocalimab in pediatric participants with gMG.

Background

Nipocalimab is a high affinity, fully human, aglycosylated, effectorless IgG1 anti FcRn monoclonal antibody that targets the neonatal Fc receptor (FcRn) with high affinity, thereby lowering IgG pathogenic antibodies in autoimmune disease. Data from Vivacity-MG, a Phase 2, multicenter, randomized, double-blind, placebo-controlled study of nipocalimab demonstrated safety, tolerability, and efficacy of nipocalimab in adult generalized myasthenia gravis (gMG) (clinicaltrials.gov NCT03772587).

Design/Methods

This global study will enroll at least 12 participants, aged 2 to <18, with a diagnosis of gMG and an insufficient clinical response to ongoing, stable standard-of-care therapy, as reflected by a Myasthenia Gravis Foundation of America (MGFA) Class of IIa/b through IVa at screening. Participants must have a positive serologic test for either acetylcholine receptor or Muscle Specific Tyrosine Kinase pathogenic autoantibody. The study will consist of a screening period of up to 4 weeks, a 24-week open-label Active Treatment Phase where participants will receive nipocalimab intravenously every two weeks, and a Long-term Extension phase; after last dose, a safety follow-up assessment will be conducted at 8 weeks. The primary outcome is the effect of nipocalimab on total serum IgG, safety and tolerability, and PK in pediatric participants with gMG.

Results

Study enrollment will begin in 2022.

Conclusions

The vibrance-mg study will assess the PK/PD, safety and activity of Nipocalimab in pediatric gMG.

Disclosure: Dr. Ramchandren has received personal compensation for serving as an employee of Janssen Pharmaceutical Companies of Johnson & Johnson. Dr. Ramchandren has a non-compensated relationship as a Scientific Advisory Board Member with CMT Research Foundation (CMTRF) that is relevant to AAN interests or activities. Mr. Black has received personal compensation for serving as an employee of Janssen. Mr. Black has stock in Janssen. Dr. Sun has received personal compensation for serving as an employee of Janssen.

Vivacity MG Phase 3 Study: Clinical Trial of Nipocalimab Administered to Adults With Generalized Myasthenia Gravis

Sindhu Ramchandren, Panna Sanga, Michel Burcklen, Hong Sun

Objective

We describe Vivacity-MG3. our pivotal Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy, Safety, Pharmacokinetics (PK), and Pharmacodynamics (PD) of Nipocalimab Administered to Adults With gMG (NCT04951622).

Background

Nipocalimab is a high affinity, fully human, aglycosylated, effectorless IgG1 anti FcRn monoclonal antibody that targets the neonatal Fc receptor (FcRn) with high affinity, thereby lowering IgG pathogenic antibodies in autoimmune disease. Data from Vivacity-MG, a Phase 2 randomized placebo-controlled study of nipocalimab in adult generalized myasthenia gravis (gMG), demonstrated safety, tolerability, and efficacy of nipocalimab (clinicaltrials.gov NCT03772587).

Design/Methods

This global study will enroll approximately 180 participants with gMG, aged 18 and older, with an insufficient clinical response to ongoing, stable standard-of-care therapy, as reflected by a Myasthenia Gravis-Activities of Daily Living (MG-ADL) score of >/= to 6 at screening and baseline, and a Myasthenia Gravis Foundation of America (MGFA) Class of IIa/b–IVa/b at screening. The study will consist of a screening period of up to 4 weeks, a 24-week double-blind placebo-controlled phase where participants will be randomly assigned in a 1:1 ratio to receive either placebo or nipocalimab intravenously every two weeks, and an open label extension phase of variable duration. The primary outcome is the average change in MG-ADL score from baseline to weeks 22, 23 and 24 of the double-blind placebo-controlled phase.

Results

Study enrollment began in July 2021 and is ongoing.

Conclusions

The ongoing Vivacity MG Phase 3 study will assess the efficacy, safety, and PK/PD of Nipocalimab in adult gMG.

Disclosure: Dr. Ramchandren has received personal compensation for serving as an employee of Janssen Pharmaceutical Companies of Johnson & Johnson. Dr. Ramchandren has a non-compensated relationship as a Scientific Advisory Board Member with CMT Research Foundation (CMTRF) that is relevant to AAN interests or activities. Panna Sanga has received personal compensation for serving as an employee of Janssen Research & Development. Panna Sanga has stock in Johnson & Johnson. Michel Burcklen has received personal compensation for serving as an employee of Actelion Pharmaceuticals, a Janssen pharmaceutical company of Johnson & Jonhson. Michel Burcklen has received stock or an ownership interest from Johnson & Johnson. Dr. Sun has received personal compensation for serving as an employee of Janssen.

Response of Sjogren-related Small Fiber Neuropathy to rituximab

Anita Venkatesh, Suraj Muley

Objective

Small fiber neuropathy typically presents as loss of temperature and pain sensation and may be associated with other autonomic symptoms. Sjogren's-related ganglionopathy can selectively affect small fibers. Current treatment options for small fiber neuropathy secondary to

Safety, Efficacy, And Pharmacokinetics of Argx-117 in Adults With Multifocal Motor Neuropathy: A Global, Multicenter, Placebo Controlled Phase 2 Study (Arda)

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