

argenx reports positive topline results from Phase 2 proof-of-concept trial of efgartigimod in primary immune thrombocytopenia

- Favorable safety and tolerability consistent with efgartigimod clinical trials to date
- Clinically meaningful platelet count improvements showed clear separation from placebo at increasing response thresholds
- Plan to advance ITP program into Phase 3 development
- CIDP announced as fourth indication for efgartigimod
- Management to host conference call today at 8:00 am ET (2:00 pm CEST)

September 17, 2018

Breda, the Netherlands / Ghent, Belgium - argenx (Euronext & Nasdaq: ARGX) today announced positive topline results from its Phase 2 proof-of-concept clinical trial of efgartigimod (ARGX-113) in adult primary immune thrombocytopenia (ITP) patients. The Phase 2 data of efgartigimod showed a favorable safety and tolerability profile consistent with the Phase 1 healthy volunteer trial and the Phase 2 proof-of-concept trial in generalized myasthenia gravis (gMG). Patients treated with efgartigimod exhibited clinically meaningful platelet count improvements across doses and ITP patient classifications, including newly diagnosed, persistent and chronic, and correlated with a consistent reduction in IgG levels.

"These data highlight the differentiating features of efgartigimod in this difficult-to-treat disease. Efgartigimod has the potential to address ITP in a novel way across patient types, targeting disease at the source by eliminating IgGs and restoring platelet numbers. Also, efgartigimod continues to be well-tolerated, which we attribute to the unique binding characteristics of our Fc fragment and view as an important advantage in this new therapeutic class. We intend to leverage our leadership position in the FcRn antagonist space and advance efgartigimod in ITP to reach patients as quickly as possible," commented Nicolas Leupin, Chief Medical Officer of argenx.

"The need for new modalities in ITP is an urgent one as patients remain insufficiently managed despite several approved therapies. The variability seen across patients makes a one-size-fits-all treatment approach very difficult and current therapies come with significant side effects. Efgartigimod has been well-tolerated in clinical trials to date with a distinct mechanism of action, and has shown the potential to induce robust disease score improvements across a broad range of patients. We look forward to watching the progress of this program closely in hopes of offering this novel therapeutic option to patients," commented Dr. Adrian Newland, M.D., Professor of Hematology, The Royal London Hospital and Principal Investigator on the trial.

Topline Results

Thirty-eight patients on standard of care therapy with platelet count $\leq 30 \times 10^9/L$ at screening were enrolled in the double-blind, randomized, placebo-controlled Phase 2 trial to receive either 5mg/kg or 10mg/kg of intravenous (IV) efgartigimod, or placebo. argenx initiated an open-label extension study approximately halfway through the Phase 2 trial enabling 12 patients across the three cohorts to enroll and receive 10mg/kg of efgartigimod. This included four patients from the placebo cohort who received efgartigimod treatment for the first time.

The primary endpoint of the Phase 2 trial was safety and tolerability. Efgartigimod was reported to be well-tolerated in all patients, with most adverse events (AEs) characterized as mild and deemed unrelated to the study drug. One serious adverse event was reported in the primary study and was deemed unrelated to the study drug.

Post-hoc analyses around secondary endpoint measures relating to magnitude of effect, onset of action and durability showed efgartigimod treatment was associated with clinically meaningful improvements in platelet count to $\geq 50 \times 10^9/L$.

Magnitude of effect:

- 46% of patients improved platelet count to $\geq 50 \times 10^9/L$ during two or more visits in each of the 5mg/kg and 10 mg/kg dosing cohorts compared to 25% in the placebo cohort.
- 58% of patients (n=12) improved platelet count to $\geq 50 \times 10^9/L$ during two or more visits following the first dosing cycle of the open-label extension study.

Onset of action:

- Onset of platelet count reaching $50 \times 10^9/L$ for the first time ranged from week 1 to week 10, consistent with disease heterogeneity.
- All efgartigimod-treated patients showed a rapid and deep reduction of total IgG levels, consistent with the pharmacodynamic effects observed in previous clinical trials.

Durability:

- For efgartigimod-treated patients with clinically meaningful platelet responses ($\geq 50 \times 10^9/L$ during two or more visits), the mean duration of platelet response was 40 days versus 16 days for placebo treated patients, with responses lasting the study duration.
- 38% of efgartigimod-treated patients showed durable platelet count improvements to clinically meaningful and statistically significant levels of $\geq 50 \times 10^9/L$ for at least 10 cumulative days, compared to 0% of placebo patients (p=0.03).

argenx plans to present the full data from the Phase 2 proof-of-concept trial of efgartigimod in ITP during a workshop around the American Society of Hematology Annual Meeting (San Diego, December 1-4, 2018).

Based on these data, argenx plans to advance efgartigimod (IV) to Phase 3 development in ITP. The Company also expects to initiate a Phase 2 trial in ITP using a subcutaneous formulation of efgartigimod. Efgartigimod is currently being evaluated in a global Phase 3 registration trial in gMG and a Phase 2 proof-of-concept trial in pemphigus vulgaris (PV).

In addition, argenx announced plans to initiate a Phase 2 proof-of-concept trial of efgartigimod (IV) in chronic inflammatory demyelinating polyneuropathy (CIDP) in the first half of 2019.

The results will be discussed during a conference call and webcast presentation today at 8:00 am ET/2:00 pm CEST. The conference call can be accessed by dialling +1 929-477-0402 in the U.S. or selecting from the numbers below if international. The confirmation code is **1283706**. The webcast may be accessed on the homepage of the argenx website at www.argenx.com or by clicking [here](#).

Efgartigimod Phase 2 Immune Thrombocytopenia Trial Design

The double-blind, randomized, placebo-controlled Phase 2 trial enrolled 38 ITP patients with platelet levels $\leq 30 \times 10^9/L$ at screening despite being on standard of care therapy. Patients were randomized to receive four weekly doses of either 10mg/kg or 5mg/kg of efgartigimod or placebo in addition to their standard of care therapy. Standard of care therapies included corticosteroids and/or immunomodulatory agents, TPO-Receptor agonists and splenectomy. Patients were followed for up to 21 weeks after the treatment phase and platelet counts were monitored weekly. Patients from all three cohorts were eligible to enroll in a one-year open-label extension study at the 10mg/kg dose of efgartigimod. The primary endpoints of the trial were safety and tolerability, and secondary endpoints included effect on platelet count and use of rescue treatment, and an assessment of pharmacokinetics (PK) and pharmacodynamic (PD) markers. All 38 patients were evaluable.

About Adult Primary Immune Thrombocytopenia

In patients with adult primary ITP (~55,000 patients in the United States), pathogenic IgGs destroy platelet producing cells in the bone marrow and blood platelets, which play an active role in blood clotting and healing. Current therapies for ITP include corticosteroids, IVIg, plasmapheresis (PLEX), TPO-Receptor agonists and splenectomy. Pathogenic IgGs drive disease progression in a multimodal approach: they accelerate platelet clearance, inhibit platelet production, directly induce platelet killing and interfere with platelet's ability to perform their clotting function.

About Chronic Inflammatory Demyelinating Polyneuropathy

CIDP is a chronic, progressive autoimmune disorder (~16,000 patients in the United States) that affects peripheral nerves and nerve roots. The disease is driven by an autoimmune-mediated destruction of the myelin sheath or myelin producing cells, which serve to insulate the axon of the nerves and enable speed of signal transduction. Demyelination and axonal damage in CIDP lead to loss of sensory and/or motor neuron function, which can cause weakness, sensory loss, imbalance and/or pain. CIDP is among the largest indications for IV/SC Ig in the United States.

About Efgartigimod

Efgartigimod (ARGX-113) is an investigational therapy for IgG-mediated autoimmune diseases and was designed to exploit the natural interaction between IgG antibodies and the recycling receptor FcRn. Efgartigimod is the Fc-portion of an IgG1 antibody that has been modified by the argenx proprietary ABDEG(TM) technology to increase its affinity for FcRn beyond that of normal IgG antibodies. As a result, efgartigimod blocks antibody recycling through FcRn binding and leads to fast depletion of the autoimmune disease-causing IgG autoantibodies. The development work on efgartigimod is conducted in close collaboration with Prof. E. Sally Ward (University of Texas Southwestern Medical and Texas A&M University Health Science Center, a part of Texas A&M University (TAMHSC)).

About argenx

argenx is a clinical-stage biotechnology company developing a deep pipeline of differentiated antibody-based therapies for the treatment of severe auto-immune diseases and cancer. The company is focused on developing product candidates with the potential to be either first-in-class against novel targets or best-in-class against known, but complex, targets in order to treat diseases with a significant unmet medical need. argenx's ability to execute on this focus is enabled by its suite of differentiated technologies. The SIMPLE Antibody™ Platform, based on the powerful llama immune system, allows argenx to exploit novel and complex targets, and its three complementary Fc engineering technologies are designed to expand the therapeutic index of its product candidates.
www.argenx.com

Dial-in numbers:

Please dial in 5-10 minutes prior to 2:00 pm CET/ 8:00 am EST using the number and conference ID below.

Confirmation Code: 1283706

Belgium	Tollfree/Freephone	0800 38625
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A question and answer session will follow the presentation of the results. Go to www.argenx.com to access the live audio webcast. The archived webcast will also be available (30 days) for replay shortly after the close of the call from the "Downloads" section of the argenx website.

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Forward-looking Statements

The contents of this announcement include statements that are, or may be deemed to be, "forward-looking statements." These forward-looking statements can be identified by the use of forward-looking terminology, including the terms "believes," "estimates," "anticipates," "expects," "intends," "may," "will," or "should," and include statements argenx makes concerning the intended results of its strategy and argenx's advancement of, and anticipated clinical development and regulatory milestones and plans, including the timing and success of planned clinical trials and expected data readouts, related to efgartigimod and the commercial potential of efgartigimod. By their nature, forward-looking statements involve risks and uncertainties and readers are cautioned that any such forward-looking statements are not guarantees of future performance. argenx's actual results may differ materially from those predicted by the forward-looking statements as a result of various important factors, including argenx's expectations regarding the inherent uncertainties associated with competitive developments; preclinical study and clinical trial and product development activities and regulatory approval requirements; argenx's reliance on collaborations with third parties; estimating the commercial potential of argenx's product candidates; argenx's ability to obtain and maintain protection of intellectual property for its technologies and drugs; argenx's limited operating history; and argenx's ability to obtain additional funding for operations and to complete the development and commercialization of its product candidates. A further list and description of these risks, uncertainties and other risks can be found in argenx's U.S. Securities and Exchange Commission (SEC) filings and reports, including in argenx's most recent annual report on Form 20-F filed with the SEC as well as subsequent filings and reports filed by argenx with the SEC. Given these uncertainties, the reader is advised not to place any undue reliance on such forward-looking statements. These forward-looking statements speak only as of the date of publication of this document. argenx undertakes no obligation to publicly update or revise the information in this press release, including any forward-looking statements, except as may be required by law.