argenx Announces U.S. FDA Acceptance of Biologics License Application for Subcutaneous Efgartigimod in Generalized Myasthenia Gravis with Priority Review

- Prescription Drug User Fee Act (PDUFA) target action date is March 20, 2023

- Submission based on positive results from the Phase 3 bridging study demonstrating noninferior total IgG reduction at day 29 with subcutaneously (SC) administered efgartigimod compared to intravenous (IV) administration

November 22, 2022

Amsterdam, the Netherlands – argenx SE (Euronext & Nasdaq: ARGX), a global immunology company committed to improving the lives of people suffering from severe autoimmune diseases, today announced the U.S. Food and Drug Administration (FDA) has accepted for priority review a Biologics License Application (BLA) for SC efgartigimod (1000mg efgartigimod-PH20) for the treatment of adult patients with generalized myasthenia gravis (gMG). The application has been granted a Prescription Drug User Fee Act (PDUFA) target action date of March 20, 2023.

SC efgartigimod is co-formulated with recombinant human hyaluronidase PH20 (rHuPH20), Halozyme's ENHANZE® drug delivery technology. ENHANZE facilitates the SC injection delivery of biologics that are typically administered via IV infusion.

“The FDA’s acceptance of our BLA is an exciting step toward fulfilling our vision of delivering the broadest gMG treatment offering that reflects the unique disease experience for each patient as they navigate life with this debilitating disease. We’re excited about the potential of SC efgartigimod to offer patients multiple ways to receive treatment through various administrations and an individualized dosing schedule,” said Keith Woods, Chief Operating Officer of argenx. “With an established PDUFA date, we are preparing for our second commercial product launch and look forward to potentially bringing forth another first-in-class option for gMG patients.”

The BLA submission is supported by data from the Phase 3 ADAPT-SC study evaluating the noninferiority of the pharmacodynamic (PD) effect of SC efgartigimod as compared with IV administered VYVGART in adult patients with gMG. The majority of enrolled patients were positive for acetylcholine receptor (AChR) antibodies, but the trial also included patients where AChR antibodies were not detected.

ADAPT-SC met its primary endpoint ($p< 0.0001$) of total IgG reduction from baseline at day 29 demonstrating noninferiority of SC efgartigimod to VYVGART. Patients treated with SC efgartigimod achieved mean total IgG reduction of 66.4% from baseline at day 29, compared to 62.2% reduction with VYVGART. Results were consistent across the overall population, including those with AChR antibodies and patients where AChR antibodies were not detected. Further, 69.1% of patients treated with SC efgartigimod were responders on the Myasthenia Gravis Activities of Daily Living (MG-ADL) score. Responders are defined as having at least a two-point improvement on the MG-ADL score for at least four consecutive weeks. 65.5% of patients treated with SC efgartigimod were responders on the Quantitative Myasthenia Gravis (QMG) score. Responders are defined as having at least a three-

point improvement on the QMG score for at least four consecutive weeks. Minimal symptom expression (MSE), a measure of symptom-free status, was achieved in 37% of SC efgartigimod-treated patients after one treatment cycle. Onset of effect was also consistent with the Phase 3 ADAPT study.

The safety profile for SC efgartigimod was consistent with the ADAPT study. It was generally well-tolerated; the most frequent adverse event being injection site reactions (ISRs), commonly observed with biologics administered subcutaneously. All ISRs were mild to moderate and resolved over time. After completing ADAPT-SC, 95% of participants entered ADAPT-SC+, a three-year open-label extension study evaluating the long-term safety and tolerability of SC efgartigimod.

Phase 3 ADAPT-SC Trial Design

The Phase 3 ADAPT-SC trial was a multicenter, randomized, open-label, parallel-group study evaluating the noninferiority of the pharmacodynamic (PD) effect of SC efgartigimod (1000mg efgartigimod-PH20) as compared with VYVGART (10mg/kg) in patients with gMG. The pharmacodynamic effect as measured by percent change from baseline in total IgG levels at day 29, one week after the last dose of IV or SC efgartigimod, served as the primary endpoint in the ADAPT-SC trial. The correlation between total IgG reduction and clinical benefit in gMG was demonstrated in a Phase 2 trial and the Phase 3 ADAPT trial, which served as the basis for approval of VYVGART in the U.S., Japan and Europe. Safety, clinical efficacy, immunogenicity and pharmacokinetics (PK) were also assessed.

A total of 110 adult patients with gMG in North America, Europe and Japan enrolled in the ADAPT-SC trial and were treated. Inclusion criteria of the trial were the same as the Phase 3 ADAPT trial of VYVGART; enrolled patients had a confirmed gMG diagnosis and an MG-ADL total score of at least 5 with greater than 50% of the total score attributed to non-ocular symptoms, at screening and baseline. Patients were on a stable dose of at least one gMG treatment prior to randomization, including acetylcholinesterase inhibitors, corticosteroids or nonsteroidal immunosuppressive drugs, and were required to remain on that stable dose throughout the primary trial. Patients were eligible to enroll in ADAPT-SC regardless of antibody status, including patients with AChR antibodies (AChR-Ab+) and patients where AChR antibodies were not detected.

Patients were randomized in a 1:1 ratio to receive SC efgartigimod or IV efgartigimod for one treatment cycle consisting of four doses at weekly intervals. The total study duration was approximately 12 weeks, including seven weeks of follow-up after the treatment cycle.

See the full Prescribing Information for VYVGART in the U.S., which includes the below Important Safety Information. For more information related to VYVGART in Japan, visit argenx.jp.

IMPORTANT SAFETY INFORMATION FOR VYVGART® (efgartigimod alfa-fcab) intravenous (IV) formulation (U.S. PRESCRIBING INFORMATION)

What is VYVGART® (efgartigimod alfa-fcab)?

VYVGART is a prescription medicine used to treat a condition called generalized myasthenia gravis, which causes muscles to tire and weaken easily throughout the body, in adults who are positive for antibodies directed toward a protein called acetylcholine receptor (anti-AChR antibody positive).

What is the most important information I should know about VYVGART?

VYVGART may cause serious side effects, including:

Infection. VYVGART may increase the risk of infection. In a clinical study, the most common infections were urinary tract and respiratory tract infections. More patients on VYVGART vs placebo had below normal levels for white blood cell counts, lymphocyte counts, and neutrophil counts. The majority of infections and blood side effects were mild to moderate in severity. Your health care provider should check you for infections before starting treatment, during treatment, and after treatment with VYVGART. Tell your health care provider if you have any history of infections. Tell your health care provider right away if you have signs or symptoms of an infection during treatment with VYVGART such as fever, chills, frequent and/or painful urination, cough, pain and blockage of nasal passages/sinus, wheezing, shortness of breath, fatigue, sore throat, excess phlegm, nasal discharge, back pain, and/or chest pain.

Undesirable immune reactions (hypersensitivity reactions). VYVGART can cause the immune system to have undesirable reactions such as rashes, swelling under the skin, and shortness of breath. In clinical studies, the reactions were mild or moderate and occurred within 1 hour to 3 weeks of administration, and the reactions did not lead to VYVGART discontinuation. Your health care provider should monitor you during and after treatment and discontinue VYVGART if needed. Tell your health care provider immediately about any undesirable reactions.

Before taking VYVGART, tell your healthcare provider about all of your medical conditions, including if you:

Have a history of infection or you think you have an infection.
Have received or are scheduled to receive a vaccine (immunization). Discuss with your health care provider whether you need to receive age-appropriate immunizations before initiation of a new treatment cycle with VYVGART. The use of vaccines during VYVGART treatment has not been studied, and the safety with live or live-attenuated vaccines is unknown. Administration of live or live-attenuated vaccines is not recommended during treatment with VYVGART.
Are pregnant or plan to become pregnant and are breastfeeding or plan to breastfeed.
Tell your health care provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

What are the common side effects of VYVGART?

The most common side effects of VYVGART are respiratory tract infection, headache, and urinary tract infection.

These are not all the possible side effects of VYVGART. Call your doctor for medical advice about side effects. You may report side effects to the US Food and Drug Administration at 1-800-FDA-1088.

Please see the full Prescribing Information for VYVGART and talk to your doctor.

About Efgartigimod

Efgartigimod is an antibody fragment designed to reduce pathogenic immunoglobulin G (IgG) antibodies by binding to the neonatal Fc receptor and blocking the IgG recycling process. Efgartigimod is being investigated in several autoimmune diseases known to be mediated by disease-causing IgG antibodies, including neuromuscular disorders, blood disorders, and skin blistering diseases, in both an intravenous and subcutaneous (SC) formulation. SC efgartigimod is co-formulated with recombinant human hyaluronidase PH20 (rHuPH20), Halozyme's ENHANZE® drug delivery technology.

About VYVGART

VYVGART® (efgartigimod alfa-fcab) is a human IgG1 antibody fragment that binds to the neonatal Fc receptor (FcRn), resulting in the reduction of circulating immunoglobulin G (IgG) autoantibodies. It is the first and only approved FcRn blocker. VYVGART is approved in the United States and Europe for the treatment of adults with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody positive, and in Japan for the treatment of adults with gMG who do not have sufficient response to steroids or non-steroidal immunosuppressive therapies (ISTs).

About Generalized Myasthenia Gravis

Generalized myasthenia gravis (gMG) is a rare and chronic autoimmune disease where IgG autoantibodies disrupt communication between nerves and muscles, causing debilitating and potentially life-threatening muscle weakness. Approximately 85% of people with MG progress to gMG within 24 months[1], where muscles throughout the body may be affected. Patients with confirmed AChR antibodies account for approximately 85% of the total gMG population.

About argenx

argenx is a global immunology company committed to improving the lives of people suffering from severe autoimmune diseases. Partnering with leading academic researchers through its Immunology Innovation Program (IIP), argenx aims to translate immunology breakthroughs into a world-class portfolio of novel antibody-based medicines. argenx developed and is commercializing the first-and-only approved neonatal Fc receptor (FcRn) blocker in the U.S., Japan and the EU. The Company is evaluating efgartigimod in multiple serious autoimmune diseases and advancing several earlier stage experimental medicines within its therapeutic franchises. For more information, visit www.argenx.com and follow us on LinkedIn, Twitter, and Instagram.

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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," "hope," "estimates," "anticipates," "expects," "intends," "may," "will," or "should" and include statements argenx makes concerning the acceptance of the Biologics License Application to the U.S. Food and Drug Administration for Subcutaneous (SC) Efgartigimod for Treatment of Generalized Myasthenia Gravis, the potential commercial launch of SC efgartigimod for treatment of generalized myasthenia gravis and the long-term safety and tolerability of SC 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. 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 publicly update or revise the information in this press release, including any forward-looking statements, except as may be required by law.