argenx Advances Clinical Development of Efgartigimod SC in Idiopathic Inflammatory Myopathies

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- Phase 2 data establish proof-of-concept of efgartigimod SC in myositis
- Enrollment to continue in Phase 3 across all three subtypes (IMNM, ASyS, DM) under evaluation in ALKIVIA
- Potential for efgartigimod SC to be first targeted approach for myositis patients who have limited treatment options

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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 decision to continue development of efgartigimod subcutaneous (SC) (efgartigimod alfa and hyaluronidase-qvfc) in the ongoing Phase 2/3 ALKIVIA study in adults with idiopathic inflammatory myopathies (IIM or myositis), following analysis of topline data from the Phase 2 portion of the study. ALKIVIA will continue to enroll patients across each of the three myositis subtypes in the study, including immune-mediated necrotizing myopathy (IMNM), anti-synthetase syndrome (ASyS), and dermatomyositis (DM).

“Efgartigimod SC continues to show its promise for patients suffering from chronic autoimmune diseases,” said Luc Truyen, M.D., Ph.D., Chief Medical Officer of argenx. “Idiopathic inflammatory myopathies are debilitating diseases that can cause muscle weakness, affect multiple organs, and have a severe impact on patients’ quality of life, including increased morbidity and early mortality. We are excited to continue the development of efgartigimod SC across all three subtypes, allowing us to explore the broad potential of this precision therapy for those whose needs remain unmet by current treatments like steroids, plasma-derived therapies, and broad immunosuppressants. We are grateful for the patients and investigators participating in the ALKIVIA study, and hope to bring efgartigimod to patients living with myositis as soon as possible.”

The decision to continue clinical development of efgartigimod SC in each of the three myositis subtypes is supported by the efficacy and safety results from the Phase 2 portion of the seamless Phase 2/3 ALKIVIA study. Overall, the study met its primary endpoint, demonstrating a statistically significant treatment effect in mean total improvement score (TIS) at Week 24, and showed improvement across all six core set measures of the TIS in favor of efgartigimod SC compared to placebo. The observed safety and tolerability profile was consistent to that demonstrated with other clinical trials.

ALKIVIA Study Design

The ALKIVIA study is a randomized, double-blind, placebo-controlled, multicenter, operationally seamless Phase 2/3 study of efgartigimod SC for the treatment of idiopathic inflammatory myopathies (IIM or myositis) across three subtypes, including immune-mediated necrotizing myopathy (IMNM), anti-synthetase syndrome (ASyS), and dermatomyositis (DM). The ALKIVIA study will enroll 240 patients in total and is being conducted in two phases, with an analysis of the Phase 2 portion of the clinical trial after the first 90 patients completed the study, followed by a Phase 3 portion if a signal is observed in the Phase 2 portion. The primary endpoint is the mean total improvement score (TIS) at the end of the treatment period (24 weeks in Phase 2 and 52 weeks in Phase 3) of all treated patients (IMNM, ASyS, DM) compared to placebo. Key secondary endpoints include response rates at the end of treatment, time to response, and duration of response in TIS, as well as change from baseline in individual TIS components. Other secondary endpoints include quality of life and other functional scores.

About Idiopathic Inflammatory Myopathies

Idiopathic inflammatory myopathies (myositis) are a rare group of autoimmune diseases that can be muscle specific or affect multiple organs including the skin, joints, lungs, gastrointestinal tract and heart. Myositis can be very severe and disabling and have a material impact on quality of life. Initially, myositis was classified as either DM or polymyositis, but as the underlying pathophysiology of myositis has become better understood, including through the identification of characteristic autoantibodies, new polymyositis subtypes have emerged. Two of these subtypes are IMNM and ASyS. Proximal muscle weakness is a unifying feature of each subtype. IMNM is characterized by skeletal muscle weakness due to muscle cell necrosis. ASyS is characterized by muscle inflammation, inflammatory arthritis, interstitial lung disease, thickening and cracking of the hands (“mechanic’s hands”) and Raynaud’s phenomenon. DM is characterized by muscle inflammation and degeneration and skin abnormalities, including heliotrope rash, Gottron’s papules, erythematous, calcinosis and edema.

About Efgartigimod SC

Efgartigimod SC (efgartigimod alfa and hyaluronidase-qvfc) is a human IgG1 antibody fragment designed to reduce pathogenic immunoglobulin G (IgG) antibodies by binding to the neonatal Fc receptor (FcRn) and blocking the IgG recycling process. Efgartigimod SC is the first-approved FcRn blocker globally and is marketed as VYVGART® Hytrulo in the United States and China for the treatment of generalized myasthenia gravis (gMG) and chronic inflammatory demyelinating polyneuropathy (CIDP), and as VYVGART SC or VYVDURA (Japan) for gMG in other regions globally. Efgartigimod SC is currently being evaluated in more than 15 severe autoimmune diseases where pathogenic IgGs are believed to be mediators of disease.

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 approved neonatal Fc receptor (FcRn) blocker, globally in the U.S., Japan, Israel, the EU, the UK, China and Canada. 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, X/Twitter, Instagram, Facebook, and YouTube.

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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 "aim," "continue," "hope," "potential," or "will," and include statements argenx makes concerning its continued development of efgartigimod SC (efgartigimod alfa and hyaluronidase-qvfc) in the ongoing Phase 2/3 ALKIVIA study; its plan to continue enrollment of patients across all three myositis subtypes (IMNM, ASyS, DM) under evaluation in the ALKIVIA study; the potential of efgartigimod SC to be the first targeted treatment approach for myositis patients who have limited other treatment options and whose needs remain unmet by current treatments; its hope to bring efgartigimod to patients living with myositis as soon as possible; its plan for the study design of the ALKIVIA study; and its goal of translating immunology breakthroughs into a world-class portfolio of novel antibody-based medicines. 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 the results of argenx's clinical trials; expectations regarding the inherent uncertainties associated with the development of novel drug therapies; preclinical and clinical trial and product development activities and regulatory approval requirements in products and product candidates; the acceptance of argenx's products and product candidates by patients as safe, effective and cost-effective; the impact of governmental laws and regulations on our business; disruptions caused on our reliance of third parties suppliers, service provides and manufacturing; inflation and deflation and the corresponding fluctuations in interest rates; and regional instability and conflicts. 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.