argenx Advances Clinical Development of ARGX-119 in Congenital Myasthenic Syndromes

Phase 1b study supports proof-of-concept in DOK7 congenital myasthenic syndromes

Decision informed by favorable safety profile and consistent functional improvement over time across multiple efficacy measures

Advancing ARGX-119 further validates strong track record of Immunology Innovation Program (IIP), argenx's collaborative discovery model

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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 its plan to advance the clinical development of ARGX-119, a first-in-class agonist antibody to muscle-specific kinase (MuSK), to a registrational study in patients with congenital myasthenic syndromes (CMS) following the analysis of topline data from the Phase 1b study. Detailed results will be presented at a future medical meeting.

“The results of our Phase 1b ARGX-119 study in congenital myasthenic syndromes, an ultra-rare disorder that affects patients from birth, builds on our experience and understanding of myasthenic disorders and aligns with our aspiration to serve even more patients living with these debilitating diseases,” said Luc Truyen, M.D., Ph.D., Chief Medical Officer of argenx.

“ARGX-119 is the sixth molecule developed through our Immunology Innovation Program to show proof-of-concept, reflecting the strength of our innovation model where our deep knowledge of the biology and expertise in antibody engineering come together to push the boundaries of what’s possible. argenx remains focused on uncovering new biological insights into misunderstood diseases to meaningfully change the lives of patients who have long-been underserved,” said Peter Ulrichts, Ph.D., Chief Scientific Officer of argenx.

The decision to advance the development of ARGX-119 in CMS is supported by the results of the Phase 1b study. ARGX-119 demonstrated a favorable safety and tolerability profile, which was the primary endpoint. Efficacy was evaluated across multiple secondary and exploratory endpoints, including Six-Minute Walk Test (6MWT), Quantitative Myasthenia Gravis (QMG) score and Myasthenia Gravis Activities of Daily Living (MG-ADL) score. Consistent improvements were observed in treated DOK7-CMS patients through the 12-week study across multiple efficacy scores.

Phase 1b CMS Study Design
The Phase 1b, multicenter, randomized, double-blinded, placebo-controlled clinical trial assessed safety, tolerability, PK, immunogenicity, and preliminary efficacy of ARGX-119 in participants with DOK7-CMS. The study was also designed to demonstrate proof-of-biology through preliminary efficacy assessments, including muscle weakness, fatigability, daily activities and patient-reported global health outcomes. The clinical trial spanned approximately 11 months across a screening period (up to 28 days), a 12-week treatment period and a follow-up period of nearly seven months. At baseline, eligible participants were randomized 4:1 to receive intravenous ARGX-119 or placebo. The primary objective was to evaluate safety and tolerability; secondary objectives included PK, immunogenicity and preliminary efficacy of ARGX-119 in participants with DOK7-CMS. All but one of the patients enrolled in the Phase 1b study also participated in an observational natural history study initiated by argenx in 2024 to better understand the CMS patient journey and disease burden, helping to inform future development plans.

About Congenital Myasthenic Syndromes
Congenital Myasthenic Syndromes (CMS) are an ultra-rare and heterogenous group of congenital neuromuscular disorders caused by genetic defects that are essential for the integrity of the neuromuscular junction. Early age of onset and fatigable muscle weakness are considered clinical hallmarks of CMS. Muscle weakness can be debilitating and life-threatening causing difficulties in speaking or swallowing, impaired or absent mobility, proximal arm and leg weakness, and respiratory insufficiency. DOK7 variations are one of the more frequent and severe causes of CMS, accounting for approximately 24% of CMS cases. There are no approved treatments. The prevalence of CMS is estimated to be 5 per 1M (DOK7-CMS estimated to be 1.2 per 1M).

About ARGX-119
ARGX-119 is a first-in-class humanized agonist monoclonal antibody (mAb) that specifically targets and activates muscle-specific tyrosine kinase (MuSK) to promote maturation and stabilization of the neuromuscular junction (NMJ). It is a mAb derived from llamas and discovered using the argenx SIMPLE Antibody™ platform technology. ARGX-119 is being developed for patients with neuromuscular disease, including congenital myasthenic syndromes (CMS), amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy (SMA). ARGX-119 was developed through argenx’s IIP program in collaboration with the world’s leading key opinion leaders on MuSK and the NMJ, Professor Steven J. Burden from MGH, Professor Shohei Koide from NYU and Professor Jan Verschuuren and Associate Professor Maartje Huijbers from LUMC.

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 and is evaluating its broad potential in multiple serious autoimmune diseases while advancing several earlier stage experimental medicines within its therapeutic franchises. For more information, visit www.argenx.com and follow us on LinkedIn, 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 “aims,” “committed,” or “plan” and include statements argenx makes concerning its commitment to improving the lives of people suffering from severe autoimmune diseases; its plan to advance the clinical development of ARGX-119 to a registrational study in CMS patients; 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 but not limited to, 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; the acceptance of its products and product candidates by its patients as safe, effective and cost-effective; the impact of governmental laws and regulations, including tariffs, export controls, sanctions and other regulations on its business; its reliance on third-party suppliers, service providers and manufacturers; 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.