



## **argenx announces publication of Phase 3 ADAPT trial results of efgartigimod for the treatment of generalized myasthenia gravis in The Lancet Neurology**

June 16, 2021

*Results from ADAPT showed treatment with efgartigimod provided clinically meaningful improvements in strength and quality of life measures in generalized myasthenia gravis (gMG)*

*Efgartigimod is under U.S. Food and Drug Administration (FDA) review with December 17, 2021 Prescription Drug User Fee Act (PDUFA) target action date*

Breda, the Netherlands —Jun. 16, 2021—argenx (Euronext & Nasdaq: ARGX), today announced that *The Lancet Neurology* has published pivotal trial results from the Phase 3 ADAPT trial of efgartigimod, an FcRn antagonist, for the treatment of adults living with generalized myasthenia gravis (gMG). Efgartigimod is currently under review with the U.S. Food and Drug Administration (FDA) for the treatment of gMG with a Prescription Drug User Fee Act (PDUFA) target action date of December 17, 2021, and if approved, would be the first-and-only approved FcRn antagonist.

"Myasthenia gravis can have a devastating impact on a person's life and independence, potentially affecting one's ability to swallow, speak, walk and even breathe. In addition, each patient experiences the course of MG differently, which can make disease management unpredictable," said James F. Howard Jr., M.D., Professor of Neurology (Neuromuscular Disease), Medicine and Allied Health, Department of Neurology, The University of North Carolina at Chapel Hill School of Medicine and principal investigator for the ADAPT trial. "In the ADAPT trial, we observed clinically meaningful improvements in the first two weeks of dosing in a majority of patients treated with efgartigimod. These results are important for the MG community and I am hopeful efgartigimod will provide a first-in-class targeted therapy that can be dosed in an individual way for people living with this chronic autoimmune disease."

The ADAPT trial met its primary endpoint demonstrating significantly more acetylcholine receptor-antibody positive (AChR-Ab+) gMG patients were responders on the Myasthenia Gravis Activities of Daily Living (MG-ADL) score following treatment with efgartigimod compared with placebo (67.7% vs. 29.7%;  $p < 0.0001$ ). Responders were defined as having at least a two-point improvement sustained for four or more consecutive weeks on the MG-ADL score. Additionally, 40% of patients treated with efgartigimod achieved minimal symptom expression defined as MG-ADL scores of zero (symptom free) or one, compared to 11.1% of patients who received placebo. Among AChR-Ab+ responders, 84.1% showed clinically meaningful improvement on the MG-ADL score within the first two weeks of treatment. The safety profile of efgartigimod was comparable to placebo.

After completing ADAPT, 90% of participants entered ADAPT-plus, an ongoing three-year open-label extension study evaluating the long-term safety and tolerability of efgartigimod. In total across ADAPT and ADAPT-plus, at least 118 patients have been on efgartigimod therapy for 12 months or more.

"The publication of the ADAPT results provides an exciting opportunity to share these data with the clinical community as we aim to introduce a new treatment option for gMG patients. gMG is a chronic, debilitating and potentially life-threatening disease where both the disease symptoms and side effects from current therapies can cause significant impairment on a person's life," said Wim Parys, M.D., Chief Medical Officer of argenx. "Efgartigimod is currently under review with the FDA for the treatment of gMG, and if approved, we look forward to bringing this therapy to MG patients who are in great need of new treatment options."

### **Phase 3 ADAPT Trial**

The Phase 3 ADAPT trial was a randomized, double-blind, placebo-controlled, multi-center, global trial evaluating the safety and efficacy of efgartigimod in patients with gMG. A total of 167 adult patients with gMG in North America, Europe and Japan enrolled in the trial and were treated. Patients were eligible to enroll in ADAPT 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 efgartigimod or placebo for a total of 26 weeks. ADAPT was designed to enable an individualized treatment approach with an initial treatment cycle followed by a variable number of subsequent treatment cycles. The primary endpoint was the number of AChR-Ab+ patients who achieved a response on the MG-ADL score defined by at least a two-point improvement for four or more consecutive weeks.

### **About Efgartigimod**

Efgartigimod is an investigational antibody fragment designed to reduce disease-causing immunoglobulin G (IgG) antibodies and block the IgG recycling process. Efgartigimod binds to the neonatal Fc receptor (FcRn), which is widely expressed throughout the body and plays a central role in rescuing IgG antibodies from degradation. Blocking FcRn reduces IgG antibody levels representing a logical potential therapeutic approach for several autoimmune diseases known to be driven by disease-causing IgG antibodies, including: myasthenia gravis (MG), a chronic disease that causes muscle weakness; pemphigus vulgaris (PV), a chronic disease characterized by severe blistering of the skin; immune thrombocytopenia (ITP), a chronic bruising and bleeding disease; and chronic inflammatory demyelinating polyneuropathy (CIDP), a neurological disease leading to impaired motor function.

### **About Myasthenia Gravis**

Myasthenia gravis (MG) is a rare and chronic autoimmune disease, often causing debilitating and potentially life-threatening muscle weakness. More than 85% of people with MG progress to generalized MG (gMG) within 18 months, where muscles throughout the body may be affected, resulting in extreme fatigue and difficulties with facial expression, speech, swallowing and mobility. In more life-threatening cases, MG can affect the muscles

responsible for breathing. There are approximately 65,000 people in the United States and 20,000 people in Japan living with the disease.

#### **About argenx**

argenx is a global immunology company committed to improving the lives of people suffering from severe autoimmune diseases and cancer. 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 is evaluating efgartigimod in multiple serious autoimmune diseases. argenx is also advancing several earlier stage experimental medicines within its therapeutic franchises. argenx has offices in Belgium, the United States, Japan, and Switzerland. For more information, visit [www.argenx.com](http://www.argenx.com) and follow us on LinkedIn at <https://www.linkedin.com/company/argenx/> and Twitter at <https://twitter.com/argenxglobal>.

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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,” “could,” “estimates,” “anticipates,” “expects,” “intends,” “plan,” “may,” “will,” or “should” and include statements argenx makes concerning the Prescription Drug User Fee Act (PDUFA) target action date of December 17, 2021; the clinical and commercial potential of efgartigimod and clinical studies of efgartigimod or its commercial readiness; its hope that efgartigimod will provide a first-in-class targeted therapy that can be dosed in an individual way; its statements regarding the therapeutic potential of efgartigimod in patients; the therapeutic potential of its product candidates; and the intended results of its strategy. 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 regulatory approval requirements and process, the effects of the COVID-19 pandemic, argenx’s expectations regarding its the inherent uncertainties associated with competitive developments, preclinical and clinical trial and product development activities; 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.