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# argenx receives feedback from Japan's PMDA on Phase 3 clinical trial and regulatory pathway for efgartigimod in generalized myasthenia gravis

August 29, 2018

**Breda, the Netherlands / Ghent, Belgium** – argenx (Euronext & Nasdaq: ARGX), a clinical-stage biotechnology company developing a deep pipeline of differentiated antibody-based therapies for the treatment of severe autoimmune diseases and cancer, today announced it has received feedback from the Pharmaceuticals and Medical Devices Agency (PMDA) in Japan on the design of a Phase 3 trial and regulatory pathway towards potential marketing authorization of efgartigimod (ARGX-113) in patients with generalized myasthenia gravis (gMG).
Based on feedback from the PMDA, argenx expects the data from the planned global Phase 3 registration trial, if positive, will serve as the basis to submit for marketing authorization in Japan.

"Japan represents a very important potential market for us and one we see as a top priority as we advance the development of efgartigimod in gMG patients globally. There remains a significant unmet need in Japan for new treatment options for MG, and we are grateful for the feedback from the PMDA in facilitating a regulatory path towards achieving this goal," commented Keith Woods, Chief Operating Officer of argenx. "We look forward to launching the global Phase 3 trial of efgartigimod in gMG before the end of the year, including in sites across Japan, and continuing the ongoing discussions with the PMDA as we map out a potential path to market for our drug candidate, if approved."

The global Phase 3 trial expects to enroll approximately 150 patients with gMG, including patients from Japan as well as North America and Europe. The trial will be placebo-controlled and will evaluate the efficacy over 26 weeks of a 10 mg/kg dose of efgartigimod in approximately 150 gMG patients, including both acetylcholine receptor (AChR) autoantibody positive and AChR autoantibody negative patients whose disease is driven primarily by MuSK and LRP4 autoantibodies. In addition, patients can roll over into an open-label extension study for a period of one year.

argenx plans to initiate the global Phase 3 registration trial of efgartigimod in gMG before the end of 2018. Data from the trial, if positive, may also serve as the basis for a Biologics License Application (BLA) in the U.S. based on feedback from the U.S. Food and Drug Administration (FDA).

**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 antibody that has been modified by the argenx proprietary ABDEG™ 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 AntibodyTM 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.

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