# argenx announces publication of full data from Phase 1 healthy volunteer study of efgartigimod in Journal of Clinical Investigation

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**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 the publication in the *Journal of Clinical Investigation* (JCI) of the full study results from the Phase 1 study of FcRn-antagonist efgartigimod (ARGX-113) in healthy volunteers. A link to the JCI publication can be accessed here.

"The Phase 1 data published today in the peer-reviewed *Journal of Clinical Investigation* highlight the unique design of efgartigimod as an Fc fragment modified by our proprietary ABDEG(TM) technology. The drug candidate's binding properties with FcRn are enhanced by the ABDEG mutation, which may contribute to the differentiated pharmacokinetic and pharmacodynamic activity we saw in the Phase 1 study, as well as its favorable tolerability profile," said Nicolas Leupin, CMO of argenx. "We have launched a broad development strategy to explore efgartigimod in a range of indications mediated by pathogenic IgGs to further our understanding of its mechanism of action and evaluate whether FcRn blockage translates into clinical improvements in severe autoimmune diseases."

The Phase 1 data published in JCI show that the FcRn antagonist efgartigimod was observed to have a favorable tolerability profile and led to a specific, profound and sustained reduction of total immunoglobulin G (IgG) levels. In healthy volunteers, multiple doses of efgartigimod lowered IgG levels on average by approximately 75% with levels returning to baseline approximately eight weeks following the last administration. Efgartigimod was observed to be selective to IgG and did not affect the homeostasis of albumin or immunoglobulins other than IgG.

These results have been further supported in a Phase 2 proof-of-concept trial in myasthenia gravis (MG) and in the first cohort of patients from a Phase 2 trial in pemphigus vulgaris (PV). Data to-date showed the IV formulation of efgartigimod was well-tolerated with promising pharmacodynamic effects relating to speed, depth and duration of total and pathogenic IgG reduction. The reductions seen in the MG trial in total and pathogenic IgGs also correlated with clinically meaningful and statistically significant improvements in clinical disease scores. The full dataset from the Phase 2 proof-of-concept trial in PV is expected in the first half of 2019. A Phase 3 trial of MG is expected to launch before the end of the year. Additionally, argenx is studying efgartigimod in a Phase 2 proof-of-concept trial in immune thrombocytopenia expected to read out in the third quarter of 2018.

#### 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(TM) 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 done 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 Antibody<sup>TM</sup>Platform, based on the powerful llama immune system, allows

argenx to exploit novel and complex targets, and the three antibody engineering technologies are designed to enable expansion of the therapeutic index of the company's product candidates. www.argenx.com

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#### Forward-looking Statements

The contents of this announcement include statements that are, or may be deemed to be, "forwardlooking statements." These forward-looking statements can be identified by the use of forward-looking terminology, including the terms "believes," "estimates," "anticipates," "expects," "intends," "may," "will," or "should," and include statements argenx makes concerning the intended results of its strategy and argenx's advancement of, and anticipated clinical development and regulatory milestones and plans, including the timing of planned clinical trials and expected data readouts, related to 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, including argenx's expectations regarding its the inherent uncertainties associated with competitive developments, preclinical and clinical trial and product development activities and regulatory approval requirements; 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 product candidates; 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.