

## **argenx reports interim data from first cohort of Phase 2 proof-of-concept clinical trial of efgartigimod for the treatment of pemphigus vulgaris**

- Rapid disease control observed in 4/6 patients
- Strong PD effect correlates with improvement in PDAI score
- Favorable tolerability profile; Independent Data Monitoring Committee (IDMC) recommends advancing to cohort 2

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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 interim data from the first cohort of the Phase 2 proof-of-concept clinical trial of efgartigimod (ARGX-113) in pemphigus vulgaris (PV) patients.

"There is a clear unmet need among PV patients for a treatment to better manage their disease. The data announced today on efgartigimod are very encouraging, particularly around the speed in which patients reach disease control as characterized by the start of healing of existing lesions and absence of formation of new lesions," commented Nicolas Leupin, CMO of argenx. "This is the second disease indication in which we have observed the potential of efgartigimod to yield fast reduction in pathogenic IgG levels, resulting in a rapid clinical benefit to patients. This validates both the mechanism of action and the pipeline in product potential of efgartigimod."

In the first cohort of the Phase 2 trial, six mild to moderate PV patients with no or low-dose corticosteroid therapy were treated with efgartigimod. Disease control was reached in three out of six patients in one week, which was characterized by patients having signs of healing of existing lesions and the absence of new lesions forming. One patient reached disease control after four weeks. Two patients had progression of disease. In all patients exhibiting disease control, a mean maximum reduction in Pemphigus Disease Area Index (PDAI) of 55% correlated with a mean maximum decrease in pathogenic autoantibodies levels of 57%. No meaningful anti-drug antibody signals were reported.

Efgartigimod was well-tolerated in all treated PV patients with no severe or serious study drug-related adverse events reported.

The Independent Data Monitoring Committee (IDMC) evaluated the results of the first patient cohort and determined the tolerability profile to be favorable. The IDMC recommended maintaining the dose at 10 mg/kg, but adjusted the dosing frequency and duration of the maintenance phase for the next cohort. The second patient cohort will dose every two weeks during the maintenance phase and will add two additional administrations for a period of eight total weeks of maintenance, up from six weeks in cohort 1.

### **About the efgartigimod Phase 2 PV Trial**

The open-label, non-controlled Phase 2 clinical trial is expected to enroll approximately 12 patients with mild to moderate PV. Patients in cohort 1 were dosed for an initial three weeks as an induction period followed by a six-week maintenance period and a subsequent eight-week follow-up period. The primary endpoints of the trial are safety and tolerability, and secondary endpoints include efficacy and an assessment of pharmacokinetics (PK), pharmacodynamic (PD) markers and immunogenicity.

### **About efgartigimod Phase 2 Trials**

Efgartigimod is currently being evaluated in two Phase 2 proof-of-concept trials for immune thrombocytopenia (ITP) and pemphigus vulgaris (PV). argenx is also preparing for a Phase 3 clinical trial in myasthenia gravis (MG), which is expected to begin before the end of the year. In a Phase 2 clinical trial in generalized MG (gMG) patients, efgartigimod treatment was well-tolerated and showed promising pharmacodynamic effects relating to speed, depth and duration of total IgG and pathogenic IgG

reduction. 75% of gMG patients treated with efgartigimod had a clinically meaningful and statistically significant improvement in Myasthenia Gravis Activity-of-Daily-Living (MG-ADL) scores (at least a two-point reduction from baseline) for a period of at least six consecutive weeks, versus 25% of patients on the placebo ( $p = 0.0391$ ).

### **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 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 Antibody™ 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 the expansion of the therapeutic index of the company's product candidates. [www.argenx.com](http://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, "forward-looking 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 Phase 2 program in PV, the progress of its Phase 2 clinical trials of efgartigimod; the timing of expected data readouts related to efgartigimod; and the commercial potential of 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; the risk that interim, topline and preliminary data from argenx's clinical trials may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data; 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.*