arGEN-X Submits Investigational New Drug Application to Evaluate ARGX-110 in Waldenström's Macroglobulinemia

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Breda, the Netherlands / Ghent, Belgium – arGEN-X N.V. (Euronext Brussels: ARGX), a clinical-stage biopharmaceutical company focused on creating and developing differentiated therapeutic antibodies for the treatment of cancer and severe autoimmune diseases, announced today the submission of an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) to initiate a Phase 1b/2 trial of ARGX-110 in patients with relapsed or refractory Waldenström's macroglobulinemia. ARGX-110 is a novel anti-CD70 antibody currently being evaluated in a Phase 1b study in hematological and solid cancers in Europe.

"The IND submission is a very important step in the clinical development of ARGX-110 and highlights our commitment to evaluate the drug in patients with orphan diseases and few treatment options. Based on a sound biological rationale, we are eager to advance ARGX-110 further in the clinic and plan to do so upon FDA's decision," said Alain Thibault, CMO of arGEN-X. "We feel fortunate to have support from The Leukemia & Lymphoma Society in developing this program in Waldenström's macroglobulinemia and look forward to getting underway."

The Phase 1b/2 study planned aims to enrol 30 patients and will be conducted at two leading cancer centers in the US: Dana-Farber Cancer Institute and Memorial Sloan Kettering Cancer Center. The principal investigator of the study is Steven P. Treon, MD, PhD, Director of the Bing Center for Waldenström's macroglobulinemia at Harvard Medical School and a leading authority on the disease and its treatment. The trial is sponsored by the Leukemia & Lymphoma Society (LLS), under an agreement signed between LLS and arGEN-X in June 2014. The Phase 2 part of the study will assess overall safety and efficacy of ARGX-110 following a dedicated dose optimization (Phase 1b). Both phases will correlate an extensive panel of biomarkers with clinical outcome. Patient enrolment in the study is planned to start during the first quarter of 2015, to complete recruitment by 1H 2016, with efficacy results expected in 2017.

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Notes to Editors

About ARGX-110

ARGX-110 is a SIMPLE Antibody™ targeting CD70, an immune checkpoint target involved in hematological malignancies, several solid tumors and severe autoimmune diseases. ARGX-110 works in three ways: i) blocks growth of tumor cells, ii) kills cancer cells and iii) restores immune surveillance against tumors (ref. 1). ARGX-110 is currently being evaluated in hematological and solid tumors in a Phase 1b study in Europe and will be developed in partnership with the Leukemia & Lymphoma Society (LLS) for the rare lymphoma Waldenström's macroglobulinemia.

About Waldenström's macroglobulinemia

Waldenström's macroglobulinemia is a rare type of B-cell lymphoma in which overactive CD70 signaling contributes to tumor growth. In pre-clinical studies, antibody-mediated CD70 blockade

arrests tumor growth (ref. 2). While most patients respond to combinations of cytotoxic and/or targeted therapy (small molecules or biologics), it has a low cure rate and recurrence is common, defining an unmet medical need for a significant number of patients. In the US only, Waldenström's macroglobulinemia affects an average of four people per million, qualifying it as an orphan disease.

References

1. Silence K. *et al*. ARGX-110, a highly potent antibody targeting CD70, eliminates tumors via both enhanced ADCC and immune checkpoint blockade. *mAbs* 2014; 6 (2):523-532.
2. Ho A.W. *et al.* CD27-CD70 interactions in the pathogenesis of Waldenström's macroglobulinemia. *Blood* 2008; 112 (12): 4683-4689.

About arGEN-X

arGEN-X is a clinical-stage biopharmaceutical company focused on creating and developing differentiated therapeutic antibodies for the treatment of cancer and severe autoimmune diseases. arGEN-X has generated a pipeline of differentiated clinical and preclinical antibody candidates using its SIMPLE Antibody™ discovery platform. SIMPLE Antibody™ has a particular strength in addressing novel, complex disease targets that are difficult to access using established antibody technology platforms. Proprietary Fc engineering technologies (NHance® and ABDEG™) and POTELLIGENT® technology (licensed from BioWa, Inc.) further enhance the therapeutic properties of SIMPLE Antibody™ leads in terms of tissue penetration/residence time in the body, ability to clear disease targets or pathogenic antibodies and cell-killing potency through Antibody-Dependent Cell-mediated Cytotoxicity (ADCC), respectively. arGEN-X has leveraged its suite of antibody technologies in forging strategic collaborations with pharmaceutical and biotechnology companies to provide new approaches to diseases with unmet medical needs.

arGEN-X is listed on the Euronext Brussels exchange under the symbol ARGX.

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