

## arGEN-X – Third Quarter Business Update

14 November 2014

**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, today provides a business update for the third quarter 2014 and financial highlights for the nine months ended 30 September 2014.

### RECENT PIPELINE HIGHLIGHTS

- Submitted Investigational New Drug application (IND) to evaluate lead candidate ARGX-110, a novel anti-CD70 therapeutic antibody, in the United States for the treatment of Waldenström's macroglobulinemia (a rare, incurable B-cell lymphoma) in partnership with The Leukemia and Lymphoma Society (LLS)
- Initiated the efficacy evaluation of ARGX-110 in patients with relapsed/refractory CD70-positive T-cell lymphomas as an expansion arm of the ongoing Phase 1b study
- Completed enrolment of first cohort of patients with CD70-positive hematological malignancies into the ongoing Phase 1b expansion trial with ARGX-110
- Completed enrolment of first cohort of patients with CD70-positive solid tumors into the Phase 1b expansion trial with ARGX-110
- Announced positive preclinical data for ARGX-113 supporting its use as a potential breakthrough concept for the treatment of severe autoimmune diseases
- Key patents granted in the US relating to ARGX-110 and ARGX-111, providing patent protection for both until 2031-2032 and allowing for up to an additional five years of patent term extension
- Patent granted reinforcing protection for the SIMPLE Antibody™ platform and antibodies in arGEN-X' pipeline

### FINANCIAL HIGHLIGHTS

- Completed successful Initial Public Offering (IPO) on Euronext Brussels (pricing on 8 July with overallotment option exercised on 11 August) raising total gross proceeds of EUR 41.8 million
- Net loss for the nine-month period to 30 September 2014 was EUR 6.3 million compared to EUR 4.7 million for the same period in 2013
- Cash position as at 30 September 2014 was EUR 59.9 million. Cash position at the end of June, before the receipt of the proceeds from the IPO, was EUR 20.6 million

**Tim Van Hauwermeiren, Chief Executive Officer of arGEN-X, said:** "We are well on track executing our IPO business plan, progressing the product pipeline through a series of clinical value inflection points and leveraging the power of our technology suite in the complex target space. We are encouraged by the highly favorable, cumulative safety profile of ARGX-110. The adaptive design of the Phase 1b study enabled us to select compelling orphan indications for further study including T-cell lymphomas and Waldenström's macroglobulinemia, both characterized by high unmet medical need and lack of treatment options. In addition, we expect to select a safe starting dose for further clinical studies with ARGX-111 before year-end and we are thrilled by the promising preclinical data with ARGX-113 highlighting its potential to become a breakthrough treatment for severe autoimmune diseases based on its unique ability to deplete pathogenic auto-antibodies. With our strong cash position, highly competitive technology suite and diverse product pipeline we are well positioned to create substantial value for our shareholders."

**BUSINESS UPDATE*****Successful Initial Public Offering***

arGEN-X successfully completed its IPO on Euronext Brussels in July raising EUR 40 million in gross proceeds from the sale of 4,705,882 new shares at the offer price of EUR 8.50 per share. Additionally, the partial exercise of the over-allotment option raised a further EUR 1.8 million for the Company from the sale of 208,725 shares at the offer price, taking the total gross proceeds to EUR 41.8 million.

The funds raised through the IPO will enable arGEN-X to advance the development of ARGX-110, ARGX-111 and ARGX-113 through to clinical proof of concept or beyond, and to further enhance its SIMPLE Antibody™ platform on which its pipeline of differentiated therapeutic antibodies has been created. Data from these studies will be leveraged to partner these therapeutic antibodies for development and commercialization across a number of major indications.

***Pipeline Progress*****ARGX-110 – a clinical-stage anti-CD70 therapeutic antibody**

arGEN-X started two Phase 1b expansion cohorts in January 2014 to further investigate the safety of ARGX-110 in CD70-positive cancer patients with either end-stage hematological malignancies (lymphomas, leukemias) or end-stage solid tumors, and to identify potential efficacy signals across these patient populations. Read-out from the study is expected in 2H 2015.

Enrolment of the first cohorts of 15 patients with solid tumors and 15 patients with hematological malignancies into the study was completed during September and October, respectively. Based on demonstrated signs of biological activity in patients with relapsed/refractory T-cell lymphomas in the dose-escalation phase of the study, an efficacy evaluation has been initiated in this indication (see below) as an expansion arm. Selection of an additional indication for an expansion arm among solid tumors is expected in 2015.

***IND submitted for clinical study in Waldenström's macroglobulinemia according to plan***

In November, arGEN-X submitted an IND to evaluate ARGX-110 for the treatment of Waldenström's macroglobulinemia; a rare, incurable B-cell lymphoma in which overactive CD70 signaling has been shown to contribute to tumor growth. Patient enrolment into a Phase 1b/2 study is planned to start in the first quarter of 2015 with efficacy results expected in 2017. The study will be conducted in partnership with The Leukemia and Lymphoma Society (LLS) at two leading cancer centers in the US (Dana-Farber Cancer Institute and Memorial Sloan-Kettering Cancer Center), and under the guidance of Steven P. Treon, MD, PhD, Director of the Bing Center for Waldenström's macroglobulinemia at Harvard Medical School, a leading authority on the disease and its treatment.

***Evaluation of efficacy in T-cell lymphomas***

The observation of clear biological responses and antitumor effects of ARGX-110 in patients with relapsed/refractory CD70-positive T-cell lymphomas, including a complete response in a patient with Sézary syndrome, has prompted arGEN-X to initiate an efficacy evaluation with ARGX-110 in these indications, ahead of schedule. Enrolment of up to 30 patients with relapsed/refractory CD70-positive T-cell lymphomas is planned and the evaluation will be conducted as an expansion arm of the ongoing Phase 1b study.



### **ARGX-111 – a clinical-stage therapeutic antibody targeting c-Met**

The clinical development for ARGX-111, a novel antibody targeting c-Met, a receptor involved in cancer spread (metastasis) in solid tumors, has begun with a Phase 1b trial to characterize the safety profile and biological activity of ARGX-111 in 34 patients with advanced solid tumors over-expressing c-Met.

arGEN-X is on track to complete the dose-escalation part of the trial with preliminary results expected in 2H 2015 and to select a safe starting dose for the expansion phase of the trial during 2H 2014 with patient enrolment expected to begin in 1H 2015.

To date, arGEN-X has observed encouraging signs of biological activity in a gastric cancer patient who remained progression-free for six months.

### **ARGX-113 – a novel pre-clinical antibody fragment that promotes autoantibody degradation**

arGEN-X is developing ARGX-113 as a novel approach to treating severe autoimmune diseases. ARGX-113 is a proprietary antibody fragment based on ABDEG™ technology that targets the neonatal Fc receptor (FcRn), and modulates the process of antibody recycling. ARGX-113 works by preventing pathogenic autoantibodies (i.e. those that target and damage healthy tissues) from being recycled, promoting their degradation and thereby removing, or clearing, them from the circulation.

ARGX-113 is in preclinical development. Results of a recent study assessing the pharmacokinetic and pharmacodynamic (PK/PD) behaviors of ARGX-113 (announced in August) found it to be highly effective in rapidly clearing a tracer antibody from circulation in a dose-dependent manner in non-human primates, thus acting as a surrogate of autoantibody clearance.

arGEN-X expects to submit a Clinical Trial Application (CTA) for ARGX-113 to the applicable regulatory body in 2H 2015, proposing the initiation of a Phase 1 study in healthy volunteers to establish safety and tolerability of the drug.

### **Patent update**

In September 2014, arGEN-X announced the grant of key patents in the US relating to ARGX-110 and ARGX-111, providing patent protection for both until 2031-2032 and allowing for up to an additional five years of patent term extension. The Company also announced the grant of a patent reinforcing protection for the SIMPLE Antibody™ platform and antibodies in arGEN-X' pipeline.

## FINANCIAL REVIEW

<i>in thousands of euros</i>	Nine months ended September 30, 2014	Nine months ended September 30, 2013	Variance
Revenue	2,196	2,047	149
Other operating income	1,398	1,951	(554)
<b>Total operating income</b>	<b>3,594</b>	<b>3,999</b>	<b>(405)</b>
Research and development expenses	(8,023)	(7,297)	(726)
General and administrative expenses	(2,187)	(1,514)	(673)
<b>Operating profit/(loss)</b>	<b>(6,616)</b>	<b>(4,812)</b>	<b>(1,804)</b>
Financial income	93	130	(37)
Exchange gains/(losses)	233	(47)	280
<b>Profit/loss for the period</b>	<b>(6,289)</b>	<b>(4,729)</b>	<b>(1,560)</b>
Net increase (decrease) in cash	36,953	3,506	33,447
Cash at the end of the period	59,907	18,889	41,018

Operating income was EUR 3.6 million for the nine-month period ended 30 September 2014 compared to EUR 4.0 million for the same period in 2013. The lower operating income in 2014 is mainly explained by the decrease of other operating income due to the end in 2014 of a grant received from the Flemish government's Institute for the Promotion of Innovation by Science and Technology in Flanders (IWT).

For the first nine months of 2014, research and development expenses amounted to EUR 8 million, compared to EUR 7.3 million during the first nine months in 2013. The EUR 0.7 million increase in 2014 reflects (i) increased clinical trial and product manufacturing activities, and (ii) the recruitment of additional R&D personnel following the signing of a collaboration agreement with Bayer (May) and a new Strategic Alliance with Shire (June).

General and administrative expenses were EUR 2.2 million and EUR 1.5 million for the nine-month period ended 30 September 2014 and 2013, respectively. The EUR 0.7 million increase in 2014 is principally explained by the costs incurred in relation to IPO preparation.

In the nine-month period ended 30 September 2014, the Group generated a net loss of EUR 6.3 million compared to a net loss of EUR 4.7 million in the same period of 2013.

On 30 September 2014 the Group's cash and cash equivalents amounted to EUR 59.9 million compared to EUR 18.9 million on 30 September 2013.



The Group's increase in net cash of 37.0 million in the first nine months of 2014 is due to the EUR 41.8 million (including the exercise of the over-allotment option) in proceeds received following the successful completion of its IPO on Euronext Brussels on 8 July 2014.

The Group believes that it has sufficient cash to finance its current business plan until the end of 2017.

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#### **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.

[www.arGEN-X.com](http://www.arGEN-X.com)

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