



## argenx Provides Detailed Data from Phase 2 Clinical Trial of Efgartigimod in Immune Thrombocytopenia and Phase 1/2 Clinical Trial of Cusatuzumab in Acute Myeloid Leukemia

- | Detailed Phase 2 ITP data show clear correlation between IgG reduction, platelet count increase and reduction of bleeding events
  - | ITP program expected to advance to Phase 3 with IV efgartigimod and Phase 2 with subcutaneous formulation
    - | New cusatuzumab data in AML resulted in a 92% response rate with 10 of 12 patients showing a complete response (CR/CRi)
  - | Workshop today at 12:00 p.m. PT in conjunction with ASH Annual Meeting

**December 3, 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 that it will share the detailed data from its Phase 2 clinical trial of efgartigimod (ARGX-113) in immune thrombocytopenia (ITP) and the Phase 1 portion of its Phase 1/2 clinical trial of cusatuzumab (ARGX-110) in acute myeloid leukemia (AML) and high-risk myelodysplastic syndromes (MDS) during a workshop being held in conjunction with the 60<sup>th</sup> American Society of Hematology (ASH) Annual Meeting and Exposition.

The workshop is being held on Monday, December 3, 2018 at 12:00 p.m. PT. A [live webcast](#) of the workshop will be available on argenx's website at [www.argenx.com](http://www.argenx.com). A replay of the webcast will be available for 90 days following the presentation.

"These datasets highlight the power of the collaborations we've forged with leading academic institutions. As part of these collaborations, we combine our antibody discovery capabilities with our collaborators' deep disease biology insights to together unravel the functions of novel targets. We have built our broad pipeline in this way and have demonstrated strong execution with each new product candidate we bring forward. Based on the clinically meaningful results and clean tolerability profiles we have observed to date, we believe we have two antibody molecules with efgartigimod and cusatuzumab that are both first-in-class and potentially best-in-class," commented Tim Van Hauwermeiren, Chief Executive Officer of argenx.

"We established strong proof-of-concept with efgartigimod in a second autoimmune indication showing a clear correlation between IgG reductions, platelet count increases and reduced bleeding events. The improvements in platelet counts were clinically meaningful in the treatment arms after a short drug exposure in a truly refractory ITP patient population. With these results and the drug candidate's continued favorable tolerability, we look forward to advancing into a potential pivotal trial next year," commented Nicolas Leupin, Chief Medical Officer of argenx.

### Key ITP Clinical Results

This trial evaluated 38 adult patients with primary ITP who were inadequately controlled on standard of care (platelet count  $\leq 30 \times 10^9/L$  at screening) in a Phase 2 proof-of-concept trial of efgartigimod. Patients received 4 doses over 3 weeks of either 5 mg/kg or 10 mg/kg of intravenous (IV) efgartigimod, or placebo. The Phase 2 trial was amended in December 2017 to extend the patient follow-up period to 21 weeks and to include the option to enter an open-label extension (OLE) trial. Data being presented today are the full data set.

The primary endpoint was safety and tolerability; efgartigimod was well-tolerated in all patients, with most adverse events (AEs) characterized as mild and deemed unrelated to trial drug.

Clinically meaningful improvements in platelet counts were seen across ITP classifications and standard of care and included:

- | 46% of patients improved platelet count to  $\geq 50 \times 10^9/L$  during two or more visits in each of the 5 mg/kg and 10 mg/kg dosing cohorts compared to 25% in the placebo cohort.
- | Updated data show that 67% of patients in the OLE trial improved platelet count to  $\geq 50 \times 10^9/L$  during two or more visits following the first dosing cycle. Responders from the 10 mg/kg arm in the primary trial all responded again upon retreatment in the OLE trial.
- | Onset of platelet count reaching  $50 \times 10^9/L$  for the first time ranged from week 1 to week 10, consistent with disease heterogeneity.
- | For efgartigimod-treated patients with clinically meaningful platelet responses ( $\geq 50 \times 10^9/L$  during two or more visits), the mean duration of platelet response was 40 days versus 16 days for placebo treated patients, with responses lasting the trial duration.
- | 38% of efgartigimod-treated patients showed durable platelet count improvements to clinically meaningful and statistically significant levels of  $\geq 50 \times 10^9/L$  for at least 10 cumulative days, compared to 0% of placebo patients ( $p=0.03$ ).

Lasting IgG reductions consistent with levels achieved in previous studies (updated results) included:

- | All efgartigimod-treated patients showed a rapid and deep reduction of total IgG levels, consistent with the pharmacodynamic effects observed in previous clinical trials. Reduction of IgG levels was consistent across IgG subtypes.
- | Reduction in platelet-associated autoantibodies observed in the majority of patients with clinically meaningful platelet increase.
- | Low titer of anti-drug antibodies was detected in 16.7% of placebo patients and 30.8% of treated patients in the 10 mg/kg arm with no apparent effect on pharmacokinetics or pharmacodynamics.

Bleeding events were assessed using three metrics—adverse event reporting, the WHO scale and the ITP-BAT scale—and showed that efgartigimod reduced bleeding events (updated results) across each scale including:

- 1 AE reporting showed no severe bleeding events in any patient, mild bleeding events only were reported in the 10 mg/kg arm and mild and moderate in the 5 mg/kg and placebo arm.
- 1 Incidence of bleeding events was reduced by efgartigimod treatment as assessed by the WHO bleeding scale, with separation from placebo as early as the third dose in the 10 mg/kg arm.
- 1 Incidence of bleeding events in the skin was reduced by efgartigimod treatment as assessed by the ITP-BAT bleeding scale, with no clear signal of bleeding events in the mucosa or organs in either treatment arm.
- 1 Efgartigimod treatment resulted in clear correlation between IgG reduction, platelet count improvement and bleeding event reduction.

Based on these data, argenx plans to advance efgartigimod (IV) to Phase 3 development in ITP. argenx also expects to initiate a Phase 2 trial in ITP using a subcutaneous formulation of efgartigimod.

### **Key AML Clinical Results**

argenx evaluated 12 newly diagnosed AML patients unfit for intensive chemotherapy in the Phase 1 dose escalation part of the open-label Phase 1/2 clinical trial. Patients received cusatuzumab in combination with Vidaza®. Data being presented today are updated as of the new cut-off date of October 15, 2018.

- 1 Cusatuzumab continued to be well-tolerated in AML patients on all four doses (1 mg/kg, 3 mg/kg, 10 mg/kg, 20 mg/kg).
- 1 The data show an overall response rate (ORR) across the 12 patients of 92% (11/12 patients), including 10 patients (91%) with a complete remission with or without hematologic recovery (CR/CRi) and 1 (9%) partial remission (PR).
- 1 Responses were seen in patients across age and risk category, including IDH2 and TP53 mutations.
- 1 The median duration on trial as of data cut-off was 8.1 months, ranging from 2 to 17.4 months, with 6 patients still on trial.
- 1 Five patients (42%) achieved minimal residual disease (MRD) negativity as measured by flow cytometry and molecular genetics in the bone marrow.
- 1 Translational data demonstrated that cusatuzumab monotherapy and in combination with Vidaza® significantly reduced leukemic stem cells in the bone marrow of AML patients.

argenx is currently enrolling an initial 21 AML patients in the Phase 2 part of its Phase 1/2 clinical trial using the 10 mg/kg dose of cusatuzumab.

“We continue to be excited by the encouraging dataset from our Phase 1/2 trial of cusatuzumab in AML and MDS. This agent targets the CD70/CD27 pathway which has the potential to be a novel and selective mechanism in treating newly diagnosed AML patients regardless of age or cytogenetic profile. Today we are seeing a growing depth of responses from patients on cusatuzumab, with 10 out of 12 patients reaching complete response and 8 of these 10 with hematologic recovery, which patients tolerated well. Six patients remain on trial, and we will watch as these data mature, including the durability of responses,” added Nicolas Leupin, Chief Medical Officer of argenx.

### **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 IgG1 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 Cusatuzumab**

Cusatuzumab (ARGX-110) is an investigational SIMPLE Antibody™ targeting CD70, an immune checkpoint target involved in hematological malignancies, several solid tumors and severe autoimmune diseases. Cusatuzumab is designed to: block CD70, kill cancer cells expressing CD70 through complement dependent cytotoxicity, enhanced antibody-dependent cell-mediated phagocytosis and enhanced antibody-dependent cell-mediated cytotoxicity, and restore immune surveillance against solid tumors (*Silence K. et al. mAbs 2014; 6 (2):523-532*). Cusatuzumab is currently being evaluated in patients with hematological malignancies, including a Phase 1/2 trial in combination with Vidaza® in patients with newly diagnosed acute myeloid leukemia and high-risk myelodysplastic syndromes and the Phase 2 part of a Phase 1/2 trial in patients with relapsed/refractory cutaneous T-cell lymphoma (CTCL). Preclinical work on cusatuzumab in AML was performed in collaboration with the Tumor Immunology Lab of Prof. A. F. Ochsenbein at the University of Bern, who won, together with Prof. Manz at the University Hospital of Zürich, the prestigious 2016 *Otto Naegeli Prize* for his breakthrough research on CD70/CD27 signaling with therapeutic potential for cancer patients.

### **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 its three complementary Fc engineering technologies are designed to expand the therapeutic index of its product candidates.

[www.argenx.com](http://www.argenx.com)

**For further information, please contact:**

Joke Comijn, Director Corporate Communications & Investor Relations (EU)  
+32 (0)477 77 29 44  
+32 (0)9 310 34 19  
info@argenx.com

Beth DelGiacco, VP Investor Relations (US)  
+1 518 424 4980  
bdelgiacco@argenx.com

## **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, data readouts and regulatory milestones and plans, including the timing of planned clinical trials and expected data readouts, related to efgartigimod and cusatuzumab. 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 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.*