



argenx announces publication of efgartigimod Phase 2 primary immune thrombocytopenia trial results in American Journal of Hematology

Efgartigimod was well-tolerated and showed a correlation of reduced IgG levels, increased platelet counts and reduced bleeding in ITP patients

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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 of results from the completed Phase 2 clinical trial of its FcRn antagonist, efgartigimod, in adult patients with primary immune thrombocytopenia (ITP) in the *American Journal of Hematology*. The peer-reviewed article is titled, “Phase 2 study of efgartigimod, a novel FcRn antagonist, in adult patients with primary immune thrombocytopenia,” and can be accessed [here](#).

“We are pleased to have this publication of the full Phase 2 ITP trial results available to the hematology community, highlighting the novel approach of efgartigimod as a new treatment modality in ITP. Patients continue to need additional therapeutic options for this difficult-to-treat disease. Efgartigimod has demonstrated a favorable tolerability profile and the ability to drive encouraging response rates in a refractory population after a limited exposure. We now look forward to advancing efgartigimod into a global Phase 3 program in ITP before the end of 2019 where we have the opportunity to dose in a longer-term setting,” said Wim Parys, M.D., chief medical officer of argenx.

The Phase 3 program of efgartigimod in ITP remains on track to initiate this month and will evaluate the intravenous (IV) formulation in the first clinical trial, ADVANCE, and an IV induction followed by a subcutaneous (SC) maintenance injection in the second clinical trial, ADVANCE SC.

In addition to ITP, efgartigimod is currently being evaluated as a treatment for other severe autoimmune diseases, including generalized myasthenia gravis (gMG), pemphigus vulgaris (PV), and chronic inflammatory demyelinating polyneuropathy (CIDP). The Phase 3 ADAPT trial of IV efgartigimod is ongoing in gMG with data expected in the second half of 2020. A Phase 2 proof-of-concept trial of IV efgartigimod is ongoing in PV and data are expected in the first half of 2020. A Phase 2 proof-of-concept trial of SC efgartigimod in CIDP is expected to start before the end of 2019.

About efgartigimod

Efgartigimod is an IgG Fc fragment engineered to optimally antagonize the neonatal Fc Receptor (FcRn) for the treatment of IgG-mediated autoimmune diseases. FcRn plays a central role in rescuing IgG from degradation in the lysosome through a recycling pathway. Through inhibition of FcRn, efgartigimod leads to fast depletion of the disease-causing IgG autoantibodies. Efgartigimod binds in the same way as endogenous IgG, the natural ligand of FcRn, and has been engineered with ABDEG™ mutations to increase its affinity for FcRn while preserving the characteristic pH-dependent binding, contributing to its long serum half-life, pharmacodynamic effect and potentially enhanced tissue penetration. The development work on efgartigimod is conducted in close collaboration with Prof. E. Sally Ward (Department of Molecular and Cellular Medicine, Texas A&M University Health Science Center, College Station, TX; Center for Cancer Immunology, University of Southampton, Southampton, UK).

About argenx

argenx is a clinical-stage biotechnology company developing a deep pipeline of differentiated antibody-based therapies for the treatment of severe autoimmune 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.

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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.