ABLYNX’S ANTI-vWF NANOBODY, CAPLACIZUMAB, ACHIEVES CLINICAL PROOF-OF-CONCEPT IN PHASE II TITAN STUDY

- First-in-class potential with orphan drug status for the treatment of acquired thrombotic thrombocytopenic purpura
- Proof-of-concept with statistically significant 39% decrease in time to normalisation of platelet count compared to placebo for patients who received no plasma exchange prior to initial treatment with caplacizumab
- The group of patients treated with caplacizumab in conjunction with the standard of care achieved confirmed platelet normalisation at more than twice the rate of the group receiving the standard of care plus placebo during the 30-day period after the start of study drug. The p-value was 0.013
- 81% of patients achieved complete remission compared to 46% in the placebo arm
- 73% less exacerbations compared to placebo during treatment with caplacizumab
- Generally well-tolerated, with manageable increase in bleeding tendency

Results will be discussed during a webcast presentation today at 17h CET, 11 am EST
The webcast may be accessed on the home page of the Ablynx website at www.ablynx.com
or by clicking here
To participate in the Q&A, dial +32(0)2 400 19 72 with confirmation code 5793077.

GHENT, Belgium, 17 June 2014 – Ablynx [Euronext Brussels: ABLX] today announced that it has achieved positive results in the Phase II TITAN study with the anti-vWF Nanobody®, caplacizumab, in patients with acquired thrombotic thrombocytopenic purpura (TTP), a rare disorder of the blood coagulation system that causes microthrombi to form which can block small blood vessels throughout the body. Treatment with caplacizumab plus standard of care resulted in a statistically significant reduction in time to confirmed normalisation and was associated with fewer exacerbations and more complete remissions as compared to patients receiving the standard of care plus placebo.

The worldwide Phase II TITAN clinical trial was a single-blinded, randomised, placebo-controlled study which recruited from January 2011 to January 2014. In total, 75 patients were randomized on a 1:1 basis with one active drug treatment arm and one placebo arm. All patients received the current standard of care which is primarily multiple plasma exchanges. The protocol for the study was adapted in September 2013, to also allow one day of plasma exchange prior to study enrolment. Those patients in the active drug treatment arm immediately received an intravenous bolus dose of 10 mg caplacizumab and then a 10 mg subcutaneous dose of the drug daily until 30 days had elapsed after the final plasma exchange. Patients in the control arm received placebo at the same time points.

The primary endpoint of the trial was the time to confirmed platelet normalisation which guides the clinical decision to stop the daily plasma exchanges. The results from this study showed that the group
of patients treated with caplacizumab, in conjunction with plasma exchange, achieved confirmed platelet normalisation at more than twice the rate of the group receiving the standard of care plus placebo at any time during the 30-day period after start of study drug, as demonstrated by the hazard ratio of 2.2 (p = 0.013, 95% confidence interval [1.28-3.78]); where the “hazard” is the rate at which an event occurs, with the event here being confirmed platelet normalisation. Time to confirmed platelet normalisation for patients who had not received a plasma exchange prior to initial dosing with caplacizumab was a median of 3.0 days in the caplacizumab arm compared to a median of 4.9 days in the placebo arm – a potentially clinically meaningful 39% decrease.

The potential protective effect of caplacizumab in the treatment of TTP was also shown by the 73% fewer patients who experienced an exacerbation in the active treatment arm compared to the control group, with 3 (8%) of patients treated with caplacizumab experiencing an exacerbation compared to 11 (28%) treated with placebo. An exacerbation is defined as the recurrence of thrombocytopenia (low platelet count) requiring re-initiation of plasma exchange treatment within 30 days after stopping the initial daily plasma exchanges. Importantly, 81% of caplacizumab treated patients achieved complete remission compared to 46% of placebo treated patients, where “complete remission” is defined as confirmed platelet normalisation together with an absence of exacerbations.

TTP is a serious and potentially life-threatening disease. There were two deaths in the trial, both in the placebo arm. The number of patients with a treatment-emergent serious adverse event was similar across both treatment arms, with 20 (57%) patients in the caplacizumab arm compared to 19 (51%) patients in the placebo arm. The number of treatment-emergent adverse events was also similar across both arms of the study. Study treatment was stopped due to a treatment-emergent adverse event in four patients treated with caplacizumab and in two patients treated with placebo. An increased bleeding tendency was observed for caplacizumab compared to placebo, with 66 events compared to 35 respectively (11% and 6% of all reported treatment-emergent adverse events per treatment arm respectively). Of these treatment-emergent adverse events, five were serious adverse events in two subjects treated with caplacizumab compared to two serious adverse events in two subjects on placebo.

Based on the top-line data at this time, caplacizumab can be considered to be generally well tolerated compared to placebo, with a manageable increase in bleeding tendency.

Dr Flora Peyvandi, Principal Investigator for the TITAN study at IRCCS Maggiore Hospital Foundation, University of Milan, Italy, commented: “The outcome of this study makes us believe that caplacizumab has the potential to become an important pillar in the management of acquired TTP. The results show that caplacizumab is able to control the acute phase of the disease and buys time for the later onset of the immunosuppressive treatment which is often needed to fully resolve a TTP episode.”

Dr Dominique Tersago, Chief Medical Officer of Ablynx said: “The potential of caplacizumab to act as a protective agent for patients with acquired TTP has been demonstrated in this trial. Further and more detailed analyses of the study results are still ongoing and we look forward to working closely with clinical experts and regulatory authorities to ensure the design of an optimal Phase III trial which we plan to start in 2015.”

Dr Edwin Moses, Chief Executive Officer of Ablynx added: “We are very pleased with the results of the study and the potential of our first-in-class anti-vWF Nanobody. It is our third Nanobody programme to achieve clinical proof-of-concept in the space of just three years, which we believe demonstrates the exceptional ability of our platform to deliver clinically meaningful assets. We will proceed with preparations for the start of a Phase III study in 2015 and in parallel we will be talking to potential partners and evaluating all of our strategic options for the further development and commercialisation of caplacizumab.”
About caplacizumab

Caplacizumab is a bivalent anti-vWF Nanobody which is highly potent and selective. It received Orphan Drug Designation in the US and EU in 2009 and could be the first drug specifically approved for the treatment of acquired TTP as an adjunct to plasma exchange.

Von Willebrand factor (vWF) is a blood glycoprotein involved in haemostasis, a complex process that causes the bleeding process to stop. vWF’s primary function is to bind to other proteins, including glycoprotein Ibb in the initiation of platelet adhesion. vWF is implicated in TTP where ultra-large, multimeric precursors of vWF (UL-vWF) are present in the blood of patients leading to unwanted blood clot formation. UL-vWF can readily bind platelets leading to the formation of characteristic string-like clots in small blood vessels.

Caplacizumab inhibits platelet binding to UL-vWF and thus has the potential to prevent the formation of these string-like clots in the blood of patients with acquired TTP.

About thrombotic thrombocytopenic purpura (TTP)

TTP is a rare disorder of the blood coagulation system that causes extensive microscopic thromboses in small blood vessels throughout the body. It is a potentially life-threatening disorder characterised by thrombocytopenia, haemolytic anaemia and microvascular thrombosis causing variable degrees of tissue ischemia and infarction. TTP exists in two forms: a congenital and an acquired form, with the latter accounting for >90% of the patients. There are currently no drugs specifically approved for the treatment of TTP. The standard of care for the acquired form of TTP is multiple daily plasma exchanges until confirmed platelet normalisation which occurs when the patient’s platelet count returns to normal. This treatment requires lengthy hospital stays and may be associated with clinical complications. Additionally, a significant number of patients will subsequently suffer a relapse after recovering from a first TTP episode. There are believed to be approximately 10,000 TTP-related events in the US and top 15 European markets per year.

About Ablynx

Ablynx is a biopharmaceutical company engaged in the discovery and development of Nanobodies®, a novel class of therapeutic proteins based on single-domain antibody fragments, for a range of serious human diseases, including inflammation, haematology, oncology and pulmonary disease. Today, the Company has more than 30 programmes in the pipeline and seven Nanobodies in clinical development. Ablynx has on-going research collaborations and significant partnerships with major pharmaceutical companies including AbbVie, Boehringer Ingelheim, Merck & Co, Merck Serono and Novartis. The Company is headquartered in Ghent, Belgium. More information can be found on www.ablynx.com.

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