RESULTS FROM THE PHASE III HERCULES STUDY OF CAPLACIZUMAB FOR THE TREATMENT OF ACQUIRED TTP SELECTED FOR PRESENTATION IN THE LATE-BREAKING ABSTRACTS SESSION AT THE 2017 ASH ANNUAL MEETING

GHENT, Belgium, 21 November 2017 (3.30pm CET) – Ablynx NV [Euronext Brussels and Nasdaq: ABLX] today announced that results from its Phase III HERCULES study of caplacizumab have been selected as one of only six abstracts for oral presentation in the late-breaking abstracts session at the 59th Annual Meeting of the American Society of Hematology (ASH) taking place in Atlanta, GA, on 12 December 2017.

Caplacizumab is Ablynx's wholly-owned anti-von Willebrand factor (vWF) Nanobody® being developed for the treatment of acquired thrombotic thrombocytopenic purpura (aTTP). Positive topline results from the Phase III HERCULES study, meeting primary and two key secondary endpoints, were announced on 2 October 2017.

The abstract (LBA-1), "Results of the Randomized, Double-Blind, Placebo-Controlled, Phase III HERCULES study of Caplacizumab in Patients with Acquired Thrombotic Thrombocytopenic Purpura", will be presented by Professor Marie Scully, M.D., Department of Haematology, University College London Hospitals NHS Trust, London, UK.

The abstract is available at https://ash.confex.com/ash/2017/webprogram/Paper109057.html and will be included in the 8 December online issue of *Blood*.

Commenting on today's announcement, Dr Edwin Moses, CEO of Ablynx, said: "We are very pleased that our positive Phase III HERCULES data have been accepted for this presentation at the world's leading hematology conference, the 2017 ASH Annual Meeting in Atlanta, USA. The data confirm the significant potential of caplacizumab for patients with aTTP for whom there is currently no approved therapeutic drug available."

Session Information

Name: Late-Breaking Abstracts Session
Date: Tuesday 12 December 2017
Time: 7:30 AM - 9:00 AM ET
Presentation Time: 7:30 AM ET
Room: Building C, Level 1, Hall C2-C3 (Georgia World Congress Center)

About HERCULES

The HERCULES study recruited 145 patients and is the largest randomised, double-blind, placebo-controlled study conducted in patients with aTTP. Patients with an acute episode of aTTP were randomised 1:1 to receive either caplacizumab or placebo in addition to daily plasma exchange (PEX) and immunosuppression. Patients received a single intravenous bolus of 10mg caplacizumab or placebo followed by a daily subcutaneous dose of 10mg caplacizumab or placebo for 30 days after the last daily PEX. If at the end of

this treatment period there was evidence of persistent underlying disease activity (indicative of an imminent risk for recurrence), treatment could be extended for additional seven-day periods up to a maximum of 28 days and was to be accompanied by optimisation of immunosuppression. Patients were followed for a further 28 days after discontinuation of treatment.

A three-year follow-up study (NCT02878603) of patients who have completed the HERCULES study is in progress and will further evaluate the long-term safety and efficacy of caplacizumab and repeated use of caplacizumab, as well as characterising the long-term impact of aTTP.

About caplacizumab

Caplacizumab is a bivalent anti-vWF Nanobody that received Orphan Drug Designation in Europe and the United States in 2009. Caplacizumab blocks the interaction of ultra-large vWF multimers (ULvWF) with platelets and, therefore, has an immediate effect on platelet aggregation and the ensuing formation and accumulation of the micro-clots that cause the severe thrombocytopenia, tissue ischemia and organ dysfunction in aTTP. This immediate effect of caplacizumab has the potential to protect the patient from the manifestations of the disease while the underlying disease process resolves.

In February 2017, based on the Phase II study results, a Marketing Authorisation Application (MAA) was submitted to the European Medicines Agency (EMA) for approval of caplacizumab in aTTP. In July 2017, Ablynx received Fast Track designation from the Food and Drug Administration (FDA) for caplacizumab for the treatment of aTTP. In October 2017, positive results from the Phase III HERCULES study, meeting primary and two key secondary endpoints, were announced. These data are expected to further support the MAA, as well as a planned Biologics License Application (BLA) filing in the United States in 2018. If approved by regulatory authorities, caplacizumab would be the first therapeutic specifically indicated for the treatment of aTTP.

About aTTP

aTTP is a rare, acute, life-threatening, autoimmune blood clotting disorder. It is caused by impaired activity of the ADAMTS13 enzyme, leaving ULvWF molecules uncleaved (vWF is an important protein involved in the blood clotting process). These ULvWF molecules spontaneously bind to blood platelets, resulting in severe thrombocytopenia (very low platelet count) and clot formation in small blood vessels throughout the body[1], leading to ischemia and widespread organ damage[2].

Despite the current standard-of-care treatment consisting of PEX and immunosuppression, episodes of aTTP are still associated with a mortality rate of up to 20%, with most deaths occurring within 30 days of diagnosis[3]. Furthermore, patients are at risk of acute thromboembolic complications (e.g. stroke, myocardial infarction) and of recurrence of disease. Some patients are refractory to therapy[1], which is associated with a poor prognosis for survival of an acute episode of aTTP. Long term, patients are at increased risk for hypertension, major depression, and premature death[4].

About Ablynx

Ablynx is a biopharmaceutical company engaged in the development of Nanobodies, proprietary therapeutic proteins based on single-domain antibody fragments, which combine the advantages of

[1] Veyradier, NEJM 2016: "von Willebrand Factor – A new target for TTP treatment?"
[2] Scully *et al.*, Br J Hem 2012; Sarode *et al.*, J Clin Apher 2014; Chaturvedi *et al.*, Am J Hem 2013
[3] Benhamou, Y. *et al.*, Haematologica 2012
[4] Deford *et al.*, Blood 2013

conventional antibody drugs with some of the features of small-molecule drugs. Ablynx is dedicated to creating new medicines which will make a real difference to society. Today, the Company has more than 45 proprietary and partnered programmes in development in various therapeutic areas including inflammation, haematology, immuno-oncology, oncology and respiratory disease. The Company has collaborations with multiple pharmaceutical companies including AbbVie; Boehringer Ingelheim; Eddingpharm; Merck & Co., Inc., Kenilworth, New Jersey, USA; Merck KGaA; Novartis; Novo Nordisk; Sanofi and Taisho Pharmaceuticals. The Company is headquartered in Ghent, Belgium. More information can be found on www.ablynx.com.

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