ABLYNX ANNOUNCES POSITIVE DATA FROM ITS JAPANESE ETHNO-BRIDGING STUDY OF CAPLACIZUMAB

- **Comparable pharmacokinetics (PK) observed in Japanese and Caucasian healthy volunteers**
- **Caplacizumab was well-tolerated in all groups**

GHENT, Belgium, 21 December 2017 – Ablynx NV [Euronext Brussels and Nasdaq: ABLX] today announced that the single and multiple dose Phase I study demonstrated comparable PK of caplacizumab in Japanese and Caucasian subjects. Caplacizumab is the Company's wholly-owned anti-von Willebrand factor (vWF) Nanobody® being developed for the treatment of acquired thrombotic thrombocytopenic purpura (aTTP).

The Phase I single centre study enrolled 60 healthy Japanese and Caucasian subjects and consisted of single ascending dose and multiple dose parts. At all doses studied, the PK of caplacizumab in the Japanese population were similar to those observed in Caucasians. Caplacizumab was well-tolerated in all groups and its safety profile was consistent with its mechanism of action.

Dr Robert K. Zeldin, Chief Medical Officer at Ablynx, commented:
"We are very pleased with these results as they enable us to bridge available clinical data for caplacizumab between Japanese and Caucasian populations. This is a very important step in making caplacizumab available to Japanese patients suffering from aTTP. We look forward to discussing these results, together with the recent Phase III HERCULES study results of caplacizumab, with the Japanese Regulatory Agency and aligning on a path forward for regulatory submission in Japan."

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.

The efficacy and safety of caplacizumab in addition to daily PEX and immunosuppression were demonstrated in the Phase II TITAN and Phase III HERCULES studies. In the HERCULES study, treatment with caplacizumab in addition to standard-of-care resulted in a significantly shorter time to platelet count response ($p<0.01$), a significant reduction in aTTP-related death, recurrence of aTTP, or at least one major thromboembolic event during study drug treatment ($p<0.0001$), and a significantly lower number of aTTP recurrences in the overall study period ($p<0.001$). Importantly, treatment with caplacizumab resulted in a clinically meaningful reduction in the use of PEX and length of stay in the ICU and the hospital, compared to the placebo group. In addition, caplacizumab has the potential to prevent refractory disease and have a positive impact on the normalisation of organ damage markers (lactate dehydrogenase, cardiac troponin I and serum creatinine). Caplacizumab has a favourable safety profile, consistent with its mechanism of action. No deaths were reported during the study drug treatment in

the caplacizumab group in both the TITAN and HERCULES studies, while for the placebo group, two deaths were reported in the TITAN study and three deaths in the HERCULES study.

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.

In February 2017, based on the Phase II TITAN 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. The positive results from the Phase III HERCULES study 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 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.

[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

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