



ABLYNX ESTABLISHES SUBSIDIARY IN THE USA AND APPOINTS A GENERAL MANAGER

GHENT, Belgium, 16 October 2017 - **Ablynx [Euronext Brussels: ABLX; OTC: ABYLY]** today announced the establishment of Ablynx, Inc., its subsidiary in the USA, and the appointment of Mr Daniel Schneider as the General Manager to lead the commercialisation of caplacizumab in North America. Mr Daniel (Dan) Schneider will be based in a US office, to be located on the East Coast. Caplacizumab is the Company's wholly-owned anti-von Willebrand factor (vWF) Nanobody® being developed for the treatment of acquired thrombotic thrombocytopenic purpura (aTTP).

Dan Schneider has 25 years of experience in establishing and leading the commercial operations for a number of companies in the life sciences industry and has been deeply involved in the successful launch of many pharmaceutical products, including those for orphan indications. Until recently, Dan was the General Manager of the Specialty Pharmaceuticals Business Unit at BTG International Inc. Previously, he held senior commercial roles at a number of life science companies where he developed the commercial strategy and led the sales efforts across all sectors of the business. Dan holds a BSBA from Saint Louis University and an MBA from Washington University in St. Louis.

Dr Edwin Moses, CEO of Ablynx, commented: "The establishment of Ablynx, Inc. is an important milestone for the Company and confirms our commitment to becoming a fully integrated international biopharmaceutical company. We are very pleased that Dan is joining us. He brings many years of experience in setting up commercial organisations and leading multiple successful product launches in the USA. We look forward to joining forces to further develop our commercial infrastructure in preparation of the potential launch of caplacizumab."

Commenting on his appointment, Mr Schneider added: "I am delighted to join Ablynx at this very important moment as the Company prepares for the potential launch of its first product. I look forward to building and leading the commercial activities in North America and contributing to the growth of the Company."

About caplacizumab

Caplacizumab is a bivalent anti-vWF Nanobody that received Orphan Drug Designation for aTTP 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 standard-of-care were evaluated in the Phase II TITAN study (N=75)¹ and the Phase III HERCULES study (N=145)² in patients with aTTP. In both studies, treatment with caplacizumab was well-tolerated and the primary endpoint was met resulting in a statistically significant reduction in time to platelet count response ($p<0.01$), a measure of prevention of further microvascular thrombosis. The Phase III HERCULES study further demonstrated that treatment with caplacizumab resulted

¹ Press release [June 2014](#); [Manuscript](#) in the NEJM, Feb 2016; [Manuscript](#) in the JTH, Apr 2017

² Press release [October 2017](#)

in a 74% reduction in the percentage of patients with aTTP-related death, recurrence of aTTP, or at least one major thromboembolic event during study drug treatment ($p<0.0001$). In addition, the proportion of patients with a recurrence of aTTP in the overall study period (including the 28 day follow-up after discontinuation of study drug treatment) was 67% lower in the caplacizumab arm compared to the placebo arm ($p<0.001$), demonstrating the durability of the treatment effect. No patients treated with caplacizumab were refractory to treatment compared to three patients treated with placebo ($p=0.057$). There was also a trend to faster normalisation of the organ damage markers (lactate dehydrogenase, cardiac troponin I and serum creatinine) in patients treated with caplacizumab. The safety profile of caplacizumab was consistent with its mechanism of action. There were three deaths in the placebo group and none in the caplacizumab group during the study drug treatment period. One patient in the caplacizumab group died in the follow-up period after completing the study drug treatment and this was assessed by the investigator not to be related to study drug.

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 aTTP³. In July 2017, Ablynx received Fast Track designation from the Food and Drug Administration (FDA) for caplacizumab for the treatment of aTTP⁴. 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 body⁵, leading to ischemia and widespread organ damage⁶.

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 diagnosis⁷. 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, 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 death⁸.

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.,

³ Press release [February 2017](#)

⁴ Press release [July 2017](#)

⁵ Veyradier, NEJM 2016: "von Willebrand Factor – A new target for TTP treatment?"

⁶ Scully *et al.*, Br J Hem 2012; Sarode *et al.*, J Clin Apher 2014; Chaturvedi *et al.*, Am J Hem 2013

⁷ Benhamou, Y. *et al.*, Haematologica 2012

⁸ Deford *et al.*, Blood 2013

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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