Tyrogenex Announces Publication of Phase 1 Data for X-82 for the Oral Treatment of Neovascular Age-Related Macular Degeneration in the Journal of the American Medical Association

Rockville, MD, June 8, 2017 – Tyrogenex, a privately held biopharmaceutical company, today announced that the Journal of the American Medical Association (JAMA Ophthalmology) has published the results from its Phase 1 dose-escalation study of the company’s lead product candidate, X-82 (vorolanib). The research publication, “Oral Tyrosine Kinase Inhibitor for Neovascular Age-Related Macular Degeneration,” led by Timothy L. Jackson, PhD, FRCPht, consultant ophthalmic surgeon of King’s College Hospital and Reader in Retinal Research at King’s College London, presents data evaluating the safety and preliminary efficacy of orally administered X-82 for the treatment of neovascular age-related macular degeneration (AMD). These results support the further clinical development of X-82 as a novel oral treatment for neovascular AMD, and follows the recent announcement by Tyrogenex of completed patient enrollment in the company’s Phase 2 randomized, clinical APEX study of X-82 in wet AMD.

The Phase 1 trial (ClinicalTrials.gov #NCT02348359) was a randomized, double-masked, placebo-controlled study assessing the safety and preliminary efficacy in 35 patients with active neovascular AMD. Patients received oral X-82 for 24 weeks in doses of either 50 mg on alternate days, 50 mg daily, 100 mg on alternate days, 100 mg daily, 200 mg daily, or 300 mg daily concomitantly with intravitreous anti-VEGF therapy utilized on a PRN basis using predefined retreatment criteria. Of the 25 participants who completed the 24 weeks of X-82 treatment, 60% required no anti-VEGF injections.

As reported in the publication, participants underwent best-corrected visual acuity measurement, fundus examination, and spectral-domain optical coherence tomography every four weeks. The primary outcome measures were the number and percentage of adverse events in the treatment period. The most common adverse events attributed to X-82 were diarrhea (n=6), nausea (n=5), fatigue (n=5) and transaminase elevation (n=4). A dose relationship to the transaminase elevations was not identified, and all levels normalized when X-82 was discontinued. There were no reported SAEs attributed to X-82.

Dr. Jackson noted, “Whilst this is primarily a safety study, these results strongly suggest X-82 has biological activity, as the frequency of intravitreal anti-VEGF injections was far lower than expected. Subject to confirmatory studies, the possibility of a pill to treat wet AMD is undoubtedly exciting, as the current treatment with repeated eye injections imposes a substantial burden on patients, and healthcare providers.”

About X-82
X-82 is an investigational orally administered inhibitor of the vascular endothelial growth factor receptor (VEGFR) and platelet derived growth factor receptor (PDGFR). The binding of X-82 to these receptors may lead to efficacy in angiogenic diseases such as wet age-related macular degeneration (wAMD). X-82 is being evaluated in a Phase 2 clinical trial (APEX Study) for use in combination with anti-VEGF drugs that represent the current standard of care for the treatment of wAMD. The Company has completed one Phase 1 trial in patients previously treated with Lucentis® (ranibizumab), Eylea®
(aflibercept) and Avastin® (bevacizumab) and in naïve patients. The Company is also investigating the potential of X-82 for oncology indications.

About Tyrogenex
Tyrogenex is a biopharmaceutical company focused on improving the lives of patients with wet Age-related Macular Degeneration and solid tumors by discovering medicines to help provide additional treatment options. Tyrogenex’s lead compound is X-82. For more information, visit

Forward-Looking Statements
This press release contains forward-looking statements about us and our industry that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this press release are forward-looking statements. Tyrogenex has based these forward-looking statements on Tyrogenex’s management’s current beliefs, expectations and projections about future events and trends. These forward-looking statements are subject to known and unknown risks, uncertainties, and assumptions. You should not rely on forward-looking statements as predictions of future events. Tyrogenex’s statements should not be read to indicate that Tyrogenex has conducted an exhaustive inquiry into, or review of, all relevant information. These statements are inherently uncertain, and investors and stockholders are cautioned not to unduly rely on these statements.

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