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Veralox Therapeutics Announces EMA Orphan Drug Designation for VLX-1005

- Receives New Designation for Treatment of Platelet-Activating Anti-Platelet Factor 4 (PF4) Disorders
- Complements FDA Orphan Drug Designation and Fast Track Designation for VLX-1005

FREDERICK, Md., Aug. 14, 2024 (GLOBE NEWSWIRE) -- Veralox Therapeutics, a clinical-stage biotechnology company developing first-in-class therapies targeting the 12-lipoxygenase (12-LOX) pathway, announced that the European Medicines Agency (EMA) has granted Orphan Drug Designation (ODD) to VLX-1005, a small molecule 12-LOX inhibitor for the treatment of platelet-activating anti-platelet factor 4 (PF4) disorders. VLX-1005 previously secured ODD from the U.S. Food and Drug Administration for prophylaxis of thrombosis in patients with heparin-induced thrombocytopenia (HIT) as well as FDA Fast Track Designation. This latest decision in Europe represents another regulatory achievement and builds on the successful development of VLX-1005 that has progressed through completed Phase 1 clinical studies, and into the ongoing Phase 2 trial ALATHEA (“A study of VLX-1005 to evaluate thrombocyte change in HEpArin-induced thrombocytopenia”).

“This EMA designation is an important step forward in the development of VLX-1005 in addressing serious diseases caused by platelet-activating anti-PF4 disorders such as HIT,” said Michael Hanna, M.D., Chief Medical Officer of Veralox Therapeutics. “This is a significant regulatory milestone for Veralox and is an important acknowledgement of the potential of VLX-1005 as treatment for HIT.”

HIT is a serious, potentially life-threatening platelet disorder that can arise after taking the blood thinner heparin, which by American Heart Association and European Society of Cardiology guidelines is the standard-of-care anticoagulant for many cardiovascular procedures. Heparin is commonly used to prevent blood clots following heart surgeries, cardiopulmonary bypass and many other procedures such as kidney dialysis. In rare cases, heparin triggers a reaction that activates platelets, consuming them in a process that lowers platelet counts (thrombocytopenia) to unsafe levels while promoting clot formation, which then increases the risk for potentially fatal or life-threatening thromboembolic events. Between one quarter and one third of those who develop HIT die from the condition and a significant proportion of patients develop new blood clots, which could result in deep vein thrombosis, pulmonary embolism, heart attack or stroke.

VLX-1005 has successfully completed Phase 1 clinical trials and has demonstrated a favorable safety and tolerability profile and preferred pharmacokinetics in healthy participants and is currently enrolling patients in the Phase 2 clinical study.

Orphan Drug Designation

ODD is a designation that provides incentives to advance the development of therapeutics for rare diseases, including reduced fees for multiple steps in the development process such as protocol assistance, marketing authorization applications, and 10 years of market exclusivity. In addition to the above-mentioned benefits within the European Union, member states may also offer specific stimuli for the development of orphan drugs.

Heparin-Induced Thrombocytopenia

HIT is a rare but serious complication of heparin exposure during heart, orthopedic, vascular and other surgeries and during kidney dialysis, where heparin is commonly used to prevent blood clotting. Due to the high utilization of heparin, there are more than 300,000 suspected cases of HIT each year in the United States. Approximately 50,000 cases are confirmed each year and these patients have a high risk of developing severe consequences including death (25-30 percent); amputation (10 percent); new thrombosis including deep vein thrombosis (DVT), stroke, myocardial (MI) infarction (20 percent) and major bleeding (20 percent). The diagnosis and management of HIT is complex and a large unmet need remains for new therapies due to the limited available therapeutic options.

About VLX-1005

VLX-1005 is a novel, small molecule inhibitor of 12-lipoxygenase (12-LOX) intended for the treatment or prevention of thrombosis in adults with heparin-induced thrombocytopenia (HIT). A potent and selective inhibitor of 12-LOX, VLX-1005 inhibits platelet 12-HETE production. *In vivo* inhibition of 12-HETE synthesis and efficacy of VLX-1005 have been shown in animal models of thrombosis and HIT and the efficacy in preventing or treating thrombosis was not accompanied by increases in bleeding. Two Phase 1 studies completed in 96 healthy participants showed favorable safety and tolerability with no deaths, no serious adverse events and no trend in AE reporting with increased dosing of VLX-1005. A Phase 2 study (VLX-1005-003) to evaluate VLX-1005 in patients with suspected HIT is currently enrolling patients across 12 clinical sites. Additional information is available at clinicaltrials.gov, identifier NCT05785819.

Veralox Therapeutics

Veralox Therapeutics Inc. is developing first-in-class therapeutics targeting 12-lipoxygenase, pioneering a new class of therapies that treat the underlying pathologies of serious immune-inflammatory diseases. The company's lead candidate, VLX-1005, is in development for the treatment of patients with heparin-induced thrombocytopenia. VLX-1005 has previously been awarded Orphan Drug and Fast Track Designations by the U.S. Food and Drug Administration. The company has second-generation orally bioavailable 12-LOX antagonists under development for type 1 diabetes and other immune-mediated and inflammatory diseases.

For more information, visit our website: https://veralox.com/.

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