Vtesse, Inc. Announces Phase 1/2 Clinical Data Showing Slowing of Disease Progression from VTS-270 Treatment for Niemann-Pick Type C1 Disease

GAITHERSBURG, Md., March 4, 2016 /PRNewswire/ -- Vtesse, Inc. announced today data from a Phase 1/2 clinical trial demonstrating a reduction in the rate of Niemann-Pick Type C1 (NPC1) Disease progression from Vtesse's lead drug candidate VTS-270. The *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD) and the National Center for Advancing Translational Sciences, two components of the National Institutes of Health, collaborated closely with patients, patient advocacy groups and academia to initiate the drug development phase for VTS-270. Vtesse is leading the late-stage formal drug development process.

The data were presented at the 2016 World Symposium on Lysosomal Storage Disease in San Diego, California.

The announcement comes as Vtesse is in the midst of conducting its pivotal Phase 2b/3 clinical trial of VTS-270 for treatment of NPC, a progressive, irreversible, chronically debilitating – and ultimately lethal – genetic disease. Vtesse expects to enroll a total of 51 patients at approximately 20 sites (across the United States, the European Union, and other countries) in its ongoing clinical trial.

"We are encouraged by this preliminary Phase 1/2 data, showing a clear effect on severity of symptoms from Niemann-Pick Type C1 disease," said Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc. "We believe this shows that VTS-270 has the potential to substantially slow down disease progression for NPC1, which supports our continuing clinical study in the hope that we can apply for regulatory approval of VTS-270 as a treatment for the patients in need."

The Phase 1/2 clinical data from 14 patients with Niemann-Pick Type C1 showed that after 12 months and 18 months of treatment with VTS-270, disease progression as measured by the NPC

Neurological Severity Score (or NSS, which looks at, among others, ambulation, fine motor ability, cognition, speech, memory and swallowing) was significantly reduced compared to data from a control group who did not receive VTS-270.

"We are highly encouraged by these findings," said Forbes D. Porter, M.D., Ph.D., Clinical Director of the NICHD. "We also are grateful to the families who are participating in the Phase 1 study and those enrolling in the Phase 2b/3 clinical trial."

In January, the U.S. Food and Drug Administration (FDA) granted VTS-270 Breakthrough Therapy designation status. The Breakthrough Therapy designation is granted to help accelerate development and review of drug candidates when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. Both the FDA and the European Medicines Agency (EMA) had previously granted Orphan Drug status to VTS-270.

About NPC

NPC is a progressive, irreversible, chronically debilitating – and ultimately lethal – genetic disease. It is caused by a defect in lipid transportation within the cell, which leads to excessive accumulation of lipids in the brain, liver and spleen. VTS-270 has been shown to significantly reduce disease progression in animal studies and to offer preliminary indications of efficacy in the Phase 1/2 study clinical trial. For more information on Vtesse's pivotal Phase 2b/3 clinical trial, visit www.theNPCstudy.com.

About Vtesse

Vtesse, Inc. is a rare disease company dedicated to developing drugs for patients suffering from diseases that are underserved. Vtesse is working collaboratively with the NIH and other leading academic centers to advance a pivotal clinical study of VTS-270 (a proprietary formulation of 2-hydroxypropyl-beta-cyclodextrin) to treat NPC, and to conduct pre-clinical discovery and development of other novel drugs for NPC and other lysosomal storage diseases (LSDs). A highly experienced management team that has been involved in the development of more than 20 approved drugs leads Vtesse. Its experienced consortium of investors, including Alexandria Real Estate Equities, Inc., Bay City Capital LLC, Lundbeckfond Ventures, New Enterprise Associates, and Pfizer Venture Investments, has committed initial funding that is expected to bring this compound through pivotal clinical trial. Vtesse is based in Gaithersburg, Maryland and is the first spin-out company from Cydan Development, Inc. For more information, visit www.vtessepharma.com.