Vtesse Advances Phase 2b/3 Clinical Trial of VTS-270 in Niemann-Pick Type C1 Disease with Dose Selection for Evaluation in Second and Final Portion of Trial and Expansion into Europe

Ten clinical sites now enrolling patients in the United States, the United Kingdom, Spain, France and Germany

GAITHERSBURG, Md., May 23, 2016 /PRNewswire/ -- Vtesse, Inc. announced today that the dose-finding portion of the company's global, pivotal Phase 2b/3 clinical trial for its lead investigational product, VTS-270, for treatment of Niemann-Pick Type C1 Disease (NPC) is now complete, and a dose level for further testing has been selected by an independent dose selection committee (DSC). Vtesse's ongoing Phase 2b/3 prospective, randomized, double-blind, sham-controlled trial is a three-part, efficacy and safety trial of VTS-270, administered by the lumbar intrathecal (IT) route every two weeks. VTS-270 is based on a specific, well-characterized composition of 2-hydroxypropyl-beta-cyclodextrin.

Twelve patients from the United States and the United Kingdom, spanning ages four to 21, made up the dose-finding portion of the Phase 2b/3 clinical trial. Based on all available data gathered from the first 12 patients' experiences, an independent dose selection committee (DSC) recommended a dose of 900 mg to be administered every two weeks for the duration of the trial to each patient who is randomized for active treatment. This dose was selected for its appropriate balance of safety, tolerability, and potential for efficacy. In addition to its use in the dose-finding phase, there has been significant experience with 900 mg in the Phase 1 clinical trial with VTS-270, further bolstering confidence in the DSC's dose selection.

Vtesse also successfully expanded its clinical trial, which now includes ten study sites in the United States, the United Kingdom, Spain, France and Germany with ten additional sites expected to be initiated soon. Vtesse also anticipates expansion into Turkey and Australia based on discussions with local investigators and patient advocacy groups in those countries.

"We are encouraged by the Phase 1/2 data with VTS-270 to date as well as by the substantial progress in our Phase 2b/3 trial. In selecting a dose and activating 10 clinical sites, we at Vtesse are demonstrating our commitment to rapidly advance this clinical trial and to make it as convenient as possible for patients and their families to participate," said Ben Machielse, Drs., Vtesse President & CEO. "We are pleased that patients and their familes have expressed interest in enrolling in the second portion of this pivotal trial and we are working to fully enroll the remainder of the study in the next few months. With dose selection now complete, families of patients with NPC who are interested in participating should contact one of our

clinical trial sites, as listed on www.theNPCstudy.com, to determine if they are eligible to participate in the remainder of the trial."

Drs. Machielse added, "The entire Vtesse team is exceptionally grateful to the patients, their parents and families, clinicians, and patient advocacy groups who participated in and helped execute the first part of our study. They have made the advancement of this pivotal trial a reality."

In January 2016, Vtesse announced that the U.S. Food & Drug Administration (FDA) granted Breakthrough Therapy designation status for VTS-270 for the treatment of NPC. Both the FDA and the European Medicines Agency (EMA) had previously granted Orphan Drug status to VTS-270. Vtesse's clinical program is designed specifically for VTS-270, which has a unique, well-defined chemical fingerprint based on rigorous manufacturing controls and extensive (bio)chemical characterization. The safety and tolerability of other cyclodextrin compositions, at the doses being studied for VTS-270 and when delivered through the IT route of administration, cannot be assured and is not supported by the scientific evidence that has been developed for 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. The NIH's National Center for Advancing Translational Sciences (NCATS) and *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD), in close collaboration with patients and patient advocacy groups, initiated the drug development phase for VTS-270. Vtesse is leading the late-stage formal drug development process. VTS-270 has been shown to significantly reduce disease progression in animal studies and preliminary data indicate positive trends in exploratory efficacy markers in a Phase 1 clinical trial in NPC patients.

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, other leading academic centers, parents and patient advocacy groups, to advance a pivotal clinical study of VTS-270 (a specific, well-characterized composition of beta-cyclodextrins) to treat NPC, and to conduct pre-clinical discovery and development of other novel drugs for NPC and other lysosomal storage diseases (LSDs). The company is led by a highly experienced management team that has been involved in the development of more than 20 approved drugs. An experienced consortium of investors, including Alexandria Venture Investments, Bay City Capital LLC, Lundbeckfond Ventures, New Enterprise Associates, and Pfizer Venture Investments, has committed initial funding adequate to bring this compound through a 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.

CONTACT: Corporate Contact: Ravi Rao, Ph.D., ravi@vtessepharma.com or Media Contact: Jamie Lacey-Moreira, PressComm PR, LLC, 410-299-3310, jamielacey@presscommpr.com