Vtesse, Inc. Announces Preliminary Data from Ongoing Phase 1 Study of VTS-270 for Treatment of Niemann-Pick Disease Type C

Post-Hoc Analyses of Data Show Preliminary Evidence of Overall Disease Stabilization with Improvement in Several Disease Domains

Gaithersburg, MD, August 6, 2015 – Vtesse, Inc. announced preliminary results today from an open-label Phase 1 clinical trial with VTS-270 (a formulation of (2-hydroxypropyl)-beta-cyclodextrin) for treatment of Niemann-Pick Disease Type C (NPC) conducted by researchers at the National Institutes of Health (NIH) Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD). Preliminary analyses, conducted post-hoc, suggest that the rate of disease progression had slowed down (based on a standardized measure) in children treated with VTS-270 in the Phase 1 trial as compared to the rate in an age- and disease severity-matched cohort obtained from a separate natural history study of NPC patients. The analyses also show that children treated with VTS-270 demonstrated improvement on several disease domains.

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. Researchers at NIH's National Center for Advancing Translational Sciences (NCATS) and NICHD, in close collaboration with Vtesse, patients and patient advocacy groups, developed VTS-270 as part of a project focused on finding treatments for NPC. VTS-270 has been shown to significantly reduce disease progression in naturally occurring animal models and is currently being tested in this Phase 1 clinical trial. Both the ongoing Phase 1 study and the natural history study use a standardized measurement of disease progression, the NPC score, which relies on a specific rating scale that scores the disease along major domains (or traits).

Researchers have matched participants in the Phase 1 study to individuals from the natural history data set according to baseline age and disease severity. The rate of disease progression of this matched cohort was compared to the rate of disease progression of children treated with VTS-270 in the Phase 1 study.

In the Phase 1 trial, among the 12 children treated with direct administration of VTS-270 into the cerebrospinal fluid via an intrathecal administration for more than six months and up to 12 months, the overall NPC score showed a slowing down in the rate of decline. In these patients, when the scores for impact on hearing were removed, the NPC score showed disease stabilization and halting of progression. Domains of cognition and speech have shown improvement of the disease state for participants in the study; while ambulation, fine motor skills, cognition, swallowing, and memory demonstrate slowing down of decline. Measurements of eye movement and hearing appeared to have worsened among participants in the Phase 1 trial.

"This initial analysis of the data look encouraging and this therapy may make a meaningful difference for children with NPC," said Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc. "Based on discussions with regulators, we anticipate that a single pivotal trial would form the basis for approval of VTS-270 in the United States and Europe. The pivotal trial design will be randomized and controlled, and our planned implementation of the trial underscores the essence of our commitment to the NPC patients and their

caregivers. We intend to execute flawlessly and with the greatest care for this sensitive group of patients who are in dire need of new treatment options."

In the Phase 1 trial to date, VTS-270 has been well tolerated other than observed worsening in the eye movement and hearing of some trial participants. Clinicians have administered more than 250 intrathecal administrations of the drug to study participants thus far with minimal administration-related side effects. Vtesse plans to submit more complete Phase 1 clinical trial results for presentation at a scientific meeting later this year. More details about the study can be found athttps://www.clinicaltrials.gov/ct2/show/NCT01747135.

About VTS-270
Vtesse's lead compound, VTS-270, has shown promise in pre-clinical and clinical studies as a potential treatment for NPC. It is a well-characterized mixture of (2-hydroxypropyl)-beta-cyclodextrin that has been extensively evaluated in pre-clinical and clinical studies at NIH, as well as under individual compassionate use investigational new drug applications (iINDs) and in other academic labs. NPC is a genetic disease affecting an estimated one in 100,000 to 150,000 children and is often misdiagnosed and/or under-diagnosed. Affected patients are usually identified in early childhood with ataxia, exhibit progressive impairment of motor and intellectual function, and often die before adulthood. Vtesse is working expediently with NIH's National Center for Advancing Translational Sciences (NCATS) and NICHD, regulatory authorities, patient/parent organizations, physicians and other key stakeholders to soon start a Phase II/III clinical trial to assess the efficacy of the compound for the treatment of NPC. Based upon productive discussions with U.S. Food & Drug Administration (FDA) and the European Medicines Agency (EMA), Vtesse will anticipates that this trial will begin enrolling patients in September 2015.

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 clinical study of VTS-270 for NPC, and to conduct pre-clinical discovery and development of other novel drugs for NPC and other LSDs. Vtesse is led by a highly experienced management team that has been involved in the development of more than 20 approved drugs and vaccines. Its experienced consortium of investors, led by New Enterprise Associates, has committed initial funding that is expected to bring this compound through pivotal clinical trials. Vtesse is based in Gaithersburg, Maryland and is the first spin-out company from Cydan Development, Inc. For more information, visit www.vtessepharma.com.

###

Corporate Contact:
Ravi Venkataramani, Ph.D.
ravi@vtessepharma.com

Vtesse Media Contact:
Jamie Lacey-Moreira
PressComm PR, LLC
410-299-3310
jamielacey@presscommpr.com