FOR IMMEDIATE RELEASE

Vtesse Announces Dosing of First Patient in Germany for Phase 2b/3 Clinical Trial of VTS-270 in Niemann-Pick Type C1 Disease

- *Clinicians Continue to Enroll Children with NPC at Trial Sites in France, Spain, Turkey, the United States and the United Kingdom*
- *Expansion to Australia Expected Soon*

Gaithersburg, MD, October 5, 2016 – Vtesse, Inc., a company committed to developing drugs that will benefit patients with extremely rare, life-threatening diseases, announced today that the first German patient has been dosed in its global, pivotal clinical trial of its investigational drug VTS-270 for Niemann-Pick Type C1 disease ("NPC"). VTS-270 is a well-characterized mixture of HPbCD with a specific compositional fingerprint that distinguishes it from other HPbCD mixtures.

Prof. Dr. Thorsten Marquardt, Head of Metabolic Disorders, University Hospital Münster, and the study's lead principal investigator in Germany, noted: "The current clinical program with VTS-270 is aimed at treating the neurological disease that is the primary cause of mortality in children with NPC. Based on preclinical data suggesting that HPbCDs may not cross the blood brain barrier, it is important that we administer the drug through a lumbar puncture to increase the likelihood that the drug can address the neurological symptoms of NPC at doses that will be well tolerated."

Prof. Dr. Julia B. Hennermann, Head of the Working Group for Lysosomal Storage Diseases at the University Children's Hospital Mainz (known as Villa Metabolica) at the Johannes Gutenberg University Medical Center, dosed the first German patient in the trial. "I'm very happy to be part of this important clinical study, through which we are evaluating the intrathecal use of VTS-270 in children with NPC," she said. "There is a desperate need for more approved therapies that can arrest the neurological disease associated with NPC. As a clinician and based upon the encouraging Phase 1 clinical

data, I am hopeful that VTS-270 can fill this need."

Outside of the United States and Germany, clinical investigators have enrolled and continue to enroll patients at sites in the United Kingdom, France, Spain, and Turkey in the Phase 2b/3 clinical trial of VTS-270. Vtesse expects to have clinical sites available in Australia soon.

"The Vtesse team is committed to conducting this clinical trial at numerous sites around the world, which is critical for the development of VTS-270. We want to make clinical sites easily reachable for the patients who wish to participate in the trial, and are supporting the patients who choose to travel to our clinical sites," said Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc. "To address the unmet needs of patients with NPC, which is an ultra-orphan disease, and to further advance science around this drug candidate, we look forward to moving towards the final phase of this clinical trial."

About Vtesse's Phase 2b/3 Clinical Trial

Vtesse's ongoing Phase 2b/3 prospective, randomized, double-blind, sham-controlled trial of its investigational drug VTS-270 is being conducted in patients affected by NPC disease. It is a three-part efficacy and safety trial of VTS-270, administered by the intrathecal route (IT) every two weeks, with planned enrollment of approximately 51 patients. Intrathecal route of delivery is thought to be important to ameliorating the neurological disease based on studies in pre-clinical models of NPC disease. Phase 1/2 clinical trial data from 14 patients with NPC were presented earlier this year at the 2016 World Symposium on Lysosomal Storage Disease in San Diego, California.

In January 2016, Vtesse announced that the U.S. Food & Drug Administration (FDA) had granted Breakthrough Therapy designation status for VTS-270 for treatment of NPC. Both the FDA and the European Medicines Agency (EMA) had previously granted Orphan Drug status to VTS-270. For more information regarding Vtesse's pivotal Phase

2b/3 clinical trial, including the current list of participating study sites, visit www.theNPCstudy.com.

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) conducted the preclinical research and initiated the drug development phase for VTS-270 in close collaboration with parents and patient support groups. Vtesse is leading the late-stage drug development process.

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 well-characterized mixture of HPbCD with a specific compositional fingerprint that distinguishes it from other HPbCD mixtures) 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 VTS-270 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.

###

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

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