



FOR IMMEDIATE RELEASE

Vtesse Secures Additional \$17 Million in Series A Extension to Support Further Product Development and Expand the Ongoing Clinical Trial of VTS-270 for the Treatment of Niemann-Pick Type C1 Disease

- *Investment Supports Further Product/Technology Improvements and Expanded Access to the Company's Global, Pivotal Study*
- *Clinicians Are Now Enrolling Patients at Sites in France, Spain and Turkey in Addition to US, UK and Germany*

Gaithersburg, MD, July 25, 2016 – [Vtesse, Inc.](#), a company committed to developing drugs that will benefit patients with extremely rare, life-threatening diseases, announced today that it has secured \$17 million in additional Series A funding in support of its global, pivotal clinical trial of VTS-270 for Niemann-Pick Type C1 disease (“NPC”).

All Series A investors contributed to the financing extension, including Alexandria Venture Investments, Bay City Capital LLC, Lundbeckfond Ventures, New Enterprise Associates (NEA), and Pfizer Venture Investments. Together with proceeds from the original Series A announced in January of 2015, Vtesse has raised a total of \$42 million to fund development of its lead product to treat NPC.

“The investor syndicate is pleased to play a role in supporting the development of this breakthrough therapy for NPC patients. This additional capital enables Vtesse to broaden development and expand trial access to patients globally,” said [David Mott](#), General Partner, NEA and Vtesse Board Chair. “The additional funding also allows Vtesse to invest in further product and technology improvements and to develop the commercial strategy to ensure product availability.”

Vtesse also announced today that clinicians have begun enrolling patients at new sites in France, Spain, and Turkey in the Phase 2b/3 clinical trial.



“The early development of VTS-270 has benefited from a close collaboration with parents, patient support groups, the National Institutes of Health (NIH), and our academic collaborators,” said [Ben Machielse](#), Drs., President and Chief Executive Officer of Vtesse, Inc. “We thank them for their ongoing efforts as we broaden the footprint of our pivotal clinical trial, and we are grateful to our investors for their ongoing support of the company.”

About Vtesse’s Phase 2b/3 Clinical Trial

Vtesse’s ongoing Phase 2b/3 prospective, randomized, double-blind, sham-controlled trial of 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.

In May, Vtesse [announced](#) completion of the dose-finding portion of the clinical trial and selection of a dose level (900 mg) for further testing in the patients to be enrolled for the remainder of the trial. An independent dose selection committee (DSC) determined that this dose provides an appropriate balance of safety, tolerability, and potential for efficacy. There has also been significant experience with the 900 mg dose level in the Phase 1/2 clinical trial.

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. The VTS-270 treated group in this inpatient dose escalation study show that, after 12 months and 18 months of monthly dosing, disease progression as measured by the NPC Neurological Severity Score (NSS) was reduced by about 60 percent as compared to a matched natural history study control group. Changes in hearing, which were anticipated as an adverse event, and transient ataxia and transient fatigue were observed in the study.



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) demonstrated that VTS-270 reduces disease progression in animal models of NPC. NCATS and NICHD also 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.

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