



FOR IMMEDIATE RELEASE

Vtesse, Inc. Announces FDA's Granting of Breakthrough Therapy Designation for VTS-270 in Niemann-Pick Type C1 Disease

— *Kevin B. Johnson, Ph.D., Joined Vtesse in October 2015 as Senior Director, Regulatory Affairs*

Gaithersburg, MD, January 6, 2016 – [Vtesse, Inc.](#) announced today that the U.S. Food and Drug Administration (FDA) has granted its drug candidate, VTS-270 for treatment of Niemann-Pick Type C1 Disease (NPC), Breakthrough Therapy designation status. Both the FDA and the European Medicines Agency (EMA) had previously granted Orphan Drug status to VTS-270, which is currently in a pivotal Phase 2b/3 clinical trial.

The FDA grants Breakthrough Therapy designation to companies to help accelerate development and review of drug candidates when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. The designation is designed to ensure that patients can benefit from therapies as soon as possible, without changing FDA standards for new drug approval.

“It is both rewarding and validating to receive the FDA’s Breakthrough Therapy designation for VTS-270, which we believe may provide the first effective treatment for slowing the progress or stabilizing the devastating impacts of NPC in children and adolescents,” said [Ben Machielse](#), Drs., President and Chief Executive Officer of Vtesse, Inc. “This designation is supported by strong preclinical and early clinical data with VTS-270, including that from the Phase 1 study conducted by the National Institutes of Health (NIH). It is our hope that this designation will help to expedite the development and regulatory review process, getting the drug to patients who can benefit sooner.”

The company also announced today that [Kevin B. Johnson](#), Ph.D., M.B.A., had joined Vtesse in October 2015 as Senior Director of Regulatory Affairs in support of the continued advancement of the expedited regulatory process for VTS-270. Dr. Johnson is a 23-year veteran of regulatory affairs in the biotechnology and pharmaceutical industry,



with a background in managing global regulatory strategy and product development, as well as approval of new drugs and biologics for clinical use by the FDA, the EMA, and other regulatory bodies. Before he joined Vtesse, Dr. Johnson led several global regulatory programs in rare diseases and gene therapy for Glaxo SmithKline (GSK).

“We are delighted to have Kevin as our head of regulatory affairs as we move forward with the clinical and regulatory processes for VTS-270. His background in rare diseases and biotechnology is a perfect match for us, as is his passion for combining clear strategy and the necessary details for regulatory approvals,” added Drs. Machielse.

The FDA Breakthrough Therapy designation comes as Vtesse is in the midst of conducting its pivotal Phase 2b/3 clinical trial of VTS-270 for treatment of NPC. Vtesse expects to enroll a total of 51 patients at up to 20 sites (across the United States, the European Union, and other countries) to participate in this clinical trial. For more information on Vtesse’s pivotal Phase 2b/3 clinical trial, visit www.theNPCstudy.com.

“Breakthrough Therapy designation means that the FDA will provide intensive guidance and an organizational commitment to expedited development of VTS-270 for the treatment of NPC,” said Dr. Johnson. “These multidisciplinary interactions with the FDA will support Vtesse’s efficient design and conduct of its regulatory strategy with the goal of driving VTS-270 towards FDA approval.”

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. Researchers at 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 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 significant reduction of disease progression in animal studies and preliminary indications of efficacy in the Phase I study clinical trial.

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 lysosomal storage diseases (LSDs). A highly experienced management team that has been involved in the development of more than 20 approved drugs and vaccines 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.

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