



Vtesse Completes Enrollment of Phase 2b/3 Pivotal Clinical Trial of VTS-270 in Niemann-Pick Type C1 Disease

Company anticipates sharing topline data and completing regulatory submissions in 2018

GAITHERSBURG, Md., March 15, 2017 /PRNewswire/ -- [Vtesse, Inc.](#) announced today that its registrational study of investigational drug VTS-270 in Niemann-Pick Type C1 disease (NPC) is fully enrolled.

"Completing enrollment in the pivotal Phase 2b/3 clinical trial of VTS-270 is a significant milestone for the NPC community and Vtesse, and we are fortunate to have exceeded our goal of 51 participants. We are extremely grateful to the study participants and their families, as well as the clinical site investigators and staff, for their collective commitment and significant contributions to the development of a potential treatment for NPC," said Ben Machielse, Drs., Vtesse's President and Chief Executive Officer. "We look forward to generating topline results from the study early next year."

To reach this significant milestone, Vtesse engaged 20 clinical trial sites across the globe, in the United States, Germany, the United Kingdom, France, Spain, Australia, and Turkey, enabling broad access for study-eligible patients worldwide. Upon completion of the pivotal phase of the trial, Vtesse will continue to provide access to VTS-270 for the clinical trial participants through an open-label extension study. Additionally, Vtesse has initiated a device development program to eliminate the need for lumbar punctures for administration of VTS-270.

"As a physician committed to studying potential new treatments for children facing rare and fatal diseases, it's been incredibly rewarding for me to participate in this trial and to see the completion of patient enrollment," said Elizabeth Berry-Kravis, M.D., Ph.D., pediatric neurologist, Professor of Pediatrics, Neurological Sciences and Biochemistry at Rush University Medical Center, and co-principal investigator of the Vtesse trial. "NPC is a progressive, debilitating and ultimately lethal disease for which there is currently no approved therapy and VTS-270 is offering hope to these patients and their families."

About Vtesse

Vtesse, Inc. is a rare disease company dedicated to developing drugs for patients suffering from underserved diseases. Vtesse closely collaborates with National Institutes of Health (NIH), parents, patient support groups and other academic institutions to advance VTS-270 towards regulatory approval. Vtesse is also progressing earlier stage programs for lysosomal storage diseases, including next-generation therapeutics for NPC.

NPC is a rare genetic disorder that begins impacting the lives of those affected from birth to early adulthood. Clinical symptoms do not slow or reverse, with complications from neurological manifestations being the primary cause of eventual fatalities.

VTS-270 is a well-characterized mixture of 2-hydroxypropyl- β -cyclodextrin (HP β CD) with a specific compositional fingerprint that distinguishes it from other HP β CD mixtures. Preclinical and early clinical studies suggest that the administration of VTS-270 may slow or stop certain indicators of NPC disease. The ongoing Phase 2b/3 study is a prospective, randomized, double-blind, sham-controlled trial of VTS-270. The randomized portion of the trial concludes a year after the full enrollment of the study.

Vtesse is based in Gaithersburg, Maryland. For more information, visit www.vtessepharma.com.