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# Vtesse, Inc. Expands Scientific Advisory Board, Fills Key Patient Advocacy Position to Prepare for Further Clinical Development of VTS-270 in Niemann-Pick Disease Type C (NPC)

GAITHERSBURG, Md., June 15, 2015 /PRNewswire/ --Vtesse, Inc., a rare disease company focused on developing drugs for Niemann-Pick Disease Type C (NPC) and other severe diseases with great unmet need, announced today the expansion of its Scientific Advisory Board (SAB) and the filling of a keystaff position in patient advocacy.

The two new SAB members include Bjorn Hoffstedt, MD, PhD, Managing Director, AB Clinical Development GmbH, and Heiko Runz, MD, Director of Genetics at Merck Research Laboratories in Boston.

"It is critical for the Vtesse SAB members to have diverse experience that cumulatively combines scientific know-how with hands-on patient work and a deep understanding of international drug development processes and regulations," said Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc. "Drs. Hoffstedt and Runz are complementary additions to our SAB and join us as we advance our lead clinical candidate, VTS-270, through later-stage clinical testing as a potential therapeutic option for those facing NPC."

Dr. Hoffstedt has more than 30 years experience in drug development, for both small

molecule and biological drugs. He is board certified in infectious diseases and was cofounder of the European Society of Microbiology and Infectious Diseases and of AB Clinical Development GmbH, a consulting company assisting American and European companies in drug development in Europe.

Dr. Runz is a board-certified medical geneticist and basic scientist with a strong track record in clinical, Mendelian and complex genetics, with specific expertise in Niemann-Pick Disease Type C. His research focus is on translating genetic discoveries into functional insights and early-stage therapeutic applications. Following his medical studies (in Heidelberg, Boston and Strasbourg) and a thesis in molecular genetics, he received two years of postdoctoral training in cell biology/biophysics at the European Molecular Biological Laboratories (EMBL). As a medical doctor, he has five years of experience in all aspects of human genetics, and two more years of training in pediatrics. From 2007-2011, Dr. Runz was an independent group leader, and from 2011-2014 Assistant Professor, at the Institute of Human Genetics at Heidelberg University, as well as the Molecular Medicine Partnership Unit (MMPU), a joint interdisciplinary research initiative between Heidelberg University Clinics and EMBL. From 2013-2014, he was a visiting scholar at the Centre of Human Genetic Research at Massachusetts General Hospital in Boston, and the Broad Institute of MIT and Harvard. Dr. Runz' research spans from technology development to applied medicine and has led to high-ranking publications, significant grant funding, and several prizes.

Carrie Burke, Senior Director, Patient Advocacy, is the new addition to the Vtesse team.

"In our singular pursuit of advancing development of VTS-270, it's the right time to further build out the core strength of the Vtesse team by adding an in-house patient advocate. Carrie's role is of strategic importance in support of finding a new medical solution for those who face NPC," said Drs. Machielse. "As Senior Director, Patient Advocacy, she will expand our focus on interacting with patients and their caregivers."

Ms. Burke leads Vtesse's patient advocacy to ensure that the needs of the patients served by Vtesse will always remain at the heart of everything the company does. She has over 17 years of experience including 10 years in rare diseases.

Prior to joining Vtesse, Ms. Burke was the Director of Alliance Development for Shire where she was responsible for strategic partnerships with external advocacy organizations. She has experience in legislative policy, regulatory policy, access, clinical trials, product launches, newborn screening, awareness building, and strengthening the voice for rare diseases through alignment of external stakeholders. Before joining Shire, she held positions at Centocor, 3M Pharmaceuticals, Solvay, and Novartis. Ms. Burke attended the University of Georgia and holds a Bachelor of Science in Biochemistry and Molecular Biology.

**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. Vtesse aims to work expediently with NIH's NCATS and NICHD, regulatory authorities, patient/parent organizations, physicians and other key stakeholders to start a Phase II/III clinical trial to assess the efficacy of the compound for the treatment of NPC. Pending the outcomes of discussions with the U.S. Food & Drug Administration (FDA) and the European Medicines Agency (EMA), Vtesse will provide an update on anticipated timing for such a 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 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.

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