



Medicines and Healthcare Products Regulatory Agency ("MHRA") Designates VTS-270 as a Promising Innovative Medicine

- Designation is first step to inclusion in Early Access to Medicines Scheme ("EAMS"), providing patients with faster access to innovative medicines

GAITHERSBURG, Md., Nov. 2, 2016 /PRNewswire/ -- [Vtesse, Inc.](#), a company committed to developing drugs that will benefit patients with extremely rare, life-threatening diseases, announced today that the MHRA, an executive agency of the Department of Health in the United Kingdom that is responsible for ensuring that medicines and medical devices are acceptably safe, has granted a Promising Innovative Medicine ("PIM") designation for VTS-270, Vtesse's investigational drug for children with Niemann-Pick Type C1 disease ("NPC").

"It is extremely gratifying to the NPC community, to the team at Vtesse, and to all of the individuals, organizations, and institutions who are tirelessly working on the development of VTS-270 that it has been recognized by the MHRA as a scientific innovation that may improve the lives of people living with NPC," said [Ben Machielse](#), Drs., President and Chief Executive Officer of Vtesse, Inc. "It takes considerable collaboration and dedication to advance the clinical study and regulatory processes for drug development in the rare disease space. Above all, we thank all the parents who have supported the development of VTS-270."

"This UK PIM designation, coupled with the U.S. FDA Breakthrough Therapy Designation granted earlier this year, demonstrates the strength of the preliminary clinical data of NPC treatment with VTS-270," added [Kevin Johnson](#), PhD, MBA, Vice President, Regulatory Affairs at Vtesse. "Both designations afford us enhanced regulatory opportunities, which we will rely on as we complete our rigorous Phase 2b/3 clinical trial and seek regulatory approvals to bring this drug to market as quickly as possible."

A PIM Designation is an early indication that a medicinal product is a promising candidate for the Early Access to Medicines Scheme ("EAMS"), in the treatment, diagnosis or prevention of life-threatening or seriously debilitating conditions with unmet need. The EAMS is a UK program run by the MHRA that aims to give patients with life-threatening conditions access to specified pre-license medicines when there is a clear medical need.

"The PIM designation, which is based on Phase 1/2 clinical trial data from patients with NPC, is an important achievement for Vtesse, VTS-270 and the Niemann-Pick community," said Paul Gissen, PhD, MRCPCH, Great Ormond Street Hospital, who is an investigator in Vtesse's Phase 2b/3 clinical trial of VTS-270. "The MHRA's PIM designation is based on three criteria: a life-threatening or seriously debilitating condition with a high unmet medical need, a medicinal product that is likely to offer major advantages over current treatment used in the UK, and a

reasonable expectation of a positive benefit-risk balance for patients. We look forward to continuing study with VTS-270, and – as a clinical investigator – I'm pleased that the MHRA has granted this designation."

Results from the VTS-270 treated group in the inpatient Phase 1/2 dose escalation study that, after 12 months and 18 months of monthly dosing, disease progression as measured by the NPC Neurological Severity Score (NSS) was reduced 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.

Vtesse's ongoing Phase 2b/3 clinical trial of VTS-270 is enrolling patients in the United Kingdom at Birmingham Children's Hospital and Great Ormond Street Hospital. The trial is also ongoing at sites in the United States, France, Germany, Spain, Turkey and Australia. For more information, 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 HP β CD with a specific compositional fingerprint that distinguishes it from other HP β CD 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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