



March 25th, 2015

Vtesse, Inc. Forms Scientific Advisory Board

- Niemann-Pick Disease experts Drs. Paul Gissen, Marc Patterson and Forbes D. Porter, along with biopharmaceutical industry leader Dr. Cristina Csimma, join Vtesse in quest to develop new treatments for people facing Niemann-Pick Disease Type C and rare diseases

Gaithersburg, MD, March 25, 2015 – 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 formation of its Scientific Advisory Board (SAB). Comprising experts from a range of fields including pediatrics, translational medicine, biomarker research and genetics, the SAB will advise on clinical and regulatory strategy as well as product development for the company's lead candidate, VTS-270.

“Our SAB is comprised of leading international scientific and medical experts in NPC and in many disciplines that are important for the successful development of drugs for those facing rare diseases. These prominent researchers share Vtesse’s mission to develop new treatments to significantly improve the quality of life for people suffering from NPC and other lysosomal storage diseases (LSDs),” said Ben Machielse, Drs, President and Chief Executive Officer of Vtesse, Inc. “We also share a common, strong belief that VTS-270, our lead clinical candidate, has the potential to be an important drug based on the trove of research completed and effort made to date from parents, scientists, and clinicians.”

Members of the Vtesse SAB include:

- Cristina Csimma, PharmD, MHP, Co-Chair, an established leader across the biopharmaceutical industry, venture capital and academic settings, and former founding chief executive officer of Cydan Development, Inc.
- Forbes D. Porter, MD, PhD, Co-Chair, a Senior Investigator and Program Head in the intramural research program of the Developmental Endocrinology and Genetics Program (PDEGEN) of the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), National Institutes of Health (NIH), Principal Investigator for Phase I Clinical Trial of VTS-270
- Paul Gissen, MBChB, PhD, Professor, University College of London; Honorary Consultant in Paediatric Metabolic Diseases, Great Ormond Street Hospital; Wellcome Trust Senior Research Fellow in Clinical Sciences, University College London Institute of Child Health
- Marc C. Patterson, MD, FRACP, Chair, Division of Child and Adolescent Neurology; Professor of Neurology, Pediatrics and Medical Genetics, Mayo Clinic Children’s Center, Rochester, MN

"It's an honor for me to serve as co-chair of the Vtesse SAB and to work alongside key thought leaders to support the goal of developing new drugs for children facing NPC and for those with other rare diseases," said Dr. Csimma. "The development of VTS-270 is an important step in the understanding of NPC and enables further progress towards defeating this devastating disease."

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 National Center for Advancing Translational Sciences (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. The first spin-out company from Cydan Development, Inc., an orphan-drug accelerator that identifies and de-risks programs with therapeutic and commercial potential, Vtesse is working collaboratively with the NIH 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. For more information, visit www.vtessepharma.com.

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Corporate Contact:

Ravi Venkataramani, Ph.D.

rvenkataramani@vtessepharma.com

Vtesse Media Contact:

Jamie Lacey-Moreira

PressComm PR, LLC

410-299-3310

<http://www.vtessepharma.com/#!2015mar25-vtesse-forms-scientific-adviso/c1f19>