FOR IMMEDIATE RELEASE

Leading Life Science Syndicate Commits $25 Million to Series A Funding to Launch Vtesse, Inc., the First Rare Disease Company Spun Out of Cydan Development, Inc.

- Vtesse to collaborate with National Institutes of Health on development of VTS-270 for Niemann-Pick Disease Type C and other novel drugs for life-threatening rare diseases

Gaithersburg, MD, and Cambridge, MA, January 7, 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 that it has raised $25 million in Series A funding. Vtesse is the first spin-off company for Cydan Development, Inc., an orphan-drug accelerator that shares with Vtesse the same syndicate of leading life sciences investors that are committed to funding additional rare disease companies. New Enterprise Associates (NEA) led the Vtesse financing with participation from Pfizer Venture Investments, Lundbeckfond Ventures, Bay City Capital and Alexandria Venture Investments.

Vtesse also announced that it has established a Cooperative Research and Development Agreement (CRADA) with the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) and the National Center for Advancing Translational Sciences (NCATS), each a component of the National Institutes of Health (NIH). Vtesse and NCATS have also entered into a licensing agreement for the current rights held by NIH for the worldwide use of cyclodextrin, delta-tocopherol, and derivatives of tocopherol, alone or in combination, for the treatment of lysosomal storage diseases (LSDs), including NPC. Regulatory orphan designations for the U.S. and EU will be also be transferred to Vtesse.

Vtesse will use the proceeds from its Series A financing to conduct a clinical program for VTS-270 (a formulation of (2-hydroxypropyl)-beta-cyclodextrin) for NPC, and to discover and pre-clinically evaluate additional novel drugs for NPC and other LSDs. NPC is a
genetic disease associated with mutations in NPC1 and NPC2 genes. NPC affects an estimated one in 100,000 to 150,000 children and is often misdiagnosed and/or under-diagnosed. Affected patients are usually identified in early childhood with ataxia and exhibit progressive impairment of motor and intellectual function, and often die before adulthood.

“We’ve launched Vtesse to rapidly advance the clinical development of VTS-270, which we hope to make widely available to the many young patients suffering from the debilitating effects of NPC,” said Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc. “We expect to listen to and learn from the physicians, independent researchers, parents and patients who have worked tirelessly for many years to find a treatment for this devastating disorder. We are grateful for the work they and the NIH have conducted thus far, and we are committed to developing a broadly available treatment for NPC.”

“The launch of Vtesse is an important milestone for Cydan as this new company holds great promise for patients and is an excellent example of collaboration among scientific, patient and investment stakeholders,” said Chris Adams, Ph.D., Founder and Chief Executive Officer at Cydan and an independent member of the Vtesse Board of Directors. “Our goal at Cydan Development is to advance innovative treatments for patients with rare diseases by identifying promising assets, accelerating their pre-clinical and clinical development, selecting the right management team, and, ultimately, creating more companies like Vtesse.”

**Vtesse and NIH to Collaborate on VTS-270 Clinical Study**

Under the CRADA, Vtesse, NICHD and NCATS intend to collaborate to launch a second VTS-270 clinical study for the treatment of NPC as well as to develop other novel drugs (delta-tocopherol and combinations) for NPC and other LSDs. Under the terms of the agreement, NICHD, which is the current sponsor of a Phase I clinical trial of VTS-270 in patients with NPC, Type 1, will transfer its investigational new drug (IND) application to Vtesse along with all background data on the program. Vtesse will be responsible for all
further development of VTS-270 with the intent to seek marketing approval for VTS-270 from U.S. and European regulatory agencies.

“This is an excellent example of how launching a project to study the underlying biology of one disease can lead to advances that hold promise for an entire group of diseases — the NCATS goal of finding what is common among diseases and the translational science process,” said NCATS Director Christopher P. Austin, M.D. “I am grateful to all of the NPC patients, their families and patient support groups who have been equal partners in our efforts to find therapeutic solutions to these devastating disorders.”

**Experienced Life Science Industry Team to Lead Vtesse**

Biopharmaceutical industry veteran Ben Machielse, Drs., will serve as Vtesse President and Chief Executive Officer. Before founding Vtesse, Drs. Machielse led drug development of Omthera Pharmaceutical Inc.’s recently approved product, EPANOVA®, and was involved in the sale of the specialty drug maker to AstraZeneca. Previously, he held senior executive positions at Centocor, Xoma and MedImmune/AstraZeneca.

Vtesse’s management team also includes Allan Darling, Ph.D., Vice President, Technical Operations; Sarah Frech, D.V.M., M.P.H., Vice President, Clinical Research; and Ravi Venkataramani, Ph.D., Chief Business Officer. Each of these individuals has extensive industry experience at companies such as MedImmune/AstraZeneca, Invitrogen/BioReliance, Baxter Healthcare and Genocea Biosciences, and all have been involved in the successful clinical development and commercialization of drugs.

The Board of Directors for Vtesse, Inc. includes David Mott, NEA, Board Chair; Ben Machielse, Drs., Vtesse, Inc.; Sara Nayeem, M.D., NEA; Barbara Dalton, Ph.D., Pfizer Venture Investments; Mette Kirstine Agger, Lundbeckfond Ventures; and Carl Goldfischer, M.D., Bay City Capital; as well as veteran rare disease industry experts, Cristina Csimma, Pharm.D., and Chris Adams, Ph.D., both of whom will serve as independent directors.
“Vtesse combines an experienced management team with a strong financial and scientific foundation and a commitment to the NPC community,” said Mr. Mott. “With this vision and support, we expect Vtesse to move VTS-270 forward quickly and with the utmost attention to medical excellence.”

**About VTS-270**

Vtesse’s lead compound, VTS-270, has shown promise in pre-clinical and clinical studies as a potential treatment for Niemann-Pick Disease Type C (NPC). It is a well-characterized mixture of (2-hydroxypropyl)-beta-cyclodextrin that has been extensively evaluated in pre-clinical and clinical studies at NCATS, NICHD and the NIH Clinical Center, as well as under individual compassionate use investigational new drug applications (iINDs) and in other academic labs. Vtesse aims to work expeditiously with 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.

About Cydan Development, Inc.
Cydan is an orphan-drug accelerator that identifies and de-risks orphan drug products with significant therapeutic and commercial potential, with the goal of starting multiple companies to develop such therapies. Cydan was launched in 2013 by a management team with extensive drug discovery, clinical development, and business development experience. Cydan is financed by leading life sciences investors NEA, Pfizer Venture Investments, Lundbeckfond Ventures, Bay City Capital and Alexandria Venture Investments. The accelerator is based in Cambridge, Massachusetts. For more information, visit www.cydanco.com or contact Cydan at info@cydanco.com.

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