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# Cydan syndicate backs orphan-focused Vtesse with plans for more rare disease plays

***By Michael Fitzhugh, Staff Writer***

Vtesse Inc., a new rare disease company focused on pushing a potentially transformational lysosomal storage disease (LSD) drug into pivotal testing this year, has raised a $25 million series A round from the syndicate of life sciences investors behind Cydan Development Inc., an orphan drug accelerator building a glide-way for therapies targeting monogenic diseases.

Led by Cambridge, Mass.-based Cydan's lead backer, New Enterprise Associates, the new financing will help Vtesse carry a drug called cyclodextrin (VTS-270) through an ongoing phase I study in children with Niemann-Pick disease type C and into a phase II/III study for the rare genetic disease before the end of 2015. Pfizer Venture Investments, Lundbeckfond Ventures, Bay City Capital and Alexandria Venture Investments also participated in the round.

Licenses for both drugs, their orphan designations, comprehensive data packages and an investigational new drug application for the phase I study were handed off to Vtesse from a multi-institutional team coordinated by the National Center for Advancing Translational Sciences (NCATS), validating efforts by a team of more than 20 researchers working with NCATS' Therapeutics for Rare and Neglected Diseases (TRND) as well as its Chemical Genomics Center and the NCATS Pharmaceutical Collection to accelerate the movement of a pilot projects in a diverse range of disease areas from the lab to commercial application.

"The TRND program, as well as many others, were specifically started to derisk assets and then move them into the private sector," NCATS director Chris Austin told *BioWorld Today*. The pharmaceutical collection, from which the molecules were sourced, was created for just this type of application, he said, "If you have a rare disease, where the kids have a life expectancy of five years, which many rare diseases have, including NPC, then you don't have the time to go through the 10-to-15-year drug development process."

In addition to working on NPC type C, Gaithersburg, Md.-based Vtesse has also established a cooperative research and development agreement (CRADA) with the Eunice Kennedy Shriver National Institute of Child Health and Human Development and NCATS to explore the use of cyclodextrin in additional lipid storage diseases (LSDs), whether or not delta-tocopherol – a rare form of vitamin E – is effective in treating any LSDs, and whether its possible that cyclodextrin and delta-tocopherol will work better together than they work alone.

There are about 50 different LSDs. They're rare, inherited and often-fatal disorders that usually affect children. Fatty materials accumulate in the cells and tissues of the body with symptoms that can result in damage to the brain, peripheral nervous system, liver, and other organs and tissues. When it comes to LSDs, said Austin, cyclodextrin is a bit like the Syrup of ipecac for lysosomes, causing them to eject harmful junk collected in lysosomal storage disorders.

With a global team, the National Institutes of Health (NIH) built a data package of "exceptional quality," Ben Machielse, president and CEO of Vtesse told *BioWorld Today*. "We actually know a lot about this molecule and its possible efficacy, and that's very encouraging," he said. "Cydan is a perfect connection between an public institution like NIH and a private company like us. They allowed us to do due diligence on the preclinical package, derisk it, see where the holes are to see how strong it is, and do a commercial assessment." In working with Cydan since last May, Machielse said he's seen Cydan's six-person team play the role of a translator of the early research on its journey to becoming a commercial clinical development plan. "I think that's very powerful," he said, "because they speak the language of both sides of the aisle, so to speak."

Cyclodextrin came out of the NIH with much of the derisking work already done. That allowed Cydan to spin out Vtesse, its first company, with perhaps less effort than future spin-offs might require. But the accelerator is well prepared for that work, set up from the start to spot and vet promising opportunities with even less substantial foundations, Chris Adams, Cydan's founder and CEO, told *BioWorld Today*.

The accelerator's premise, Adams said, is that there are

often lots of opportunities that are too early for VCs to take on, because there's some key piece of data missing, or maybe the data haven't been robustly proven.

"What this group of investors thought about is, 'What if we create a dedicated unit that is just looking at genetic diseases with the right experience to go out and find these opportunities and then do the killer experiment to end up with some things that hopefully translate into potential clinical benefit for patients,' which is really the goal: to find things that we think are really disease-modifying for these patients."

Cydan launched in 2013 with $26 million in financing led by New Enterprise Associates (NEA), Pfizer Venture Investments, Lundbeckfond Ventures and Bay City Capital with participation from Alexandria Real Estate Equities Inc. That same team backed Vtesse, led again by NEA. Though unique in its focus on the orphan space, it joins diverse efforts such as Triphase Accelerator Corp., Biomotiv LLC and the Avalon Ventures/Glaxosmithkline plc effort, all of which are seeking to give promising early stage assets a thorough vetting and the resources they need to get off the ground. (See *BioWorld Insight*, Aug. 19, 2013, and *BioWorld Today*, April 24, 2013, and Nov. 12, 2014.)

"There's lots of accelerators out there," said Adams. "But there are very few that accelerate to a decision point where the capital is is reserve, so that you can seamlessly transition the asset into a newco."

One solid precedent for Vtesse's potential success: Aesrx LLC, which licensed the first drug candidate developed by NCATS and its collaborators in July 2014, has since been purchased by Baxter International. (See *BioWorld Today*, Sept. 9, 2013.)