Applied Therapeutics Announces Initiation of Registrational Phase 2/3 Study of AT-007 in SORD Deficiency

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NEW YORK, Dec. 16, 2021 (GLOBE NEWSWIRE) -- Applied Therapeutics, Inc. (Nasdaq: APLT), a clinical-stage biopharmaceutical company developing a pipeline of novel drug candidates against validated molecular targets in indications of high unmet medical need, today announced the initiation of a registrational phase 2/3 study of AT-007 in Sorbitol Dehydrogenase (SORD) Deficiency. The study, termed INSPIRE (**IN**hibition of **S**orbitol **P**roduction through **I**nhibition of the Aldose **R**eductase **E**nzyme), will investigate biomarker efficacy, clinical outcomes and safety in people living with SORD Deficiency treated with AT-007 vs. placebo.

Sorbitol Dehydrogenase Deficiency (SORD Deficiency) is a rare, progressive, debilitating hereditary neuropathy that affects peripheral nerves and motor neurons. SORD Deficiency is one of the most common forms of recessive hereditary neuropathy and affects approximately 3,300 patients in the U.S. and 4,000 patients in Europe. The disease is caused by a lack of the enzyme sorbitol dehydrogenase, responsible for metabolism of sorbitol, which causes sorbitol to accumulate at high levels and become toxic to the body. Sorbitol accumulation results in significant disability, loss of sensory function, and neuromuscular dysfunction.

Inhibition of Aldose Reductase addresses the underlying cause of SORD Deficiency by preventing the conversion of glucose into sorbitol, which has been shown to be up to one hundred times higher in the blood of patients with SORD Deficiency compared with unaffected individuals. In a pilot open-label study in 8 SORD Deficiency patients, AT-007 reduced blood sorbitol levels by approximately 66% from baseline through 30 days of treatment. The range of reduction from baseline in patients was 54%-75%. AT-007 was safe and well tolerated in all treated patients.

The global registrational phase 2/3 placebo-controlled trial is designed to evaluate both biomarker efficacy (reduction in sorbitol) and clinical outcomes in patients with SORD Deficiency. The primary biomarker efficacy endpoint will measure reduction in sorbitol at 3 months of treatment compared to baseline. The primary clinical outcome measure will assess changes in Charcot-Marie-Tooth Functional Outcome Measures (CMT-FOM) lower limb function domain, a key measure of mobility and motility in SORD patients, which is greatly impacted by the disease. Key secondary clinical outcomes include CMT-FOM domain sub-scales, CMT Health Index (CMT-HI) patient reported outcomes, fatigue assessment and muscle MRI.

“The INSPIRE registrational trial in SORD Deficiency represents an important milestone for patients and is the second late-stage program initiated with AT-007,” said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics. “The science demonstrating the role of aldose reductase, and the toxic impact of excess sorbitol, is well-established. We are excited to have achieved proof of concept in the pilot trial and look forward to continuing to advance AT-007 for SORD Deficiency.”

“People living with SORD Deficiency are in urgent need of a therapeutic option. The initiation of the first registrational trial in SORD Deficiency marks an important milestone for the patient community,” said Amy Gray, CEO of the Charcot-Marie-Tooth Association. “Developing partnerships with companies like Applied Therapeutics is central to the mission of the CMTA and important to the CMT community.”

“We look forward to collaborating with Applied Therapeutics and are excited to increase awareness of SORD Deficiency and the potential for a new treatment that could change the course of the disease,” said Allison Moore, Founder and CEO of the Hereditary Neuropathy Foundation.

If you or someone you know are interested in qualifying for a free screening for SORD Deficiency, please email: sord@appliedtherapeutics.com

About Applied Therapeutics

Applied Therapeutics is a clinical-stage biopharmaceutical company developing a pipeline of novel drug candidates against validated molecular targets in indications of high unmet medical need. The Company’s lead drug candidate, AT-007, is a novel central nervous system penetrant Aldose Reductase Inhibitor (ARI) for the treatment of CNS rare metabolic diseases, including Galactosemia, SORD Deficiency and PMM2-CDG. The Company is also developing AT-001, a novel potent ARI, for the treatment of Diabetic Cardiomyopathy, or DbCM, a fatal fibrosis of the heart. The preclinical pipeline also includes AT-003, an ARI designed to cross through the back of the eye when dosed orally, for the treatment of Diabetic retinopathy, as well as novel dual PI3k inhibitors in preclinical development for orphan oncology indications.

To learn more, please visit www.appliedtherapeutics.com and follow the company on Twitter @Applied_Tx.

Forward-Looking Statements

This press release contains “forward-looking statements” that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. Any statements, other than statements of historical fact, included in this press release regarding strategy, future operations, prospects, plans and objectives of management, including words such as “may,” “will,” “expect,” “anticipate,” “plan,” “intend,” and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are forward-looking statements. Forward-looking statements in this release involve substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by the forward-looking statements, and we, therefore cannot assure you that our plans, intentions, expectations or strategies will be attained or achieved.

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