FDA Grants AT-007 Pediatric Rare Disease Designation and Orphan Designation for Treatment of PMM2-CDG

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NEW YORK, Sept. 24, 2020 (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, announced today that the U.S. Food and Drug Administration (FDA) has granted AT-007 both Pediatric Rare Disease Designation and Orphan Drug Designation for treatment of PMM2-CDG. PMM2-CDG is a debilitating rare disease caused by deficiency in the critical enzyme phosphomannomutase-2, required for systemic glycosylation of proteins. PMM2-CDG causes multiple organ failure and severe disability, resulting in approximately 20% mortality in the first four years of life. There are currently no drugs approved to treat PMM2-CDG.

AT-007, a novel CNS penetrant Aldose Reductase inhibitor (recently given the INN scientific name gavorestat) has demonstrated biological activity in a validated model of PMM2-CDG. Applied Therapeutics plans to initiate a clinical study in 2021 and is currently working with CDG experts and the FDA to design a robust clinical program.

“PMM2-CDG is a debilitating disease with no drugs available, which is central to Applied Therapeutics’ mission of creating life-changing treatments for patients who desperately need them,” said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics. “Over the past year we have been working closely with clinicians, researchers and families of PMM2-CDG patients, and are grateful for their collaboration and support. We are pleased that the FDA recognizes the transformative potential of AT-007 in treating patients with PMM2-CDG.”

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 and PMM2-CDG. The Company initiated a Galactosemia clinical program in June 2019, read out positive data in adult Galactosemia patients in April 2020, and commenced a pediatric Galactosemia study in June 2020. Applied plans to initiate a clinical study of AT-007 in PMM2-CDG in 2021.

The Company is also developing AT-001, a novel potent ARI that is being developed for the treatment of Diabetic Cardiomyopathy, or DbCM, a fatal fibrosis of the heart. The Company initiated a Phase 3 registrational study in DbCM in September 2019. 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.

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