Applied Therapeutics to Present Data on AT-007 for the Treatment of Galactosemia at the American Society of Human Genetics (ASHG) 2020 Annual Meeting

October 21, 2020

NEW YORK, Oct. 21, 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 it will present two poster presentations covering AT-007 in Galactosemia at the upcoming American Society of Human Genetics (ASHG) 2020 Annual Meeting. The presentations include animal model efficacy data and adult clinical data on the safety and biomarker efficacy of Applied Therapeutics' investigational candidate for Galactosemia, AT-007, a Central Nervous System (CNS) penetrant Aldose Reductase Inhibitor (ARI).

"We are pleased to present data on our Galactosemia program at the ASHG conference," said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics. "Our preclinical data demonstrates that reduction in galactitol, a toxic metabolite of galactose, prevents long-term CNS complications in an animal model of Galactosemia. In parallel, our clinical data from the ACTION-Galactosemia study demonstrates rapid and sustained reduction in galactitol with once-daily AT-007 treatment. Together, this data represents an important advancement in our understanding of the disease and a potential therapeutic intervention to halt disease progression."

Presentation Details

Poster #1881 (Abstract #3646): Positive Biomarker Efficacy Results from the ACTION-Galactosemia Study
Presenter: Riccardo Perfetti, M.D., Ph.D., Chief Medical Officer of Applied Therapeutics
Time: Monday, October 26, 6:00 a.m. – 11:59 p.m. EDT

Poster #1958 (Abstract #3647): Post-Natal Galactitol Reduction is Associated with Normalization of CNS Phenotype in Animal Model of Galactosemia
Presenter: Riccardo Perfetti, M.D., Ph.D., Chief Medical Officer of Applied Therapeutics
Time: Monday, October 26, 6:00 a.m. – 11:59 p.m. EDT

Slides will be available on the Presentations and Publications section of the Applied Therapeutics website following the conference.

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 Galactosemia, a rare pediatric metabolic disease. The Company initiated a pivotal Phase 1/2 clinical trial in June 2019, read out positive top-line biomarker data in adult Galactosemia patients in January 2020 and announced full data from the trial in April 2020. A pediatric Galactosemia study commenced in June 2020. 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.

About AT-007

AT-007 is a central nervous system (CNS) penetrant Aldose Reductase inhibitor (ARI) in clinical development for treatment of Galactosemia. AT-007 has been studied in an animal model of Galactosemia, which demonstrated that AT-007 reduces toxic galactitol levels and prevents disease complications. Applied Therapeutics is conducting a biomarker based development program in patients with Galactosemia, based on the recently released industry guidance on drug development for low prevalence, slowly progressing rare metabolic diseases. The company received Orphan Designation for AT-007 for Galactosemia in May 2019 and Pediatric Rare Disease Voucher (PRV) designation in 2020.

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