Applied Therapeutics Announces Positive Data Trend in AT-007 ACTION-Galactosemia Kids Pediatric Trial; Trial Will Continue to 18 Months in Blinded Format

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• The study did not yet reach statistical significance at 12 months of treatment; however, AT-007 (gavorostat) demonstrated a trend in clinical benefit vs. placebo

• Applied Therapeutics plans to meet with the EMA to discuss potential submission of the current data package for conditional approval in the EU

• Trial will continue to 18 months in blinded format

NEW YORK, Oct. 06, 2022 (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 that the ACTION-Galactosemia Kids Phase 3 trial has demonstrated a trend in clinical benefit favoring AT-007 vs. placebo. The study is designed to evaluate the impact of AT-007 vs. placebo on clinical outcomes in children with Classic Galactosemia, with a review of safety and efficacy every 6 months by a firewalled Data Monitoring Committee (DMC) until the study reaches statistical significance. Review of the data at 12 months of treatment by the DMC indicated that while the study primary endpoint has not yet reached statistical significance, a trend exists favoring AT-007 vs. placebo. The clinical benefit at this early time point was most pronounced in patients with significant deficits in clinical performance at baseline. Safety data demonstrated that AT-007 continues to be safe and well tolerated. The study will proceed in blinded format to the next review at 18 months of treatment. In the meantime, the Company will meet with the EMA to discuss potential submission of an MAA based on existing data for conditional approval.

“Galactosemia is a debilitating disease that greatly impacts patients and families, and there are currently no approved treatments available,” said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics. “The data to date in the ACTION-Galactosemia Kids study provides hope for patients and families living with this disease. We know that entering a child in a long-term placebo-controlled study is a difficult decision, and we are truly thankful to the families who are participating in the ACTION-Galactosemia clinical program.”

About AT-007

AT-007 is a central nervous system (CNS) penetrant Aldose Reductase inhibitor (ARI) in development for the treatment of several rare neurological diseases, including Galactosemia, SORD Deficiency, and PMM2-CDG. In clinical trials, AT-007 significantly reduced plasma galactitol levels vs. placebo in adults and children with Galactosemia. AT-007 is currently being studied in a Phase 3 clinical outcomes trial (ACTION-Galactosemia Kids) in children ages 2-17 with Galactosemia, as well as a long-term open-label study in adults with Galactosemia. In a pilot study in adults with SORD Deficiency, AT-007 significantly reduced blood sorbitol levels. AT-007 is currently being studied in a Phase 3 trial (INSPIRE) investigating biomarker efficacy, clinical outcomes, and significantly reduced blood sorbitol levels in adults with SORD Deficiency. The drug has been generally safe and well tolerated in all clinical studies to date. AT-007 has received both Orphan Drug and Pediatric Rare Disease designations from the U.S. Food and Drug Administration (FDA) for the treatment of Galactosemia and PMM2-CDG, and Fast Track designation for Galactosemia.

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.

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.

Such risks and uncertainties include, without limitation, factors that may cause actual results to differ from those expressed or implied in the forward-looking statements in this press release are discussed in our filings with the U.S. Securities and Exchange Commission, including the “Risk Factors” contained therein. Except as otherwise required by law, we disclaim any intention or obligation to update or revise any forward-looking statements, which speak only as of the date they were made, whether as a result of new information, future events or circumstances or otherwise.

Contacts

Investors:

Maeve Conneighton
(212) 600-1902 or
appliedtherapeutics@argotpartners.com

Media:

media@appliedtherapeutics.com

Applied Therapeutics, Inc.