



Applied Therapeutics to Present at the 43rd Annual Meeting of the Society for Inherited Metabolic Disorders

April 4, 2022

NEW YORK, April 04, 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 poster presentations at the 2022 43rd Annual Meeting of the Society for Inherited Metabolic Disorders, April 10 - 13, 2022 in Orlando, Florida.

"Baseline data from the ACTION-Galactosemia Kids study illustrates the high burden of disease associated with Galactosemia, including neurological complications, due to high galactitol levels," said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics. "This data underscores the urgent need to bring a treatment to people living with Galactosemia."

Presentation Details

POSTER #68: Baseline Disease Characteristics of 47 Pediatric Classic Galactosemia Patients in the Action-Galactosemia Kids AT-007 Interventional Study Demonstrate the Multi-System Burden of Disease

R Perfetti, A Ahmad, H Phan, J Thomas, F Lawson, E Bailey, L Saltonstall, and S Shendelman

- In the ACTION-Galactosemia Kids study, children presented with deficits in speech, cognition, motor skills, and adaptive behavior.

POSTER #69: Galactitol, But Not Gal-1p, Level is a Predictor of Disease Severity in Children with Classic Galactosemia on Galactose Restricted Diet

R Perfetti, E Bailey, F Lawson, S Wang, L Saltonstall, M Krumnow, and S Shendelman

- Higher galactitol levels, but not higher Gal-1p levels, were associated with greater disease severity overall and on each of the four quadrants of CNS function.

About Galactosemia

Galactosemia is a rare genetic metabolic disease resulting in an inability to metabolize the simple sugar galactose. Galactose is found in foods but is also produced endogenously by the body. When not metabolized properly, galactose is converted to the toxic metabolite, galactitol, which causes neurological complications, including deficiencies in speech, cognition, behavior, and motor skills, and also results in juvenile cataracts and ovarian insufficiency (in women). There are approximately 3,000 patients with Galactosemia in the US and 80 new births per year.

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 an animal model of Galactosemia, AT-007 reduced toxic galactitol levels and prevented disease complications. 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. 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, 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.

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.

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