Applied Therapeutics Announces MRS Data from ACTION-Galactosemia Study

December 9, 2020

NEW YORK, Dec. 09, 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, today announced magnetic resonance spectroscopy (MRS) data on reduction of galactitol levels in the brain of Galactosemia patients treated with AT-007 in the ACTION-Galactodsemia adult study. Overall, plasma reduction in galactitol correlated with brain reduction in galactitol. There were two exceptions, which may have resulted from incomplete peak separation on the MRS scans. At the two doses which demonstrated statistically significant reduction in plasma galactitol, 20 and 40mg/kg, 3 out of 4 patients displayed substantial galactitol reduction ranging from 61.94% to 69.80% reduction from baseline.

“We are pleased to share our MRS data from ACTION-Galactosemia and believe this represents an important scientific advancement in the field of Galactosemia research,” said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics. “We have demonstrated that galactitol can be quantitated in the brain of Galactosemia patients on a restricted diet, and that levels of galactitol in the brain can be reduced through treatment with a AT-007, a CNS penetrant aldose reductase inhibitor.”

Additional data on galactitol reduction in ACTION-Galactosemia can be found below. Applied Therapeutics will hold a conference call to discuss the data in more detail at 8:30 a.m. ET, and slides can be downloaded prior to the meeting at https://ir.appliedtherapeutics.com/.

Graphic 1 accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/a3c76cbc-4979-4ad2-8937-24b1c5cf1706

Conference Call at 8:30 a.m. Eastern Time
Applied Therapeutics will hold a conference call to discuss MRS data from the ACTION-Galactosemia study today, December 9, 2020 at 8:30 a.m. ET. The live event will be available on the investor page of the Applied Therapeutics website at https://ir.appliedtherapeutics.com/ or by calling (800) 369-8554 (toll-free domestic) or (409) 937-8917 (international) five minutes prior to the start time and entering passcode 9978322. A replay of the call will be available on the Applied Therapeutics website approximately two hours after the completion of the call and will be archived for 30 days.

About Galactosemia
Galactosemia is a rare, slowly progressing metabolic disease caused by a genetic inability to break down the sugar galactose. Aldose Reductase (AR), an enzyme known to play a role in many diseases including Galactosemia, converts galactose into galactitol, a toxic metabolite that builds up in tissues and organs and can cause long-term disease complications. There are approximately 3,000 individuals with Galactosemia in the U.S. and about 3,500 individuals in the E.U. Most patients with Galactosemia are under the age 40, as newborn screening was not widely adopted until the 1980s. Galactosemia is now included as part of routine newborn screening in all 50 U.S. states, and in many countries in Europe.

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

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Forward-Looking Statements

This press release may contain “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. These include, without limitation, statements regarding (i) the timing of our NDA submission for potential approval of AT-007, which will include data from the ACTION-Galactosemia Kids trial and the 90-day safety data in adults with Galactosemia, (ii) the design, scope and results of our clinical trials, (iii) the timing of the initiation and completion of our clinical trials, (iv) the likelihood that data from our clinical trials will support future development of our product candidates, and (v) the likelihood of obtaining regulatory approval of our product candidates and qualifying

for any special designations, such as orphan drug designation. 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, (i) our plans to develop and commercialize our product candidates, (ii) the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and our research and development programs, (iii) our ability to take advantage of expedited regulatory pathways for any of our product candidates, (iv) our estimates regarding expenses, future revenue, capital requirements and needs for additional financing, (v) our ability to successfully acquire or license additional product candidates on reasonable terms, (vi) our ability to maintain and establish collaborations or obtain additional funding, (vii) our ability to obtain regulatory approval of our current and future product candidates, (viii) our expectations regarding the potential market size and the rate and degree of market acceptance of such product candidates, (ix) our ability to fund our working capital requirements and expectations regarding the sufficiency of our capital resources, (x) the implementation of our business model and strategic plans for our business and product candidates, (xi) our intellectual property position and the duration of our patent rights, (xii) developments or disputes concerning our intellectual property or other proprietary rights, (xiii) our expectations regarding government and third-party payor coverage and reimbursement, (xiv) our ability to compete in the markets we serve, (xv) the impact of government laws and regulations and liabilities thereunder, (xvi) developments relating to our competitors and our industry, (xvii) the impact of the COVID-19 pandemic on the timing and progress of our ongoing clinical trials and our business in general and (xviii) other factors that may impact our financial results. In light of the significant uncertainties in these forward-looking statements, you should not rely upon forward-looking statements as predictions of future events. Although we believe that we have a reasonable basis for each forward-looking statement contained in this press release, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur at all. 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.