Applied Therapeutics Reports Additional Pediatric Biomarker Data from ACTION-Galactosemia Kids

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Substantial and statistically significant reduction in plasma galactitol of 40% with new weight-based dosing parameters

Baseline galactitol levels clearly correlated with baseline clinical functional outcomes

NEW YORK, Oct. 18, 2021 (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 reported additional biomarker data from the pediatric ACTION-Galactosemia Kids study.

As previously announced, a pharmacokinetic analysis of AT-007 plasma drug levels at day 30 revealed that pediatric dosing could be further optimized by adjusting dose based on weight rather than age. As such, weight-based dosing brackets were implemented, and a subset of pediatric patients were dose-adjusted for an additional 30 days.

A new data analysis was performed on galactitol reduction across the study, including data from children who received a dose adjustment. AT-007 substantially reduced plasma galactitol by approximately 40%, which was statistically significant ($p<0.001$) vs. placebo. Reduction in plasma galactitol was rapid and sustained, with no impact on levels of galactose or Gal-1p, and was similar across dose groups. AT-007 was safe and well tolerated in children of all ages (2-17). The Company plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in Q4 of this year.

As previously announced, an analysis of the 47 children in the ACTION-Galactosemia Kids study demonstrated a clear correlation between baseline galactitol level and baseline clinical functional outcomes. Children with higher plasma galactitol levels displayed greater disease severity vs. children with lower plasma galactitol levels at baseline. This data will be presented as a late-breaking abstract at the 14th International Congress on Inborn Errors of Metabolism (ICIEM) November 21-24, 2021.

“We have demonstrated that toxic galactitol drives disease progression and greatly impacts quality of life in Galactosemia patients,” said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics. “Here we have shown that AT-007 significantly reduces galactitol levels, which we believe could transform the lives of children and adults living with this disease. We are sincerely thankful to all of the families participating in the ACTION-Galactosemia clinical program, who have made this study possible.”

“The Commercial team has made great progress in preparing for a possible launch of AT-007 in Galactosemia. Our ‘Galactosemia Together’ disease state education campaign has generated tremendous interest from the physician and patient community and our website galactosemia.com has won numerous industry awards. We look forward to potentially bringing AT-007 to these patients who are in desperate need of a treatment option,” said Adam Hansard, Chief Commercial Officer of Applied Therapeutics.

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. 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 timing of our rare disease franchise expansion programs in SORD Deficiency, (iii) the timing of the initiation and completion of our clinical trials, including ARISE-HF, (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. 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.

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