Applied Therapeutics Provides Regulatory Update on Galactosemia Program

January 3, 2022

NEW YORK, Jan. 03, 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 provided a regulatory update on the AT-007 Galactosemia program.

Following discussions with the FDA at the end of the year, the Company has decided to hold on submitting an NDA for AT-007 for treatment of Galactosemia pending additional discussions with the agency. Although the Galactosemia program had previously been discussed in the context of an NDA for Accelerated Approval based on reduction in galactitol, the FDA has now indicated that clinical outcomes data will likely be required for approval.

The ongoing ACTION-Galactosemia Kids Phase 3 study is evaluating the impact of AT-007 treatment vs. placebo on clinical outcomes over time, including cognition, speech, behavior and motor skills. Clinical outcomes are assessed every 6 months by a firewalled committee. The first assessment will be completed in the first quarter of 2022, and then every 6 months thereafter until the study reaches statistical significance.

“While disappointed by this change in direction by the FDA, we remain committed to bringing this important treatment to patients with Galactosemia,” said Shoshana Shendelman, PhD, CEO, Founder and Chair of the Board of Applied Therapeutics. “We will continue to work with the FDA to determine the most expeditious path forward to regulatory approval and will provide an update on timing and plans accordingly.”

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. A copy of the Company’s January 2022 Corporate Presentation will be posted to the Investor Relations section of Applied Therapeutics’ website.

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 open-label trial in adults with Galactosemia, (ii) the timing of the initiation and completion of our clinical trials, including the ongoing ACTION-Galactosemia Kids Phase 3 study, (iii) the likelihood that data from our clinical trials will support future development of our product candidates and (iv) 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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