Applied Therapeutics Announces Restart of Pediatric Galactosemia Study

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FDA Clinical Hold Lifted

NEW YORK, Feb. 01, 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 announced that the FDA has lifted the hold and the AT-007 ACTION-Galactosemia Kids pediatric clinical study will resume effective immediately. Applied Therapeutics has worked closely with FDA to modify the trial, with the shared goal of ensuring that all patients have the opportunity to receive clinical benefit.

Previously, the ACTION-Galactosemia Kids program was split into two separate clinical studies – a dose escalation and biomarker study followed by a separate long-term clinical outcomes study. The two studies have now been combined into a single two-part study to ensure that all patients who complete the dose escalation and biomarker portion of the study will seamlessly continue into the long-term outcomes study without treatment interruption. Additionally, the dose-escalation portion of the study has been operationally modified to ensure continuous drug treatment and participation throughout the study. All patients who were already randomized and participating in the ACTION-Galactosemia Kids study will be eligible to return to the study, and the Company anticipates being fully enrolled within a matter of weeks. The Company remains on target to submit an NDA no later than Q3 2021.

"We thank the Galactosemia community for their patience and support, and we are grateful to the FDA for their partnership," said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. "We believe that the program is in a stronger position for overall success and potential approval due to our close collaboration with FDA. AT-007 represents an important advancement for Galactosemia patients, and offering a therapeutic option to children is a priority for Applied Therapeutics."

About AT-007

AT-007 is a central nervous system (CNS) penetrant Aldose Reductase inhibitor (ARI) in clinical development for treatment of Galactosemia. AT-007 has been studied in an animal model of Galactosemia, which demonstrated that AT-007 reduces toxic galactitol levels and prevents disease complications. AT-007 was also studied in an adult biomarker-based clinical study and demonstrated significant reduction in plasma galactitol levels vs. placebo; the long-term extension in adults remains ongoing. The company received Orphan Designation for AT-007 for Galactosemia in May 2019 and Pediatric Rare Disease Voucher (PRV) designation in 2020.

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.

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