Applied Therapeutics Announces FDA Orphan Drug Designation for AT-007 in Galactosemia

May 28, 2019

NEW YORK, May 28, 2019 (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 U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to AT-007, a central nervous system (CNS) penetrant Aldose Reductase inhibitor (ARI) in the late stages of preclinical development for the treatment of Galactosemia.

"We are pleased to obtain Orphan Drug Designation for AT-007 in Galactosemia, a devastating rare metabolic disease for which there are no approved treatments," said Shoshana Shendelman, PhD, Founder, Chief Executive Officer and Chair of the Board of Applied Therapeutics. "Galactosemia patients are in desperate need of treatment options, and we are committed to advancing AT-007 into clinical development as quickly as possible due to the urgent unmet medical need. We look forward to taking advantage of the opportunities that Orphan Designation provides in order to bring this potential new treatment option to patients and families as rapidly as possible." Applied Therapeutics plans to initiate a Phase 1/2 biomarker based clinical study with AT-007 in Galactosemia patients later this year.

The FDA Office of Orphan Products Development grants Orphan Drug Designation to drugs and biologics that are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the United States. The designation allows Applied Therapeutics to qualify for a number of incentives, including: seven years of market exclusivity upon regulatory approval, if received; exemption from FDA application fees for Galactosemia; and tax credits for qualified clinical trials.

About Galactosemia
Galactosemia is a devastating rare pediatric metabolic disease that affects how the body processes a simple sugar called galactose, and for which there is no known cure or approved treatment available. Galactose is found in foods, but the human body also naturally produces galactose on its own, so dietary restriction can't prevent complications of disease. It is estimated that the U.S. Galactosemia population is approximately 2,800 patients, based on newborn screening data identifying 2,500 infants through 2014, and the estimated birth rate of 80 patients per year. High levels of galactose circulating in the blood and tissues of Galactosemia patients enables Aldose Reductase to convert galactose to a toxic metabolite, called galactitol, which causes long-term complications ranging from CNS dysfunction to cataracts.

About AT-007
AT-007 is a central nervous system (CNS) penetrant Aldose Reductase inhibitor (ARI) in late-stage preclinical 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. Applied Therapeutics is planning a biomarker-based development program in patients with Galactosemia, based on the recently released draft industry guidance on drug development for low prevalence, slowly progressing rare metabolic diseases. The company intends to advance AT-007 into a Phase 1/2 clinical trial in 2019.

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-001, is a novel Aldose Reductase inhibitor (ARI) that is being developed for the treatment of Diabetic Cardiomyopathy (DbCM), a fatal fibrosis of the heart. The company plans to initiate a Phase 2/3 pivotal study in DbCM in 2019. Applied Therapeutics is also developing AT-007, a central nervous system (CNS) penetrant ARI, for the treatment of Galactosemia, a rare pediatric metabolic disease, which is expected to advance into a Phase 1 clinical trial in 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, expected to advance into a Phase 1 study in 2020.

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 our ability to rapidly advance the clinical development of our product candidates, including AT-007, and obtain approval of such product candidates, the likelihood data will support future development and the expected timing of initiation of our clinical trials. 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, the uncertainties inherent in the initiation, execution and completion of clinical trials, in the timing of availability of trial data, in the results of the clinical trials, in the actions of regulatory agencies, in the commercialization and acceptance of new therapies. Other 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 section titled "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.

Contacts
Investors:
Maeve Conneighton
(212) 600-1902 or

appliedtherapeutics@argotpartners.com

Media:
media@appliedtherapeutics.com

Source: Applied Therapeutics