Applied Therapeutics Provides Regulatory Update on Galactosemia Program

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Pre-NDA Meeting to be held with FDA this summer to discuss potential NDA submission for govorestat (AT-007) for treatment of Classic Galactosemia

EMA Marketing Authorization Application for govorestat for the treatment of Classic Galactosemia planned in Fall 2023

NEW YORK, July 25, 2023 (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 Galactosemia program, including updates on the regulatory paths for govorestat (AT-007) in both the U.S. and Europe.

The United States Food and Drug Administration (US FDA) has granted a Pre-New Drug Application (Pre-NDA) meeting to be held this summer to discuss a potential NDA submission for govorestat (AT-007) for the treatment of Galactosemia. The Company believes that the clinical efficacy demonstrated to date, combined with galactitol biomarker data and a favorable safety profile, may support an NDA submission, and is seeking feedback from the FDA and alignment on the details of the submission. If the FDA is in agreement on the potential path forward to approval, the Company will plan to submit an NDA in the fall. The Company expects to provide a further update following the meeting.

Regarding regulatory submission plans in Europe, Applied Therapeutics and its European commercial partner, Advanz Pharma, met with the European Medicines Agency (EMA) rapporteurs earlier this summer, and plan to proceed with an EMA Marketing Authorization Application (MAA) submission as expeditiously as possible. The EMA submission is anticipated in the fall in order to provide sufficient time for approval of the Pediatric Investigational Plan (PIP), as well as incorporation of rapporteur comments and suggestions from the meeting.

"We look forward to advancing our regulatory submissions in both the U.S. and Europe," said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. "We believe that govorestat offers compelling efficacy alongside a favorable safety profile and represents a transformative treatment option for patients with Galactosemia. Galactosemia is a devastating disease that progressively worsens with age, despite adherence to a galactose-restricted diet, due to endogenous production of galactose by the body and subsequent conversion to the toxic metabolite, galactitol. There are currently no approved treatments for Galactosemia, and we hope to bring govorestat to patients as the first treatment for Galactosemia as soon as possible."

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, and approximately 4,000 patients with Galactosemia in the EU and 120 new births per year.

About Govorestat (AT-007)
Govorestat 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 a study in children with Galactosemia aged 2-17, treatment with AT-007 demonstrated clinical benefit on activities of daily living, behavioral symptoms, cognition, fine motor skills and tremor. Govorestat also significantly reduced plasma galactitol levels in both adults and children with Galactosemia. Galactitol is a toxic metabolite responsible for tissue damage and long-term complications in Galactosemia.

Govorestat is also being studied in the ongoing Phase 3 INSPIRE trial, which is evaluating the effect of AT-007 vs. placebo in patients with SORD Deficiency on sorbitol reduction as well as clinical outcomes in approximately 50 patients aged 16-55 in the U.S. and Europe. In an interim analysis, AT-007 reduced sorbitol by a mean of 52%, or approximately 16,000 ng/ml, over a 90-day period, which was highly statistically significant vs. placebo ($p<0.001$).

Govorestat has received Orphan Medicinal Product Designation from the European Medicines Agency (EMA)EMA for both Galactosemia and SORD Deficiency. Govorestat has also received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) for the treatment of Galactosemia, PMM2-CDG, and SORD Deficiency; Pediatric Rare Disease designation for 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, govorestat, 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.

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. Forward-looking statements in this release, including those about our upcoming pre-NDA meeting, the potential NDA submission, including the timing thereof and the timing for our MAA submission, 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, 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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