Applied Therapeutics Provides Regulatory Update on Govorestat for the Treatment of Classic Galactosemia

September 18, 2024

- Company completed Late-Cycle review meeting with FDA

- FDA no longer intends to hold Advisory Committee meeting

- FDA Review of NDA continues to proceed as planned with PDUFA target action date of November 28, 2024

NEW YORK, Sept. 18, 2024 (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 an update on the ongoing New Drug Application (NDA) review of govorestat for the treatment of Classic Galactosemia. The Company recently completed its late-cycle review meeting with the United States Food and Drug Administration (FDA). The FDA communicated that an Advisory Committee meeting would no longer be required, which was previously tentatively scheduled for October 9, 2024. The FDA informed the Company that the Priority Review of the NDA is continuing as planned with alignment on post-marketing requirements expected in October 2024. The previously announced Prescription Drug User Fee Act (PDUFA) target action date remains on track for November 28, 2024.

"We are incredibly pleased by the ongoing collaborative dialogue with the FDA during the NDA review process, and we look forward to continuing to work together with the agency to bring the first potential treatment to Classic Galactosemia patients," said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. "Galactosemia is a progressive disease in urgent need of treatment, and the potential approval of govorestat will be transformative for the many patients and families living with this serious disease. Our commitment to the Classic Galactosemia community is further supported by our thoughtful commercial preparation, focused on establishing an effective patient access program, high physician awareness and strong payor engagement."

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. Newborn screening for Galactosemia is mandatory in the US and most EU countries, leading to rapid identification of affected patients.

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 govorestat 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.

In the Phase 3 INSPIRE trial in patients with SORD Deficiency, an interim analysis at 12 months demonstrated a statistically significant reduction in blood sorbitol levels with govorestat treatment as compared to placebo, as well as a statistically significant correlation of sorbitol with change in clinical outcomes, such as 10-meter walk run test, dorsiflexion and 6-minute walk test. Govorestat also demonstrated highly statistically significant effects on the CMT Health Index (CMT-HI) patient reported outcome measure, with benefit of govorestat shown on lower limb function, mobility, fatigue, pain, sensory function, and upper limb function.

Govorestat has received Orphan Medicinal Product Designation from the European Medicines Agency (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 the strategy, future operations, prospects, plans and objectives of management, including words such as “may,” “will,” “expect,” “anticipate,” “plan,” “intend,” “predicts” 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 likelihood that the Company’s ongoing NDA and MMA submissions will be approved and the timing of any decision and (ii) statements related to the scheduling or timing of any potential FDA or EMA meetings, interactions or submissions. 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, market 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 and advance product candidates into, and successfully complete, clinical studies, (vi) our ability to maintain and establish collaborations or obtain additional funding, (vii) our ability to obtain and timing of regulatory approval of our current and future product candidates, (viii) the anticipated indications for our product candidates, if approved, (ix) our expectations regarding the potential market size and the rate and degree of market acceptance of such product candidates, (x) our ability to fund our working capital requirements and expectations regarding the sufficiency of our capital resources, (xi) the implementation of our business model and strategic plans for our business and product candidates, (xii) our intellectual property position and the duration of our patent rights, (xiii) developments or disputes concerning our intellectual property or other proprietary rights, (xiv) our expectations regarding government and third-party payor coverage and reimbursement, (xv) our ability to compete in the markets we serve, (xvi) the impact of government laws and regulations and liabilities thereunder, (xvii) developments relating to our competitors and our industry, (xviii) our ability to achieve the anticipated benefits from the agreements entered into in connection with our partnership with Advanz Pharma and (xiv) 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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