Applied Therapeutics Announces MAA Validation and NDA Submission of Govorestat (AT-007) for Treatment of Classic Galactosemia

January 3, 2024

NDA submitted to FDA and MAA validated by EMA in December 2023

Submissions are based on positive results from Phase 3 registrational ACTION-Galactosemia Kids study demonstrating consistent long-term clinical outcomes benefit across a range of functional measures and favorable safety profile

NEW YORK, Jan. 03, 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 it has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for govorestat (AT-007) for the treatment of Classic Galactosemia. The NDA was submitted in December 2023. In addition, the Company submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in the fourth quarter of 2023, which was subsequently validated and accepted for review in December 2023.

“The submissions of both the NDA and MAA for govorestat are supported by rapid and sustained reduction in galactitol, which resulted in a meaningful benefit on clinical outcomes across pediatric patients, alongside a favorable safety profile,” said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. “We look forward to working closely with both regulatory agencies throughout the review process and hope to bring the first treatment to patients with Galactosemia soon.”

“There are currently no treatments available for Galactosemia, a serious progressive disease affecting over 3,000 patients in the United States,” said Brittany Cudzilo, Vice President of the Galactosemia Foundation. “Govorestat offers hope to many patients and families affected by Galactosemia. I saw the positive impact of govorestat first-hand in my daughter, Ansell, who participated in the ACTION-Galactosemia Kids pediatric study. As a community, we will do everything possible to ensure that the regulatory agencies understand the importance of approving this treatment for our loved ones with Galactosemia.”

The NDA and MAA submission packages include clinical outcomes data from the Phase 3 registrational ACTION-Galactosemia Kids study in children age 2-17 with Galactosemia, the Phase 1/2 ACTION-Galactosemia study in adult patients with Galactosemia, and preclinical data. The FDA has a 60-day filing review period to determine whether the NDA is complete and accepted for review. The MAA has been validated and will move to review by the EMA’s Committee for Medicinal Products for Human Use (CHMP).

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 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) 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. 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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