Applied Therapeutics Provides FDA Update on PDUFA Target Action Date for Govorestat for the Treatment of Classic Galactosemia

March 28, 2024

NEW YORK, March 28, 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 that the U.S. Food and Drug Administration (FDA) has extended the review period for the New Drug Application (NDA) for govorestat (AT-007) for the treatment of Classic Galactosemia by three months. The FDA has set a new Prescription Drug User Fee Act (PDUFA) target action date of November 28, 2024.

The FDA notified Applied Therapeutics that it required additional time to review supplemental analyses of previously submitted data that had been provided by Applied in response to the FDA's routine information requests and determined that the additional information constitutes a Major Amendment to the NDA. In February 2024, the Company announced that the FDA accepted and granted Priority Review to the NDA. Govorestat was previously granted Pediatric Rare Disease designation, and will qualify for a Priority Review Voucher (PRV) upon approval.

"While the PDUFA action date extension represents a delay, we remain confident in the potential for govorestat approval for Galactosemia and we will continue to work closely with the FDA throughout the review process," said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. "During this time, we are committed to maintaining the expanded access program for govorestat to ensure patients with Galactosemia have the opportunity to receive this important treatment."

Govorestat is an investigational, novel Aldose Reductase Inhibitor (ARI) being developed for the treatment of several rare diseases. The NDA filing of govorestat is 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. The submission package included clinical outcomes data from the Phase 3 registrational ACTION-Galactosemia Kids study in children aged 2-17 with Galactosemia, the Phase 1/2 ACTION-Galactosemia study in adult patients with Galactosemia, and preclinical data.

If approved, govorestat would be the first medication indicated for the treatment of Galactosemia and would be Applied Therapeutics' first commercial product. The Company has also submitted a Marketing Authorization Application (MAA) for govorestat for the treatment of Classic Galactosemia to the EMA, which was validated in December 2023 and is under review by the EMA's Committee for Medicinal Products for Human Use (CHMP). The Company expects a decision by the EMA in the fourth quarter of 2024.

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.

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 12 months, govorestat achieved statistical significance on the prespecified primary endpoint of correlation of sorbitol with the clinical outcome composite and demonstrated sustained, statistically significant reduction in sorbitol level vs. placebo. Govorestat also demonstrated highly statistically significant effects on the CMT Health Index (CMT-HI) patient reported outcome measure, with benefit of govorestat on categories of 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 the likelihood that the Company’s ongoing NDA and MMA submissions will be approved and the timing of any approval decision. 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 (xix) 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.

Contacts

Investors:
Maeve Conneighton
(212) 600-1902 or
appliedtherapeutics@argotpartners.com

Media:
media@apliedtherapeutics.com