Applied Therapeutics Granted Fast Track Designation by FDA for AT-007 for Galactosemia

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NEW YORK, June 17, 2021 (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 Fast Track designation to AT-007 for the treatment of Galactosemia, a rare metabolic disease. AT-007 is the Company's central nervous system-penetrant Aldose Reductase inhibitor in development for multiple rare metabolic disorders, including Galactosemia, SORD and PMM2-CDG.

Fast Track designation is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fulfill an unmet medical need, enabling drugs to reach patients earlier. Clinical programs with Fast Track designation may benefit from early and frequent communication with the FDA throughout the regulatory review process. These clinical programs may also be eligible to apply for Accelerated Approval and Priority Review if relevant criteria are met. Applied Therapeutics plans to submit an NDA for Accelerated Approval of AT-007 for the treatment of Galactosemia in the third quarter of this year. FDA has previously granted Orphan Drug Designation and Pediatric Rare Disease status to AT-007 for Galactosemia.

"Galactosemia is a devastating rare metabolic disease that progressively worsens over time and greatly impacts patient quality of life," said Shoshana Shendelman PhD, Founder and CEO of Applied Therapeutics. "There are currently no drugs approved for Galactosemia, and we believe AT-007 has the potential to be the first drug approved for patients urgently in need of treatment. Fast Track Designation offers several important opportunities to work closely with the FDA through the review and approval process to ensure that AT-007 is available to patients as quickly as possible."

About AT-007

AT-007 is a central nervous system (CNS) penetrant Aldose Reductase inhibitor (ARI) in development for the treatment of several rare diseases. In an animal model of Galactosemia, AT-007 reduced toxic galactitol levels and prevented disease complications. AT-007 significantly reduced plasma galactitol levels vs. placebo in adults with Galactosemia in a Phase 2 registrational trial; the long-term extension in adults remains ongoing. AT-007 is currently being studied in a registrational trial in children ages 2-17 with Galactosemia. AT-007 is also in clinical development for the treatment of SORD Deficiency, a rare progressive hereditary neuropathy, and in development for the treatment of Phosphomannomutase 2 Deficiency (PMM2-CDG), a congenital disorder of glycosylation. AT-007 has received both Orphan Drug and Pediatric Rare Disease designations from the U.S. Food and Drug Administration (FDA) for the treatment of 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, AT-007, 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, as well as novel dual PI3k inhibitors in preclinical development for orphan oncology indications.

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 (i) the timing of our NDA submission for potential approval of AT-007, which will include data from the ACTION-Galactosemia Kids trial and the 90-day safety data in adults with Galactosemia, (ii) the timing of our rare disease franchise expansion programs in SORD Deficiency and PMM2-CDG, (iii) the timing of the initiation and completion of our clinical trials, (iv) the likelihood that data from our clinical trials will support future development of our product candidates and (v) the likelihood of obtaining regulatory approval of our product candidates. 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 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, (vi) our ability to maintain and establish collaborations or obtain additional funding, (vii) our ability to obtain regulatory approval of our current and future product candidates, (viii) our expectations regarding the potential market size and the rate and degree of market acceptance of such product candidates, (ix) our ability to fund our working capital requirements and expectations regarding the sufficiency of our capital resources, (x) the implementation of our business model and strategic plans for our business and product candidates, (xi) our intellectual property position and the duration of our patent rights, (xii) developments or disputes concerning our intellectual property or other proprietary rights, (xiii) our expectations regarding government and third-party payor coverage and reimbursement, (xiv) our ability to compete in the markets we serve, (xv) the impact of government laws and regulations and liabilities thereunder, (xvi) developments relating to our competitors and our industry, (xvii) the impact of the

COVID-19 pandemic on the timing and progress of our ongoing clinical trials and our business in general and (xviii) 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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