# Applied Therapeutics Reports Biomarker Data from Pilot Trial of AT-007 in SORD Deficiency

October 25, 2021

*Substantial and significant reduction in sorbitol*

*Company plans to initiate registrational trial by end of 2021*

*Company to host conference call and webcast today at 8:30 a.m. ET*

NEW YORK, Oct. 25, 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 reported biomarker data from a pilot trial of AT-007 in patients with SORD Deficiency.

Sorbitol Dehydrogenase Deficiency (SORD Deficiency) is a rare, progressive, debilitating hereditary neuropathy that affects peripheral nerves and motor neurons. SORD Deficiency affects approximately 3,300 patients in the US and 4,000 patients in Europe. The disease is caused by a lack of the enzyme sorbitol dehydrogenase, responsible for metabolism of sorbitol, which causes sorbitol to accumulate at high levels and become toxic to the body. Sorbitol accumulation results in significant disability, loss of sensory function, and neuromuscular dysfunction.

Patients with SORD Deficiency have 100 times higher sorbitol concentration in their blood compared with unaffected individuals. In a pilot open-label study in 8 SORD Deficiency patients, AT-007 reduced blood sorbitol levels by approximately 66% from baseline through 30 days of treatment. The range of reduction from baseline in patients was 54%-75%. AT-007 was safe and well tolerated in all treated patients.

These results, in addition to preclinical findings, demonstrate that AT-007 has the potential to be the first disease-modifying therapy for SORD Deficiency. The Company plans to initiate a registrational study by the end of 2021. In advance of the registrational study start, patients can now pre-screen to determine whether they have SORD and if they may qualify for the upcoming trial.

“Reduction in toxic sorbitol is critically important in patients with SORD Deficiency. This data demonstrates a significant effect on the underlying cause of the disease,” said Michael Shy, MD, Professor of Neurology and Director of the Division of Neuromuscular Medicine at the University of Iowa Hospital Carver School of Medicine.

“AT-007 represents an important advancement for patients with SORD Deficiency, and a unique opportunity to meaningfully impact patients’ lives. We look forward to beginning our registrational trial for this indication in the coming months,” said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics.

Increased access to screening and early diagnosis can dramatically improve patients’ lives, and the Company is collaborating with the Charcot Marie Tooth Association and the Hereditary Neuropathy Foundation to improve access to SORD diagnostic testing, and to better understand the perspectives of individuals living with SORD.

**Conference Call Information**

Applied Therapeutics will host a conference call today, Monday, October 25, 2021, at 8:30 a.m. Eastern Time, to discuss data from a pilot trial of AT-007 in SORD deficiency. To access the conference call, please dial (800) 369-8554 (local) or (409) 937-8917 (international) at least 10 minutes prior to the start time and refer to conference ID 2437605. A live webcast of the call will be accessible on the Events page under the Investor Relations section of the Applied Therapeutics website at www.appliedtherapeutics.com. A replay will be available on the Company’s website approximately two hours after the event.

**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.

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. These include, without limitation, statements regarding (i) the Company’s plan to initiate a registrational study by the end of 2021, (ii) AT-007 potential to be the first disease-modifying therapy for SORD Deficiency, (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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