# Applied Therapeutics to Present Data on AT-007 (Govorestat) Treatment in SORD Deficiency at the 2023 Annual Meeting of the Peripheral Nerve Society

June 15, 2023

- *Data further elucidates the pathophysiology of sorbitol toxicity, including sorbitol accumulation as the driver of disease in patient-derived motor neurons, as well as in the drosophila model and a new rat model of SORD Deficiency*
- *Clinical study data includes trial design, baseline data and 3-month sorbitol reduction from the Phase 3 INSPIRE study in patients with SORD Deficiency*

NEW YORK, June 15, 2023 (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 multiple oral presentations at the 2023 Annual Meeting of the Peripheral Nerve Society to take place June 17 - 20 in Copenhagen, Denmark.

**Presentation Details**

**The INSPIRE Study: A Randomized Study to Evaluate Pharmacodynamic and Clinical Benefit of AT-007 in Patients with Sorbitol Dehydrogenase Deficiency**
*Michael Shy, Andrea Cortese, Peter Creigh, Vera Fridman, Michael Hassman, David Hermann, Radim Mazanec, Davide Pareyson, Riccardo Perfetti, Mary Reilly, Lemuel Rivera Fuentes, Mario Saporta, Steven Scherer, Pavel Seeman, Reza Sadjadi, Shoshana Shendelman*

Oral Presentation by Michael Shy, MD: Monday, June 19, 11:50 AM CEST
Poster P-172, Abstract 1277: Monday, June 19, 6:15 – 7:25PM CEST

**Sorbitol Reduction via AT-007 Prevents Synaptic Dysfunction and Neurodegeneration in Models of Sorbitol Dehydrogenase Deficiency**
*Yi Zhu, Amanda Lobato, Adriana Rebelo, Tijana Canic, Natalie Ortiz-Vega, Xianzun Tao, Sheyum Syed, Christopher Yanick, Mario Saporta, Michael Shy, Riccardo Perfetti, Shoshana Shendelman, PhD, Stephan Zuchner, Grace Zhai*

Oral Presentation by Amanda Lobato: Monday, June 19, 12:35 PM CEST
Poster P-112, Abstract 1159: Monday, June 19, 6:15 – 7:25PM CEST

**Sorbitol Dehydrogenase Deficiency in Rats Results in a Motor-Dominant Peripheral Neuropathy**
*Adriana P Rebelo, Clemer Abad, Maike Dohrn, Jian Li, Steven Scherer, Juan Young, Katherina Walz, Stephan Zuchner*

Oral Presentation by Adriana Rebelo, PhD: Tuesday, June 20, 9:30 AM CEST

**About Sorbitol Dehydrogenase (SORD) Deficiency**

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

**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 US 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 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. 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, 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
appliedtherapeutics@argotpartners.com

**Media:**
media@appliedtherapeutics.com

Applied Therapeutics, Inc.