Applied Therapeutics Reports Fourth Quarter and Year-end 2023 Financial Results

March 6, 2024

- *NDA for govorestat accepted and granted Priority Review by FDA for the treatment of Classic Galactosemia; PDUFA target action date of August 28, 2024; MAA under review by EMA with decision expected in 4Q 2024*
- *Announced positive results from 12-month interim analysis of govorestat in ongoing INSPIRE Phase 3 trial in SORD Deficiency; Company plans to request pre-NDA meeting with neurology division of FDA*
- *Strengthened balance sheet with $100 million Private Placement, expected to extend cash runway into 2026*

NEW YORK, March 06, 2024 (GLOBE NEWSWIRE) -- Applied Therapeutics, Inc. (Nasdaq: APLT) (the "Company"), 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 financial results for the fourth quarter and full year ended December 31, 2023.

"We've made significant clinical and regulatory progress, particularly with the NDA acceptance and MAA validation for govorestat for the treatment of Galactosemia, achieving key milestones for our rare disease pipeline. Additionally, we believe that the recent positive data from the interim analysis of the INSPIRE study in SORD Deficiency confirms the role of sorbitol as a key driver of disease progression, and we plan to request a pre-NDA meeting with the FDA," said Shoshana Shendelman, PhD, Founder, Chief Executive Officer, and Chair of the Board. "As Applied enters into this next stage of growth, we are poised for continued value generation across our rare disease pipeline, supported by our recent financing and bolstered cash position."

Recent Highlights

- **Govorestat NDA Accepted and Granted Priority Review by US FDA for Treatment of Classic Galactosemia, PDUFA Target Action Date of August 28, 2024; MAA under CHMP Review by EMA.** In February 2024, the Company announced that the U.S. Food and Drug Administration (FDA) has accepted the filing of the New Drug Application (NDA) for govorestat (AT-007) for the treatment of Classic Galactosemia. The NDA was granted Priority Review status and the FDA assigned a Prescription Drug User Free Act (PDUFA) target action date of August 28, 2024. The FDA also noted that it is planning to hold an advisory committee meeting to discuss the application. Govorestat was previously granted Pediatric Rare Disease designation and will qualify for a Priority Review Voucher (PRV) upon approval. The Company has also submitted a Marketing Authorization Application (MAA) for govorestat for the treatment of Classic Galactosemia to the European Medicines Agency (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. The NDA and MAA submission packages include 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.

- **Entered into $100 Million Private Placement, Expected to Extend Cash Runway to 2026.** In February 2024, the Company entered into a securities purchase agreement for a private placement of $100 million of equity with participation from new and existing large, healthcare dedicated institutional and mutual fund investors including Perceptive Advisors, Janus Henderson Investors, Venrock Healthcare Capital Partners, Adage Capital Partners, Frazier Life Sciences, Logos Capital, Vestal Point Capital, and Rock Springs Capital. The Company intends to use the net proceeds to fund commercial activities for govorestat and to further develop other pipeline candidates, and for working capital and general corporate purposes. With cash of approximately $153.5 million as of March 1, 2024, the Company is well-capitalized with an expected runway into the first half of 2026.

- **Announced Positive Data from 12-Month Interim Analysis of Govorestat (AT-007) in Ongoing INSPIRE Phase 3 Trial in Sorbitol Dehydrogenase Deficiency.** In February 2024, the Company announced that govorestat met primary and several key secondary endpoints in a pre-specified 12-month interim analysis of the ongoing INSPIRE Phase 3 Trial in Sorbitol Dehydrogenase (SORD) Deficiency. The objective of this pre-specified, 12-month interim analysis was to evaluate trends in clinical outcome measures and biomarker correlations in order to inform future regulatory discussions and support a potential NDA submission, due to the urgent need for treatment and absence of any other options for patients with SORD Deficiency. At the time of the interim analysis, a statistically significant correlation between sorbitol levels and the prespecified CMT-FOM composite clinical endpoint (10-meter walk-run test, 4 stair climb, sit to stand test, 6-minute walk test and dorsiflexion) was observed at 12 months of treatment with govorestat (p=0.05). Govorestat treatment provided sustained reduction in sorbitol level in patients with SORD Deficiency over 12 months of treatment, which was statistically

significant compared to placebo (p<0.001). In addition, treatment with govorestat also resulted in a highly statistically significant effect (p=0.01) on the CMT Health Index (CMT-HI), an important patient-reported outcome measure of disease severity and well-being and secondary endpoint in the study. Aspects of the CMT-HI that demonstrated a treatment effect included lower limb function, mobility, fatigue, pain, sensory function, and upper limb function. Govorestat continues to be safe and well tolerated to-date. The Company believes the results from the 12-month interim analysis of the ongoing INSPIRE Phase 3 trial confirm the role of sorbitol as a key driver of disease severity and progression over time. Clinical outcomes of the ongoing INSPIRE trial are expected to be assessed again at 24 months, where the 10-meter walk run test serves as the primary clinical efficacy endpoint. The Company plans to discuss a potential NDA submission with the FDA based on the clinical data to date. The ongoing INSPIRE trial is a Phase 3 double-blind placebo-controlled registrational study evaluating the effect of once-daily oral govorestat in approximately 50 patients aged 16-55 with SORD Deficiency in the US and Europe.

- **Announced Topline Results from ARISE-HF Phase 3 Study of AT-001 (caficrestat) in Diabetic Cardiomyopathy.** In January 2024, the Company announced topline data from its Phase 3 Study of AT-001 in patients with diabetic cardiomyopathy (DbCM). AT-001 1500mg twice daily (BID) was generally safe and well tolerated, and demonstrated a strong trend in stabilizing cardiac functional capacity. In a pre-specified subgroup analysis of patients not concomitantly treated with SGLT2 or GLP-1 therapies, active treatment with AT-001 resulted in a statistically significant difference in the primary endpoint, and also resulted in a substantially lower rate of clinically significant worsening in cardiac functional capacity of 6% or more as compared to placebo. Full study results will be presented at an upcoming medical conference, along with results of the Diabetic Peripheral Neuropathy sub-study, which are still being analyzed. As a result of the encouraging ARISE-HF Phase 3 data, the Company plans to focus on identifying an appropriate path forward in order to bring AT-001 to DbCM patients.

Financial Results

- **Cash and cash equivalents and short-term investments** totaled $49.9 million as of December 31, 2023, compared to $30.6 million as of December 31, 2022. On March 1, 2024, the Company completed the private placement of shares and pre-funded warrants to purchase common stock for gross proceeds of approximately $100 million (before deducing placement agent commissions and other offering expenses).

- **Research and development expenses** for the year ended December 31, 2023 were $53.9 million, compared to $55.6 million for the year ended December 31, 2022. The decrease of approximately $1.7 million was primarily related to decreased expenses related to contract research organization (CRO) costs, partially offset by an increase in drug manufacturing and formulation costs.

- **General and administrative expenses** were $20.6 million for the year ended December 31, 2023, compared to $27.3 million for the year ended December 31, 2022. The decrease of approximately $6.7 million was primarily related to a decrease in commercial expense of $2.1 million, an overall decrease in headcount resulting in a $3.0 million decrease in expense and reduced insurance expense of $1.4 million.

- **Net loss** for the year ended December 31, 2023 was $119.8 million, or $1.42 per basic and diluted common share, compared to a net loss of $82.5 million, or $2.18 per basic and diluted common share, for the year ended December 31, 2022.

- **Cash runway:** The Company expects that its cash and cash equivalents, combined with the proceeds from the February 2024 Private Placement and potential milestones expected from its Advanz European licensing partnership, will fund the business into 2026. Additionally, the sale of the PRV, which would be granted upon a potential Galactosemia NDA approval could substantially extend the Company's cash runway.

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 (i) Company’s expectation that its cash and cash equivalents will extend into 2026; (ii) Company’s plans to request a pre-NDA meeting with the FDA; and (iii) 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 (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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Applied Therapeutics, Inc.
Balance Sheets
(in thousands except share and per share data)

	As of December 31, 2023 (Unaudited)	As of December 31, 2022
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 49,898	$ 16,657
Investments	—	13,923
Current portion of security deposits and leasehold improvements	254	57
Prepaid expenses and other current assets	4,234	6,671
Total current assets	54,386	37,308
Noncurrent portion of security deposits and leasehold improvements	—	198
Operating lease right-of-use asset	447	857
TOTAL ASSETS	$ 54,833	$ 38,363
LIABILITIES AND STOCKHOLDERS’ (DEFICIT)/EQUITY		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 429	$ 477
Accounts payable	1,742	4,534
Accrued expenses and other current liabilities	15,286	14,756
Warrant liabilities	53,725	13,657
Total current liabilities	71,182	33,424
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	38	414
Clinical holdback - long-term portion	759	464
Total noncurrent liabilities	797	878

Total liabilities	71,979	34,302
STOCKHOLDERS' (DEFICIT)/EQUITY:		
Common stock, $0.0001 par value; 200,000,000 shares authorized as of December 31, 2023 and December 31, 2022; 84,869,832 shares issued and outstanding as of December 31, 2023 and 48,063,358 shares issued and outstanding as of December 31, 2022	8	5
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of December 31, 2023 and December 31, 2022; 0 shares issued and outstanding as of December 31, 2023 and December 31, 2022	—	—
Additional paid-in capital	451,432	352,828
Accumulated other comprehensive income	—	51
Accumulated deficit	(468,586)	(348,823)
Total stockholders' (deficit)/equity	(17,146)	4,061
TOTAL LIABILITIES AND STOCKHOLDERS' (DEFICIT)/EQUITY	$ 54,833	$ 38,363

Applied Therapeutics, Inc.
Statements of Operations
(in thousands except share and per share data)
(Unaudited)

	Year Ended December 31,	
	2023	**2022**
REVENUE:		
License revenue	$ 9,219	$ —
Research and development services revenue	774	—
Total revenue	9,993	—
COSTS AND EXPENSES:		
Research and development	53,905	55,634
General and administrative	20,623	27,316
Total operating expenses	74,528	82,950
LOSS FROM OPERATIONS	(64,535)	(82,950)
OTHER INCOME (EXPENSE), NET:		
Interest income	1,372	685
Change in fair value of warrant liabilities	(56,573)	(66)
Other expense	(27)	(177)
Total other income (expense), net	(55,228)	442
Net loss	$ (119,763)	$ (82,508)
Net loss per share attributable to common stockholders—basic and diluted	$ (1.42)	$ (2.18)
Weighted-average common stock outstanding—basic and diluted	84,244,494	37,825,431