Applied Therapeutics Reports Fourth Quarter and Year-end 2022 Financial Results

March 23, 2023

Progress in Three Phase 3 Trials in Areas of High Unmet Clinical Need, Including Positive Sorbitol Reduction Data from the Ongoing Phase 3 INSPIRE Trial in Sorbitol Dehydrogenase (SORD) Deficiency

On Track to Report Additional Phase 3 Data Across Multiple Programs in 2023

NEW YORK, March 23, 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 reported financial results for the fourth quarter and full year ended December 31, 2022.

“We are pleased with the clinical progress in 2022 across all three of our registrational Phase 3 programs, and we look forward to the data readouts in the year ahead,” said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. “Our recent deal with Advanz Pharma highlights our ability to realize value in our clinical programs, and we continue to evaluate other potential opportunities for value recognition.”

Recent Highlights

- **Announced Positive Sorbitol Reduction Data from the Ongoing Phase 3 INSPIRE Trial in Sorbitol Dehydrogenase (SORD) Deficiency.** In February 2023, the Company announced positive sorbitol reduction data from the ongoing global Phase 3 INSPIRE trial. The INSPIRE trial is a Phase 3 double-blind placebo-controlled registrational study evaluating the effect of once-daily oral AT-007 in approximately 50 patients age 16-55 with SORD Deficiency in the US and Europe. SORD Deficiency (also called SORD Neuropathy or CMT-SORD) is a debilitating hereditary axonal neuropathy caused by mutations in the Sorbitol Dehydrogenase gene, leading to an inability to metabolize the sugar sorbitol, and resulting in accumulation of high levels of toxic sorbitol, which causes motor neuron degeneration and loss of mobility and motility. AT-007 (govorestat) is a central nervous system penetrant Aldose Reductase Inhibitor, which blocks conversion of glucose to sorbitol, and has previously been shown to reduce sorbitol levels in an open-label pilot study in patients with SORD Deficiency. In a pre-specified interim analysis of the ongoing Phase 3 INSPIRE trial, AT-007 reduced sorbitol levels by a mean of approximately 52% (or approximately 16,000ng/ml) over 90 days of treatment ($p<0.001$ vs. placebo) in patients with SORD Deficiency.

- **Announced Partnership with Advanz Pharma for Commercialization of Govorestat in Europe.** In January 2023, the Company announced a partnership with Advanz Pharma, a pharmaceutical company with a strategic focus on commercialization of specialty, hospital, and rare disease medicines, for commercialization of govorestat in Europe. Under the terms of the agreement, the Company will receive certain near-term development milestone payments upon clinical trial completion and marketing authorization in Europe as well as commercial sales milestones, which in the aggregate amount to over €130 million, including €10 million upfront provided upon signing. The Company will receive royalties on any future net sales of govorestat in Europe of 20% and will continue to be responsible for the development, manufacturing and supply of govorestat.

Financial Results

- **Cash and cash equivalents and short-term investments** totaled $30.6 million as of December 31, 2022, compared with $80.8 million at December 31, 2021.

- **Research and development expenses** for the year ended December 31, 2022 were $55.6 million, compared to $62.6 million for the year ended December 31, 2021. The decrease of approximately $6.9 million was primarily related to a decrease in drug manufacturing and formulation expenses of $11.0 million primarily related to the completion and release of AT-001 and AT-007 drug product batches in the year ended December 31, 2021 and a decrease of regulatory and other expenses of $0.6 million primarily related to the University of Miami license fees recognized during the year ended December 31, 2021, which was offset by an increase in clinical and pre-clinical expense of $2.6 million, primarily related to the progression of the SORD Phase 2/3 registrational study, progression of the AT-007 ACTION-Galactosemia long-term extension adult study, and progression of the AT-007 ACTION-Galactosemia Kids pediatric registrational study; an increase in personnel expenses of $1.1 million due to the increase in headcount in support of our clinical program pipeline; and an increase in stock-based compensation of $0.9 million due to new stock option and restricted stock unit grants, offset by forfeitures of stock option and restricted stock unit grants.

- **General and administrative expenses** were $27.3 million for the year ended December 31, 2022, compared to $43.0 million for the year ended December 31, 2021. The decrease of approximately $15.7 million was primarily related to a decrease of $9.1 million related to decreased spend for commercial operations; a decrease in personnel expenses of $1.1 million and a decrease in stock-based compensation of $2.9 million due to a decrease in headcount; a decrease of insurance expenses of $0.7 million related to decreased directors and officers liability insurance costs; and a decrease in other expenses of $2.4 million, primarily relating to decreased costs of other office expenses, which was offset by an increase in professional and legal fees of $0.5 million due to higher external legal fees.

- **Net loss** for the year ended December 31, 2022 was $82.5 million, or $2.18 per basic and diluted common share, compared to a net loss of $105.6 million, or $4.12 per basic and diluted common share, for the year ended December 31, 2021.

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 (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," 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 anticipated benefits of the Company's partnership with Advanz Pharma and (ii) the expected development milestone payments. 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, (xvii) 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.

Statements of Operations

(in thousands except share and per share data)

	Year Ended December 31,	
	2022	2021
OPERATING EXPENSES:		
Research and development	$ 55,634	$ 62,570
General and administrative	27,316	43,048
Total operating expenses	82,950	105,618
LOSS FROM OPERATIONS	(82,950)	(105,618)
OTHER INCOME (EXPENSE), NET:		
Interest income	685	555
Change in fair value of warrant liabilities	(66)	—
Other expense	(177)	(521)
Total other income, net	442	34
Net loss	$ (82,508)	$ (105,584)
Net loss attributable to common stockholders—basic and diluted	$ (82,508)	$ (105,584)
Net loss per share attributable to common stockholders—basic and diluted	$ (2.18)	$ (4.12)
Weighted-average common stock outstanding—basic and diluted	37,825,431	25,598,181

Applied Therapeutics, Inc.

Balance Sheets

(in thousands except share and per share data)

	As of December 31, 2022	As of December 31, 2021
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 16,657	$ 53,888
Investments	13,923	26,935
Prepaid expenses and other current assets	6,728	7,571
Total current assets	37,308	88,394
Operating lease right-of-use asset	857	1,298
Security deposits and leasehold improvements	198	200
TOTAL ASSETS	$ 38,363	$ 89,892
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 477	$ 442
Accounts payable	4,534	9,461
Accrued expenses and other current liabilities	14,756	16,559
Warrant liability	13,657	—
Total current liabilities	33,424	26,462
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	414	891
Clinical holdback - long-term portion	464	—
Total noncurrent liabilities	878	891
Total liabilities	34,302	27,353
STOCKHOLDERS' EQUITY:		
Common stock, $0.0001 par value; 200,000,000 shares authorized as of December 31, 2022 and 100,000,000 shares authorized as of December 31, 2021; 48,063,358 shares issued and outstanding as of December 31, 2022 and 26,215,514 shares issued and outstanding as of December 31, 2021	5	3
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of December 31, 2022 and December 31, 2021; 0 shares issued and outstanding as of December 31, 2022 and December 31, 2021	-	-
Additional paid-in capital	352,828	328,958
Accumulated other comprehensive gain/(loss)	51	(107)
Accumulated deficit	(348,823)	(266,315)
Total stockholders' equity	4,061	62,539
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 38,363	$ 89,892