Applied Therapeutics Reports Fourth Quarter and Year-end 2021 Financial Results

March 10, 2022

Progress in Three Phase 3 Trials in Areas of High Unmet Clinical Need, with Multiple Clinical and Regulatory Milestones Expected in 2022

NEW YORK, March 10, 2022 (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, 2021.

"We remain committed to bringing new treatments to patients in areas of high unmet need," said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. "With three ongoing Phase 3 studies across Galactosemia, Sorbitol Dehydrogenase (SORD) Deficiency and Diabetic Cardiomyopathy, we are advancing treatments forward, and expect multiple catalysts and opportunities for value creation in 2022 and 2023."

Recent Highlights

- **Provided Regulatory Update on Galactosemia Program.** In January 2022, the Company provided a regulatory update on the AT-007 Galactosemia program. Following discussions with the FDA at the end of the year, the Company decided to hold on submitting an NDA for AT-007 for treatment of Galactosemia pending additional discussions with the agency. Although the Galactosemia program had previously been discussed in the context of an NDA for Accelerated Approval based on reduction in galactitol, the FDA has now indicated that clinical outcomes data will likely be required for approval. Clinical outcomes are assessed every 6 months by a firewalled committee until the study reaches statistical significance. All children enrolled in the study have completed the 6-month clinical outcomes visits. The Company is in active dialogue with the FDA regarding a potential NDA submission and expects to complete the statistical analysis of the 6-month clinical outcomes in the second quarter. The Company expects to provide an update accordingly.
- **Announced Initiation of Registrational Phase 2/3 Study of AT-007 in SORD Deficiency.** In December 2021, the Company initiated the global registrational phase 2/3 study of AT-007 in Sorbitol Dehydrogenase (SORD) Deficiency. The study, termed INSPIRE (**IN**hibition of **S**orbitol **P**roduction through **I**nhibition of the Aldose **R**eductase **E**nzyme), will investigate biomarker efficacy, clinical outcomes and safety in people living with SORD Deficiency treated with AT-007 vs. placebo.

Financial Results

- **Cash and cash equivalents and short-term investments** totaled $80.8 million as of December 31, 2021, compared with $96.8 million at December 31, 2020.

- **Research and development expenses for the year ended December 31, 2021** were $62.6 million, compared to $61.8 million for the year ended December 31, 2020. The increase of approximately $0.8 million was primarily related to an increase in clinical and pre-clinical expense of $11.6 million, primarily related to the progression of the AT-007 ACTION-Galactosemia adult extension and the AT-007 ACTION-Galactosemia Kids pediatric registrational study; a decrease in drug manufacturing and formulation expenses of $12.5 million primarily related to the completion and release of AT-001 and AT-007 drug product batches in the three months ended March 31, 2021; an increase in personnel expenses of $2.2 million due to the increase in headcount in support of our clinical program pipeline; an increase in stock-based compensation of $0.2 million due to new stock option and restricted stock unit grants, offset by forfeitures of stock option and restricted stock unit grants; and a decrease of regulatory and other expenses of $0.6 million primarily related to the UM license fees recognized during the year ended December 31, 2020.

- **General and administrative expenses** were $43.0 million for the year ended December 31, 2021, compared to $32.7 million for the year ended December 31, 2020. The increase of approximately $10.4 million was primarily related to a decrease in professional and legal fees of $2.1 million due to lower external consulting and legal fees; an increase of $5.6 million related to increased commercial expenditures; an increase in personnel expenses of $1.0 million and an increase in stock-based compensation of $3.0 million due to an increase in headcount; an increase of insurance expenses of $0.6 million related to increased directors and officers liability insurance costs; and an increase in other expenses of $2.3 million, primarily relating to increased costs of rent and other office expenses.

- **Net loss** for the year ended December 31, 2021 was $105.6 million, or $4.12 per basic and diluted common share, compared to a net loss of $94.0 million, or $4.28 per basic and diluted common share, for the year ended December 31, 2020.

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. Applied Therapeutics' portfolio includes three Phase 3 programs for diseases with high unmet medical need and no approved treatment options. 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 and SORD Deficiency. 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.

To learn more, please visit www.appliedtherapeutics.com and follow the company on Twitter @Applied_Tx. A copy of the Company's February 2022 Corporate Presentation is posted to the Investor Relations section of Applied Therapeutics' website.

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, (ii) expectations regarding catalysts and opportunities for value creation in 2022 and 2023, (iii) the timing of the completion of our the statistical analysis of the 6 month clinical outcomes, (iv) the timing of the initiation and completion of our clinical trials, (v) the likelihood that data from our clinical trials will support future development of our product candidates and (vi) 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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Applied Therapeutics, Inc.
Statement of Operations
(in thousands, except share and per share data)

	Year Ended December 31,	
	2021	**2020**
OPERATING EXPENSES:		
Research and development	$ 62,570	$ 61,788
General and administrative	43,048	32,678
Total operating expenses	105,618	94,466
LOSS FROM OPERATIONS	(105,618)	(94,466)
OTHER INCOME (EXPENSE), NET:		
Interest income (expense), net	555	559
Other income (expense)	(521)	(54)

Total other income (expense), net	34	505
Net loss	$ (105,584)	$ (93,961)
Net loss attributable to common stockholders—basic and diluted	$ (105,584)	$ (93,961)
Net loss per share attributable to common stockholders—basic and diluted	$ (4.12)	$ (4.28)
Weighted-average common stock outstanding—basic and diluted	25,598,181	21,966,326

Applied Therapeutics, Inc.
Balance Sheet
(in thousands, except share and per share data)

	As of December 31, 2021	As of December 31, 2020
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 53,888	$ 57,466
Investments	26,935	39,363
Prepaid expenses and other current assets	7,571	5,764
Total current assets	88,394	102,593
Operating lease right-of-use asset	1,298	1,712
Security deposits and leasehold improvements	200	201
TOTAL ASSETS	$ 89,892	$ 104,506
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 442	$ 406
Accounts payable	9,461	640
Accrued expenses and other current liabilities	16,559	20,189
Total current liabilities	26,462	21,235
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	891	1,332
Total noncurrent liabilities	891	1,332
Total liabilities	27,353	22,567
STOCKHOLDERS' EQUITY:		
Common stock, $0.0001 par value; 100,000,000 shares authorized as of December 31, 2021 and December 31, 2020; 26,215,514 shares and 22,493,661 shares issued and outstanding as of December 31, 2021 and December 31, 2020, respectively	3	2
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of December 31, 2021 and December 31, 2020; 0 shares issued and outstanding as of December 31, 2021 and December 31, 2020.	-	-
Additional paid-in capital	328,958	242,780
Accumulated other comprehensive loss	(107)	(112)
Accumulated deficit	(266,315)	(160,731)
Total stockholders' equity	62,539	81,939
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 89,892	$ 104,506