Applied Therapeutics Reports First Quarter 2021 Financial Results

May 11, 2021

Phase 2 pilot study of AT-007 initiated in patients with SORD Deficiency

NDA submission for AT-007 in Galactosemia expected no later than Q3 2021; commercial preparations ongoing

Completion of enrollment in ARISE-HF Phase 3 global registrational study of AT-001 in Diabetic Cardiomyopathy on track for mid-2021

Ended 1Q21 with a strong balance sheet of $148.1 in cash and cash equivalents and short-term investments

NEW YORK, May 11, 2021 (GLOBE NEWSWIRE) -- Applied Therapeutics, Inc. (Nasdaq: APLT) ("Applied Therapeutics" or 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 first quarter ended March 31, 2021.

"In the first quarter we continued to progress our late stage programs towards commercialization in Galactosemia and Diabetic Cardiomyopathy, while advancing our additional rare disease programs in SORD and PMM2-CDG," said Shoshana Shendelman, PhD, Founder, CEO and Chair of the Board of Applied Therapeutics. "We are looking forward to our planned NDA submission in Galactosemia in the third quarter of this year, and continue to focus on our primary objective – bringing drugs to patients in desperate need of treatment."

Recent Highlights

- **Initiated Phase 2 Study of AT-007 in Patients with SORD Deficiency.** The Phase 2 pilot study of AT-007 in patients with Sorbitol Dehydrogenase Deficiency (SORD Deficiency), a progressive, debilitating hereditary neuropathy that affects peripheral nerves and motor neurons, has been initiated. Preclinical findings demonstrate that AT-007 has the potential to be the first disease-modifying therapy for SORD, targeting the underlying cause of disease.

- **Presented Data on Galactosemia Disease Progression at the 2021 Annual Clinical Genetics Meeting of the American College of Medical Genetics and Genomics.** In April 2021, the Company presented data featuring a cross-sectional analysis of nineteen pediatric patients with Classic Galactosemia, providing meaningful insight on the progressive worsening of the central nervous system phenotype with age.

- **Hosted Virtual Rare Disease Forum.** In March 2021, the Company hosted a virtual forum highlighting its AT-007 development programs in Galactosemia, SORD Deficiency, and PMM2-CDG. A replay of the presentation, which featured several rare disease key opinion leaders, is available at https://www.appliedtherapeutics.com/presentations-and-publications/.

- **Announced Restart of Pediatric Galactosemia Study**. In February 2021, the Company announced that the FDA lifted the clinical hold on the AT-007 ACTION-Galactosemia Kids pediatric clinical study, and the study resumed immediately. Applied Therapeutics worked closely with FDA to modify the trial, with the shared goal of ensuring that all patients have the opportunity to receive clinical benefit, and remains on target to submit an NDA no later than Q3 2021.

- **Closed Approximately $75 million Public Offering.** In February 2021, the Company completed an underwritten public offering of 3,450,000 shares of its common stock, including the exercise in full of the underwriters' option to purchase 450,000 additional shares of common stock at a price of $23.00 per share, resulting in aggregate net proceeds of approximately $74.4 million.

Financial Results

- **Cash and cash equivalents and short-term investments** totaled $148.1 million as of March 31, 2021, compared with $96.8 million at December 31, 2020.

- **Research and development expenses** for the three months ended March 31, 2021 were $14.4 million, compared to $7.3 million for the three months ended March 31, 2020. The increase of $7.2 million was primarily related to an increase in clinical and pre-clinical expense of $1.3 million, related to the progression of the AT-007 ACTION-Galactosemia adult extension study, the AT-007 ACTION-Galactosemia Kids pediatric registrational study and the AT-001 Phase 3 ARISE-HF clinical study; an increase in drug manufacturing and formulation costs of $5.2 million related to the progression of the manufacturing campaigns and the completion and release of AT-001 and AT-007 drug product batches; an increase in personnel expenses of $0.3 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; and an increase in regulatory and other expenses of $0.2 million relating to an increase in clinical consulting fees during the three months ended March 31, 2021.

- **General and administrative expenses** were $9.8 million for the three months ended March 31, 2021, compared to $5.2 million for the three months ended March 31, 2020. The increase of approximately $4.6 million was primarily related to an increase of $1.9 million related to the establishment of a commercial department; an increase in stock-based compensation of $1.5 million and increase in personnel expenses of $0.9 million related to an increase in headcount; an increase of $0.2 million related to increased insurance costs; an increase of $0.4 million in other expenses relating to increased costs of rent and other office expenses; and a $0.4 million decrease in legal and professional fees due to lower external legal fees.

- **Net loss** for the first quarter of 2021 was $24.2 million, or $1.00 per basic and diluted common share, compared to a net loss of $12.4 million, or $0.59 per basic and diluted common share, for the first quarter 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. The Company's lead drug candidate, AT-007, is a novel central nervous system penetrant aldose reductase inhibitor (ARI) for the treatment of Galactosemia, a rare pediatric metabolic disease. The Company initiated a pivotal Phase 1/2 clinical trial in June 2019, read out positive top-line biomarker data in adult Galactosemia patients in January 2020 and announced full data from the trial in April 2020. A pediatric Galactosemia study commenced in June 2020. The Company is also developing AT-001, a novel potent ARI that is being developed for the treatment of Diabetic Cardiomyopathy, or DbCM, a fatal fibrosis of the heart. The Company initiated a Phase 3 registrational study in DbCM in September 2019. 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.

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, which will include data from the ACTION-Galactosemia Kids trial and the 90-day safety data in adults with Galactosemia, (ii) the timing of our rare disease franchise expansion programs in SORD Deficiency and PMM2-CDG, (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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Statement of Operations
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended March 31,	
	2021	**2020**
OPERATING EXPENSES:		
Research and development	$ 14,448	$ 7,271
General and administrative	9,751	5,196
Total operating expenses	24,199	12,467
LOSS FROM OPERATIONS	(24,199)	(12,467)
OTHER INCOME (EXPENSE), NET:		
Interest income (expense), net	76	122
Other income (expense)	(56)	(24)
Total other income (expense), net	20	98
Net loss	$ (24,179)	$ (12,369)
Net loss attributable to common stockholders—basic and diluted	$ (24,179)	$ (12,369)
Net loss per share attributable to common stockholders—basic and diluted	$ (1.00)	$ (0.59)
Weighted-average common stock outstanding—basic and diluted	24,135,735	20,840,658

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

	As of March 31, 2021 (Unaudited)	As of December 31, 2020
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 84,067	$ 57,466
Investments	63,992	39,363
Prepaid expenses and other current assets	6,347	5,764
Total current assets	154,406	102,593
Operating lease right-of-use asset	1,612	1,712
Security deposits and leasehold improvements	201	201
TOTAL ASSETS	$ 156,219	$ 104,506
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 415	$ 406
Accounts payable	3,475	640
Accrued expenses and other current liabilities	15,884	20,189
Total current liabilities	19,774	21,235
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	1,224	1,332
Total noncurrent liabilities	1,224	1,332
Total liabilities	20,998	22,567
STOCKHOLDERS' EQUITY:		
Common stock, $0.0001 par value; 100,000,000 shares authorized as of March 31, 2021 and December 31, 2020; 26,010,465 shares and 22,493,661 shares issued and outstanding as of March 31, 2021 and December 31, 2020, respectively	3	2
Additional paid-in capital	320,282	242,780
Accumulated other comprehensive loss	(154)	(112)
Accumulated deficit	(184,910)	(160,731)
Total stockholders' equity	135,221	81,939
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 156,219	$ 104,506