Applied Therapeutics Reports Second Quarter 2021 Financial Results

August 12, 2021

NDA submission for AT-007 in Galactosemia expected in Q3 2021; commercial preparations ongoing

Ended Q2 2021 with a strong balance sheet with $125.6 million in cash and cash equivalents and short-term investments

NEW YORK, Aug. 12, 2021 (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 second quarter ended June 30, 2021.

“Our pediatric Galactosemia program advanced significantly in the second quarter,” said Shoshana Shendelman, PhD, CEO, Founder and Chair of the Board of Applied Therapeutics. “We continue to prepare for our NDA submission in the third quarter and our anticipated commercial launch in 2022. We are excited to launch our registrational study in SORD and complete enrollment in ARISE-HF, both of which are expected later this year.”

Recent Highlights

- **Granted Fast Track Designation by FDA for AT-007 for Galactosemia.** In June 2021, the U.S Food and Drug Administration (FDA) granted Fast Track Designation to AT-007 for the treatment of Galactosemia. Applied Therapeutics plans to submit a New Drug Application (NDA) for Accelerated Approval of AT-007 for the treatment of Galactosemia in the third quarter of this year. FDA had previously granted Orphan Drug Designation and Pediatric Rare Disease status to AT-007 for Galactosemia.

- **Presented Data on the Prevalence of Diabetic Cardiomyopathy (DbCM) at the 81st Scientific Sessions of the 2021 Annual Meeting of the American Diabetes Association.** In June 2021, the Company announced data presentations confirming the high prevalence of DbCM in adults with diabetes or pre-diabetes. The analysis estimated that ~ 1 in 5 people with diabetes or pre-diabetes have DbCM, a serious and progressive disease that limits the heart's ability to function. The Phase 3 registrational ARISE-HF study evaluating AT-001 in patients with DbCM continues to enroll.

- **Added to Russell Microcap® Index.** In June 2021, the Company announced that it had joined the Russell Microcap Index, effective after the market close on Friday, June 25, 2021. Membership in the Russell Microcap® Index, which remains in place for one year, means automatic inclusion in the appropriate growth and value style indexes. FTSE Russell determines membership for its Russell indexes primarily by objective, market-capitalization rankings and style attributes.

- **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.

Financial Results

- **Cash and cash equivalents and short-term investments** totaled $125.6 million as of June 30, 2021, compared with $148.1 million at March 31, 2021.

- **Research and development expenses** for the three months ended June 30, 2021 were $14.8 million, compared to $20.8 million for the three months ended June 30, 2020. The decrease of $6.0 million was related to a decrease in drug manufacturing and formulation costs of $7.1 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; a decrease in stock-based compensation of $0.1 million due to forfeitures of stock option and restricted stock unit grants related to personnel terminations; an increase in clinical and pre-clinical expense of $0.9 million, primarily related to the progression of the AT-007 ACTION-Galactosemia adult extension study and the AT-007 ACTION-Galactosemia Kids pediatric registrational study; and an increase in personnel expenses of $0.3 million due to the increase in headcount in support of our clinical program pipeline.

- **General and administrative expenses** were $11.1 million for the three months ended June 30, 2021, compared to $7.5 million for the three months ended June 30, 2020. The increase of $3.6 million was primarily related to an increase of $1.9 million related to the expansion of the commercial department; an increase in stock-based compensation of $1.1 million related to an increase in headcount; an increase of $0.2 million related to increased insurance costs; an increase of $0.9 million in other expenses related to increased costs of rent and other office expenses; and a decrease of $0.4 million in

legal and professional fees due to lower external legal fees.

- **Net loss** for the second quarter of 2021 was $25.8 million, or $0.99 per basic and diluted common share, compared to a net loss of $28.1 million, or $1.27 per basic and diluted common share, for the second 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 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, as well as novel dual PI3k inhibitors in preclinical development for orphan oncology indications.

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. 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, (iii) the timing of the initiation and completion of our clinical trials, including ARISE-HF, (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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Applied Therapeutics, Inc.
Statement of Operations
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
OPERATING EXPENSES:				
Research and development	$ 14,802	$ 20,758	$ 29,250	$ 28,028
General and administrative	11,073	7,522	20,824	12,725
Total operating expenses	25,875	28,280	50,074	40,753

LOSS FROM OPERATIONS	(25,875)	(28,280)	(50,074)	(40,753)
OTHER INCOME (EXPENSE), NET:				
Interest income (expense), net	169	183	245	304
Other income (expense)	(122)	38	(178)	21
Total other income (expense), net	47	221	67	325
Net loss	$ (25,828)	$ (28,059)	$ (50,007)	$ (40,428)
Net loss attributable to common stockholders—basic and diluted	$ (25,828)	$ (28,059)	$ (50,007)	$ (40,428)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.99)	$ (1.27)	$ (1.99)	$ (1.88)
Weighted-average common stock outstanding—basic and diluted	26,082,525	22,062,030	25,114,508	21,451,344

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

	As of June 30, 2021 (Unaudited)	As of December 31, 2020
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 78,434	$ 57,466
Investments	47,182	39,363
Prepaid expenses and other current assets	9,634	5,764
Total current assets	135,250	102,593
Operating lease right-of-use asset	1,508	1,712
Security deposits and leasehold improvements	201	201
TOTAL ASSETS	$ 136,959	$ 104,506
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 426	$ 406
Accounts payable	3,310	640
Accrued expenses and other current liabilities	19,669	20,189
Total current liabilities	23,405	21,235
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	1,114	1,332
Total noncurrent liabilities	1,114	1,332
Total liabilities	24,519	22,567
STOCKHOLDERS' EQUITY:		
Common stock, $0.0001 par value; 100,000,000 shares authorized as of June 30, 2021 and December 31, 2020; 26,130,536 shares and 22,493,661 shares issued and outstanding as of June 30, 2021 and December 31, 2020, respectively	3	2
Additional paid-in capital	323,360	242,780
Accumulated other comprehensive loss	(185)	(112)
Accumulated deficit	(210,738)	(160,731)
Total stockholders' equity	112,440	81,939
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 136,959	$ 104,506