Applied Therapeutics Reports Third Quarter 2024 Financial Results

November 7, 2024

- NDA and MAA for govorestat for treatment of Classic Galactosemia under FDA Priority Review and EMA review; PDUFA target action date of November 28, 2024, and EMA decision expected in Q1 2025

- NDA submission for govorestat for the treatment of SORD Deficiency under Accelerated Approval expected in early Q1 2025

NEW YORK, Nov. 07, 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 third quarter ended September 30, 2024.

"We are proud of the significant progress we've made this quarter as we prepare for a transformational year ahead, with a focus on transitioning from a clinical-stage company to a commercial organization. With regulatory submissions for govorestat underway in two rare disease indications of urgent unmet need, Classic Galactosemia and SORD Deficiency, we continue to thoughtfully execute our pre-launch initiatives," said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. "As we approach the final stages of the NDA review process for Classic Galactosemia in parallel with a near-term NDA submission for SORD Deficiency, we remain confident in the promise of govorestat and its ability to address the underlying mechanisms of both diseases. We look forward to the opportunity to bring govorestat to patients in 2025."

Recent Highlights

- **NDA Review of Govorestat for the Treatment of Classic Galactosemia Ongoing with PDUFA Target Action Date of November 28, 2024; MAA under CHMP Review by EMA.** The New Drug Application (NDA) review of govorestat for the treatment of Classic Galactosemia remains ongoing within the U.S. Food and Drug Administration (FDA)'s Division of Rare Diseases and Medical Genetics with a Prescription Drug User Fee Act (PDUFA) target action date of November 28, 2024. 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 remains under review by the EMA's Committee for Medicinal Products for Human Use (CHMP). The review remains within the Day 120 clock stop period and the Company expects a decision by the EMA early in the first quarter of 2025. The NDA and MAA submission packages are supported by rapid and sustained reduction in galactitol, which resulted in a meaningful benefit on clinical outcomes across pediatric patients, alongside a favorable safety profile. The 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. If approved, govorestat would be the first medication indicated for the treatment of Galactosemia and would be Applied Therapeutics' first commercial product.

- **NDA Submission Under Accelerated Approval for Govorestat for the Treatment of SORD Deficiency Anticipated in Early Q1 2025.** Following a Type C meeting with the Neurology I Division of the FDA to align on the regulatory path forward for govorestat for the treatment of SORD Deficiency, the Company expects to submit an NDA early in the first quarter of 2025. The review and potential approval of govorestat for the treatment of SORD is independent of the ongoing review of govorestat for Classic Galactosemia. If govorestat is approved for the treatment of Classic Galactosemia, the regulatory submission for the treatment of SORD will be submitted as a supplementary New Drug Application (sNDA). Patients in the Phase 3 INSPIRE study have been transitioned to open-label govorestat treatment and will be followed for additional safety data generation.

- **Highlighted Clinical Data and Development Characterization of Govorestat for the Treatment of Classic Galactosemia at Medical Conferences.** In the third and fourth quarters of 2024, the Company presented at the 2024 Annual Symposium of the Society for the Study of Inborn Errors of Metabolism (SSIEM) and the American Society of Human Genetics (ASHG) Annual Meeting 2024. The presentations highlighted the mechanism of disease pathogenesis for Classic Galactosemia, the design of the first clinical outcomes study in Classic Galactosemia and the results of the ACTION-Galactosemia Kids study.

Financial Results

- **Cash and cash equivalents and short-term investments** totaled $98.9 million as of September 30, 2024, compared with $49.9 million at December 31, 2023.

- **Research and development expenses** for the three months ended September 30, 2024, were $14.8 million, compared to $10.8 million for the three months ended September 30, 2023. The increase of approximately $4.0 million was primarily related to an increase in clinical, pre-clinical and regulatory expense related to govorestat, an increase in drug manufacturing and formulation costs related to the release of legacy accruals in prior year that did occur in current year, and an overall increase in personnel and stock-based compensation expenses.

- **General and administrative expenses** were $15.0 million for the three months ended September 30, 2024, compared to $4.7 million for the three months ended September 30, 2023. The increase of approximately $10.3 million was primarily related to an increase in legal and professional fees of $1.7 million, an increase in commercial expenses to support planned commercialization of govorestat of $6.8 million, an increase in personnel and stock-based compensation expenses of $1.4 million due to increased headcount, and an increase in other miscellaneous expense of $0.5 million due to an overall increase in data storage costs to support planned commercialization, offset by a decrease in insurance expenses.

- **Net loss** for the third quarter of 2024 was $68.6 million, or $0.48 per basic and diluted common share, compared to a net loss of $42.4 million, or $0.47 per basic and diluted common share, for the third quarter 2023.

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) likelihood that the Company's ongoing NDA and MMA submissions will be approved and the timing of any decision and (ii) statements related to the scheduling or timing of any potential FDA or EMA meetings, interactions or submissions. 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.
Condensed Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	As of September 30, 2024	As of December 31, 2023
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 98,867	$ 49,898
Security deposits and leasehold improvements	253	254
Prepaid expenses and other current assets	5,483	4,234
Total current assets	104,603	54,386
Operating lease right-of-use asset	1,963	447
TOTAL ASSETS	$ 106,566	$ 54,833
LIABILITIES AND STOCKHOLDERS' EQUITY/(DEFICIT)		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 264	$ 429
Accounts payable	2,837	1,742
Accrued expenses and other current liabilities	13,489	15,286
Warrant liabilities	82,377	53,725
Total current liabilities	98,967	71,182
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	1,707	38
Clinical holdback - long-term portion	—	759
Total noncurrent liabilities	1,707	797
Total liabilities	100,674	71,979
STOCKHOLDERS' EQUITY/(DEFICIT):		
Common stock, $0.0001 par value; 250,000,000 shares authorized as of September 30, 2024 and 200,000,000 shares authorized as of December 31, 2023; 116,356,474 shares issued and outstanding as of September 30, 2024 and 84,869,832 shares issued and outstanding as of December 31, 2023	11	8
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of September 30, 2024 and December 31, 2023; 0 shares issued and outstanding as of September 30, 2024 and December 31, 2023	—	—
Additional paid-in capital	624,098	451,432
Accumulated deficit	(618,217)	(468,586)
Total stockholders' equity/(deficit)	5,892	(17,146)
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY/(DEFICIT)	$ 106,566	$ 54,833

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
REVENUE:				
License revenue	$ —	$ —	$ —	$ 10,660
Research and development services revenue	122	—	455	—
Total revenue	122	—	455	10,660
COSTS AND EXPENSES:				
Research and development	14,828	10,785	37,049	38,602
General and administrative	15,037	4,710	34,683	15,585
Total costs and expenses	29,865	15,495	71,732	54,187
LOSS FROM OPERATIONS	(29,743)	(15,495)	(71,277)	(43,527)
OTHER (EXPENSE) INCOME, NET:				

Interest income	1,357	392	2,572	1,020
Change in fair value of warrant liabilities	(40,184)	(27,277)	(80,845)	(39,611)
Other (expense) income, net	(21)	10	(81)	34
Total other expense, net	(38,848)	(26,875)	(78,354)	(38,557)
Net loss	$ (68,591)	$ (42,370)	$ (149,631)	$ (82,084)
Net loss per share attributable to common stockholders	$ (0.48)	$ (0.47)	$ (1.09)	$ (1.09)
Weighted-average common stock outstanding	144,345,781	90,669,969	137,893,249	75,482,234