Applied Therapeutics Reports First Quarter 2024 Financial Results

May 9, 2024

- *Govorestat NDA for Classic Galactosemia under Priority Review, PDUFA target action date of November 28, 2024*
- *Govorestat MAA under review by EMA; Day 120 3-month clock-stop extension granted; decision expected in early Q1 2025*
- *Company discussing potential NDA submission under Accelerated Approval for govorestat for treatment of SORD Deficiency with Neurology I Division of FDA*

NEW YORK, May 09, 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 first quarter ended March 31, 2024.

"Preparations are underway for the potential approval and commercial launch of govorestat for the treatment of Classic Galactosemia in the US and EU, following the significant regulatory progress we have already made in 2024," said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. "We are also discussing a potential NDA submission for SORD Deficiency with the Neurology Division at FDA, further advancing our objective of bringing first ever treatment to patients with rare diseases."

Recent Highlights

- **Govorestat NDA Under Priority Review by US FDA for Treatment of Classic Galactosemia, PDUFA Target Action Date of November 28, 2024; MAA under CHMP Review by EMA.** In March 2024, the Company announced that the U.S. Food and Drug Administration (FDA) has extended the review period for the New Drug Application (NDA) for govorestat (AT-007) for the treatment of Classic Galactosemia to allow more time to review supplemental analyses of previously submitted data that had been provided by Applied in response to the FDA's routine information requests. No additional data or studies have been requested by the FDA at this time. The new PDUFA action date is November 28, 2024. The NDA was granted Priority Review Status, and the FDA also noted that it plans to hold an advisory committee meeting to discuss the application. 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 is under review by the EMA's Committee for Medicinal Products for Human Use (CHMP). In April 2024, the EMA granted a 3-month extension to the Day 120 clock stop period to allow sufficient time for responses to the CHMP's Day 120 list of questions. As a result, the Company now expects a decision by the EMA in early first quarter of 2025. The NDA and MAA 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.
- **Appointed Dale Hooks as Chief Commercial Officer.** In April 2024, the Company appointed Dale Hooks as Chief Commercial Officer to lead Applied's commercial preparations for the potential launch of govorestat for the treatment of Classic Galactosemia and SORD. Mr. Hooks brings over 30 years of biopharmaceutical experience in leadership, rare disease marketing and sales roles to Applied Therapeutics.
- **Presented Full Results from Phase 3 ARISE-HF Study of AT-001 (caficrestat) in Diabetic Cardiomyopathy at the 2024 American College of Cardiology Annual Scientific Session.** In April 2024, the Company presented full results from the Phase 3 ARISE-HF study of AT-001 in patients with diabetic cardiomyopathy (DbCM) in an oral presentation at the 2024 American College of Cardiology (ACC) Annual Scientific Session. In the ARISE-HF study, treatment with AT-001 demonstrated a strong trend in stabilizing cardiac functional capacity, and a statistically significant difference in cardiac functional capacity in a prespecified subgroup of patients not receiving concomitant treatment with an SGLT2 or GLP-1 while preventing clinically significant worsening. Furthermore, AT-001 treatment prevented progression to overt heart failure in patients with DbCM as compared to placebo (p=0.0285). Data also demonstrated that AT-001 was safe and well tolerated in a large cohort of patients, providing proof of concept that the technology has overcome the selectivity and safety issues of "old" aldose reductase inhibitors. Following the presentation, the full study results were published in the Journal of the American College of Cardiology (JACC).

Financial Results

- **Cash and cash equivalents and short-term investments** totaled $146.5 million as of March 31, 2024, compared with $49.9 million at December 31, 2023.

- **Research and development expenses** for the three months ended March 31, 2024, were $12.2 million, compared to $15.9 million for the three months ended March 31, 2023. The decrease of approximately $3.7 million was primarily attributed to decreased expenses related to contract research organizations (CROs) and drug manufacturing and formulation costs, partially offset by an increase in regulatory and personnel expenses.

- **General and administrative expenses** were $9.1 million for the three months ended March 31, 2024, compared to $5.6 million for the three months ended March 31, 2023. The increase of approximately $3.5 million was primarily related to an increase in legal and professional fees of $1.2 million and $1.4 million in commercial expenses to support planned commercialization of govorestat, and an increase in other expenses of $0.9 million due to an overall increase in data storage costs to support planned commercialization.

- **Net loss** for the first quarter of 2024 was $83.9 million, or $0.67 per basic and diluted common share, compared to a net loss of $10.1 million, or $0.18 per basic and diluted common share, for the first quarter 2023.

- **Cash runway:** The Company expects that its cash and cash equivalents will fund the business into 2026. Additionally, the sale of the priority review voucher (PRV), which would be granted upon a potential NDA approval of govorestat for the treatment of Galactosemia could substantially extend the Company's cash runway.

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) Company's expectation that its cash and cash equivalents will fund the business into 2026; (ii) the likelihood that the Company's ongoing NDA and MMA submissions will be approved and the timing of any approval decision and (iii) statements related to the potential commercial launch of govorestat. 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.

Contacts

Investors:
Maeve Conneighton

(212) 600-1902 or
appliedtherapeutics@argotpartners.com

Media:
media@appliedtherapeutics.com

Applied Therapeutics, Inc.
Condensed Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	As of March 31, 2024	As of December 31, 2023
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 146,484	$ 49,898
Security deposits and leasehold improvements	253	254
Prepaid expenses and other current assets	4,169	4,234
Total current assets	150,906	54,386
Operating lease right-of-use asset	328	447
TOTAL ASSETS	$ 151,234	$ 54,833
LIABILITIES AND STOCKHOLDERS' EQUITY/(DEFICIT)		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 308	$ 429
Accounts payable	4,019	1,742
Accrued expenses and other current liabilities	14,641	15,286
Warrant liabilities	64,937	53,725
Total current liabilities	83,905	71,182
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	35	38
Clinical holdback - long-term portion	—	759
Total noncurrent liabilities	35	797
Total liabilities	83,940	71,979
STOCKHOLDERS' EQUITY/(DEFICIT):		
Common stock, $0.0001 par value; 200,000,000 shares authorized as of March 31, 2024 and December 31, 2023; 114,241,803 shares issued and outstanding as of March 31, 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 March 31, 2024 and December 31, 2023; 0 shares issued and outstanding as of March 31, 2024 and December 31, 2023	—	—
Additional paid-in capital	619,807	451,432
Accumulated deficit	(552,524)	(468,586)
Total stockholders' equity/(deficit)	67,294	(17,146)
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY/(DEFICIT)	$ 151,234	$ 54,833

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

	Three Months Ended March 31,	
	2024	2023
REVENUE:		
License revenue	$ —	$ 10,660
Research and development services revenue	190	—
Total revenue	190	10,660
COSTS AND EXPENSES:		
Research and development	12,217	15,935

General and administrative	9,066	5,583
Total costs and expenses	21,283	21,518
LOSS FROM OPERATIONS	(21,093)	(10,858)
OTHER (EXPENSE) INCOME, NET:		
Interest income	586	221
Change in fair value of warrant liabilities	(63,405)	469
Other expense	(26)	31
Total other (expense) income, net	(62,845)	721
Net loss	$ (83,938)	$ (10,137)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.67)	$ (0.18)
Weighted-average common stock outstanding—basic and diluted	125,318,993	56,357,983