Applied Therapeutics Reports Second Quarter 2024 Financial Results

August 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 early Q1 2025*

- *FDA Genetic Metabolic Diseases Advisory Committee (GeMDAC) Meeting to discuss NDA for govorestat for the treatment of Classic Galactosemia tentatively scheduled for October 9, 2024*

- *Company aligned with the Neurology I Division on plans to submit an NDA under Accelerated Approval for govorestat for the treatment of SORD Deficiency; Expect to submit an NDA in early Q1 2025*

NEW YORK, Aug. 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 second quarter ended June 30, 2024.

"Momentum continues with our steady regulatory progress in Classic Galactosemia and SORD Deficiency," said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics. "We are incredibly pleased to share our alignment with the Neurology Division of the FDA regarding a potential second NDA submission for govorestat for the treatment of SORD Deficiency. Both Galactosemia and SORD Deficiency are rare neurological diseases with no currently approved treatment options. At Applied, we are dedicated to creating transformative treatments for rare diseases, and we continue to work closely with regulatory agencies and patient advocacy groups to ensure that treatments become available for patients with these debilitating diseases."

Recent Highlights

- **Govorestat PDUFA Target Action Date of November 28, 2024; MAA under CHMP Review by EMA; Updated Cognition Data Included in Review.** In the process of preparing for the United States Food and Drug Administration (FDA) inspection, it was discovered that the vendor hired to compile NIH Toolbox data for the Company used an adult formula for calculation of about one third of composite cognition and motor skills scores. Adjusting the formula to the pediatric formula resulted in significantly improved data for cognition as compared to the prior data, demonstrating improvement in the govorestat (AT-007) treated group of approximately 8 points on a standard scale, which was statistically significant compared to placebo (p=0.032). This also resulted in a statistically significant effect on the primary endpoint sensitivity analysis which included cognition (p=0.034). The motor skills data did not change substantially. These updates were disclosed and discussed with the FDA and European Medicines Agency (EMA) and will be used in the ongoing evaluation of the New Drug Application (NDA) and Marketing Authorization Application (MAA). As previously announced, the FDA Prescription Drug User Fee Act (PDUFA) target action date is 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 MAA for govorestat for the treatment of Classic Galactosemia to the EMA, which was validated in December 2023 and is under review by the EMA's Committee for Medicinal Products for Human Use (CHMP). As previously announced, 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. 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.
- **FDA Advisory Committee Meeting to Review Govorestat NDA for the Treatment of Classic Galactosemia Tentatively Scheduled for October 9, 2024.** The FDA notified the Company of their tentative plans to convene the Genetic Metabolic Diseases Advisory Committee (GeMDAC) on October 9, 2024, to discuss the Company's NDA for govorestat for the treatment of Classic Galactosemia. The date is tentative and has not yet been confirmed in the federal register. The newly formed GeMDAC will consist of experts in the fields of medical genetics, inborn errors of metabolism,

epidemiology, and other related specialties.

- **Company Aligned with the Neurology I Division of the FDA on Potential Submission of an NDA for Govorestat for the Treatment of SORD Deficiency Under Accelerated Approval.** In July 2024, the Company held a Type C meeting with the FDA to align on the regulatory path forward for govorestat for the treatment of SORD Deficiency. The Neurology I Division confirmed that the data generated to-date was appropriate for a potential NDA submission under the FDA's Accelerated Approval Program, and discussed the design of a new confirmatory study to be completed as a post-marketing requirement. The Company plans to hold a pre-NDA meeting to discuss administrative aspects of the submission in the second half of this year, and expects to submit an NDA early in the first quarter of 2025. 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 INSPIRE study will be offered open-label govorestat treatment and will be followed for additional safety data generation. The review and potential approval of govorestat for SORD is independent of the ongoing review of govorestat for Classic Galactosemia.
- **APLT Added to Russell 3000® Index.** In June 2024, as part of the Russell indexes annual reconstitution, the Company was added to the Russell 3000® Index, a market capitalization-weighted equity index that tracks the performance of the largest 3,000 U.S. stocks. Membership of the Russell indexes is primarily determined by objective, market-capitalization rankings and style attributes. Russell indexes are widely used by investment managers and institutional investors for index funds and as benchmarks for active investment strategies.
- **Participated in Multiple Medical and Patient Advocacy Group Focused Conferences.** In the second and third quarters of 2024, the Company deepened its relationships and partnership with the patient community, presenting data and giving keynote addresses at the following medical meetings and patient advocacy group conferences:
    - Hereditary Neuropathy Foundation (HNF) Charcot-Marie Tooth Syndrome (CMT) Summit, June 7-8 in San Diego, California;
    - Charcot-Marie-Tooth Associate (CMTA) Strategy to Accelerate Research (STAR) Advisory Board meeting held June 21 in Montreal, Canada;
    - Peripheral Nerve Society (PNS) 2024 Annual Meeting, held June 22-25 in Montreal, Canada;
    - Sponsored and presented at the 2024 Galactosemia Foundation Conference, held July 18-20 in Concord, North Carolina.

Financial Results

- **Cash and cash equivalents and short-term investments** totaled $122.2 million as of June 30, 2024, compared with $49.9 million at December 31, 2023.

- **Research and development expenses** for the three months ended June 30, 2024, were $10.0 million, compared to $11.9 million for the three months ended June 30, 2023. The decrease of approximately $1.9 million was primarily related to decreased expenses associated with clinical and pre-clinical expenses for the near completion of AT-001 and AT-007 and drug manufacturing and formulation costs, partially offset by an increase in regulatory and personnel expenses.

- **General and administrative expenses** were $10.6 million for the three months ended June 30, 2024, compared to $5.3 million for the three months ended June 30, 2023. The increase of approximately $5.3 million was primarily related to an increase in legal and professional fees of $1.3 million, an increase in commercial expenses to support planned commercialization of govorestat of $3.5 million, and an increase in personnel expenses of $1.1 million due to increased headcount and salary increases, offset by a decrease in stock-based compensation, insurance expenses and other miscellaneous expense.

- **Net income** for the second quarter of 2024 was $2.9 million, or $0.02 per basic common share and a net loss of $0.13 per diluted common share, compared to a net loss of $29.6 million, or $0.37 per basic and diluted common share, for the second quarter of 2023.

- **Cash runway:** The Company expects that its cash and cash equivalents will fund the business into 2026. Additionally, the Company expects that 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 decision and (iii) 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.

Contacts

Investors:
Julie Seidel/Andrew Vulis
646-970-0543
appliedtherapeutics@argotpartners.com

Media:
media@appliedtherapeutics.com

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

	As of June 30, 2024	As of December 31, 2023
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 122,197	$ 49,898
Security deposits and leasehold improvements	253	254
Prepaid expenses and other current assets	5,122	4,234
Total current assets	127,572	54,386
Operating lease right-of-use asset	206	447
TOTAL ASSETS	$ 127,778	$ 54,833
LIABILITIES AND STOCKHOLDERS' EQUITY/(DEFICIT)		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 185	$ 429
Accounts payable	2,683	1,742
Accrued expenses and other current liabilities	10,296	15,286

Warrant liabilities	42,192	53,725
Total current liabilities	55,356	71,182
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	30	38
Clinical holdback - long-term portion	—	759
Total noncurrent liabilities	30	797
Total liabilities	55,386	71,979
STOCKHOLDERS' EQUITY/(DEFICIT):		
Common stock, $0.0001 par value; 250,000,000 shares authorized as of June 30, 2024 and 200,000,000 shares authorized as of December 31, 2023; 114,846,271 shares issued and outstanding as of June 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 June 30, 2024 and December 31, 2023; 0 shares issued and outstanding as of June 30, 2024 and December 31, 2023	—	—
Additional paid-in capital	622,007	451,432
Accumulated deficit	(549,626)	(468,586)
Total stockholders' equity/(deficit)	72,392	(17,146)
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY/(DEFICIT)	$ 127,778	$ 54,833

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
REVENUE:				
License revenue	$ —	$ —	$ —	$ 10,660
Research and development services revenue	144	—	334	—
Total revenue	144	—	334	10,660
COSTS AND EXPENSES:				
Research and development	10,004	11,883	22,221	27,818
General and administrative	10,580	5,293	19,646	10,876
Total costs and expenses	20,584	17,176	41,867	38,694
LOSS FROM OPERATIONS	(20,440)	(17,176)	(41,533)	(28,034)
OTHER (EXPENSE) INCOME, NET:				
Interest income	628	408	1,215	628
Change in fair value of warrant liabilities	22,744	(12,804)	(40,660)	(12,335)
Other (expense) income, net	(34)	(5)	(62)	27
Total other income (expense), net	23,338	(12,401)	(39,507)	(11,680)
Net income (loss)	$ 2,898	$ (29,577)	$ (81,040)	$ (39,714)
Net income (loss) per share attributable to common stockholders—basic	$ 0.02	$ (0.37)	$ (0.60)	$ (0.59)
Net income (loss) per share attributable to common stockholders—diluted	$ (0.13)	$ (0.37)	$ (0.60)	$ (0.59)
Weighted-average common stock outstanding—basic	143,934,239	79,041,695	134,627,942	67,762,501
Weighted-average common stock outstanding—diluted	152,392,748	79,041,695	134,627,942	67,762,501