Athira Pharma Reports First Quarter 2023 Financial Results and Recent Pipeline and Business Updates

May 11, 2023

Phase 2/3 LIFT-AD trial of fosgonimeton for mild-to-moderate Alzheimer's disease to focus on 40 mg dose

Open label extension trial to be extended, providing up to 36 months of long-term exposure data

ATH-1105 significantly prolongs survival and improves motor function in a mouse model of ALS; Strongly supports development as a treatment for ALS

Strong balance sheet to support innovative clinical development pipeline in neurodegenerative diseases through key inflection points

BOTHELL, Wash., May 11, 2023 (GLOBE NEWSWIRE) -- **Athira Pharma, Inc.** (NASDAQ: ATHA), a late clinical-stage biopharmaceutical company focused on developing small molecules to restore neuronal health and slow neurodegeneration, today announced the company's financial results for the first quarter ended March 31, 2023, and reviewed recent pipeline and business updates.

"We continue to advance the Phase 2/3 LIFT-AD trial of fosgonimeton as a treatment for mild-to-moderate Alzheimer's disease and will focus on the 40 mg dose, as the totality of the data supports the selection of this dose to enhance both our chances for success and the trial's potential to be a registrational study," said Mark Litton, Ph.D., President and Chief Executive Officer of Athira.

"Throughout the first quarter and in the recent months, we presented supportive preclinical and clinical data describing the therapeutic potential of enhancing the HGF/MET system to protect and repair neuronal networks in neurodegenerative diseases such as Alzheimer's (AD), Parkinson's and amyotrophic lateral sclerosis (ALS). Clinical data recently presented at AAN showed a statistically significant improvement in Mini-Mental State Exam (MMSE) scores in the participants treated with fosgonimeton without concomitant acetylcholinesterase inhibitors (AChEIs) in a post-hoc analysis of the exploratory six-month Phase 2 ACT-AD trial. These data, along with a statistically significant improvement in a plasma biomarker of neurodegeneration (neurofilament light chain, NfL), and directional improvements in biomarkers of neuroinflammation and AD protein pathology continue to give us confidence in the potential for fosgonimeton to become a meaningful therapy for people living with Alzheimer's.

"We have a strong balance sheet that enables us to continue to advance fosgonimeton in Alzheimer's and ATH-1105 in ALS, through key inflection points," concluded Dr. Litton.

Clinical Development & Pipeline Programs

Fosgonimeton (ATH-1017) - Small molecule designed to enhance the HGF/MET system with the potential to protect and repair neuronal networks.

- The Company has selected the 40 mg dose for further development and potential regulatory approval for mild to moderate AD.
- Fosgonimeton continues to demonstrate a favorable safety profile in patients on both the 40 mg and 70 mg doses in all completed and ongoing clinical studies.
- The dose selection was based on a review of the totality of the data across preclinical and clinical studies and in consultation with independent regulatory and biostatistical experts.
- This decision was based on supportive data from participants treated with 40 mg fosgonimeton without concomitant acetylcholinesterase inhibitors (AChEIs):
    - Phase 1b trial results showed 73 milliseconds improvement in P300 latency from baseline compared with placebo at eight days (p=0.027);
    - Post-hoc analyses of the Phase 2 ACT-AD trial of the 40 mg arm at 6 months showed:
        - P300 latency improvement of 37 milliseconds (p=0.050) compared to placebo;
        - ADAS-Cog11 improvement of - 2.6 points (n.s.) compared to placebo; and
        - NfL, improvement of 8.15 pg/mL (p=0.019) compared to placebo.
- A higher number of participants from the ACT-AD trial receiving the 40 mg dose transitioned into the OLEX trial compared to those receiving the 70 mg dose.

LIFT-AD Phase 2/3 trial in mild-to-moderate Alzheimer's disease (NCT04488419)

- In September 2022, an independent, unblinded interim efficacy and futility analysis was performed on 100 patients without concomitant AChEIs who completed the trial. The positive outcome from the independent data monitoring committee supports the potential clinically meaningful activity of fosgo and its potential to achieve the primary endpoint, which is a composite score of cognition and function.
- The Phase 2/3 LIFT-AD trial of fosgonimeton for mild-to-moderate Alzheimer's disease will now focus enrollment and the primary analysis on 40 mg dosing and will discontinue enrollment of the 70 mg arm going forward.
- The Company was granted an end of Phase 2 meeting with the U.S. Food and Drug Administration this summer. The timeline for LIFT-AD will be updated following this meeting.

Open Label Extension (OLEX) trial (NCT04886063)

- The Company plans to further extend the current OLEX for the Phase 2/3 LIFT-AD and Phase 2 ACT-AD trials of fosgonimeton for the treatment of mild-to-moderate AD by an additional 12 months. Following this extension, eligible participants who have completed the LIFT-AD or ACT-AD trials and elect to participate in the ongoing OLEX will be able to receive up to 30 months of open-label treatment. This extension is intended to address investigator and patient interest in continuing treatment with fosgonimeton beyond 18 months. We believe it will also further enhance our long-term safety database and provide insights into fosgonimeton's long-term effects for up to 3 years.
- The OLEX continues to enroll with greater than 85% of participants who have completed either study having elected to enroll in the OLEX trial.

The ACT-AD trial and the related open-label extension for ACT-AD participants was supported by a grant from the National Institute on Aging of the National Institutes of Health under Award Number R01AG06268. The information presented in this press release is solely the responsibility of Athira and does not necessarily represent the official views of the National Institutes of Health.

Presentations and Publications

- The Company presented preclinical and clinical data in support of its small molecule pipeline of therapeutic candidates targeting the HGF/MET neurotrophic system:
  - Delivered an oral presentation at the American Association of Neurology (AAN) 2023 Annual Meeting that highlighted data from additional post hoc analyses from the completed, exploratory Phase 2 ACT-AD trial of fosgonimeton in mild-to-moderate AD, which included statistically significant improvements in MMSE scores following fosgonimeton treatment without concomitant AChEIs. This is in line with previously reported findings showing directional changes in improvements in measures of cognition and plasma biomarkers of neuroinflammation and protein pathology, as well as a statistically significant improvement in Nfl, an established biomarker of neurodegeneration, following fosgonimeton treatment without concomitant AChEIs.
  - Presented an oral presentation at AAN 2023 that reviewed compelling preclinical data that continue to elucidate fosgonimeton's multimodal mechanism of action and therapeutic potential in neurodegenerative diseases.
  - Reported new data at AAN 2023 that provided further insights into the interaction of fosgonimeton with donepezil. Preclinical data presented suggested that the neuroprotective effects of fosgonimeton are muted in combination with donepezil, which we believe to be due, in part, to an interference on neuroprotective AKT signaling, one of the multimodal mechanisms enhanced by targeting the HGF/MET system.
  - Presented three posters at the AD/PD™ 2023 International Conference on Alzheimer's and Parkinson's Diseases and Related Neurological Disorders (AD/PD) that highlighted the potential neuroprotective, neurotrophic and anti-inflammatory effects of enhancing the HGF/MET system.

ATH-1105 – A novel, orally available, small molecule designed to be a positive modulator of the HGF/MET system as a potential treatment candidate for amyotrophic lateral sclerosis (ALS).

- Presented preclinical findings for ATH-1105 at AAN that demonstrated statistically significant improvements on multiple measures of nerve and motor function, biomarkers of inflammation and neurodegeneration, and survival in an animal model of amyotrophic lateral sclerosis (ALS), including prolonged survival and delayed time to first mortality ($p=0.0035$); improvement in balance, coordination, and muscle strength in motor function tests ($p<0.0001$); protection against body weight reduction ($p<0.01$); preservation of nerve function and structure ($p<0.001$); and reduction of plasma biomarkers of systemic inflammation and neurodegeneration ($p<0.0001$).
- Completing IND-enabling work throughout 2023 in order to initiate first-in-human studies in 2024 to evaluate this promising product candidate as a treatment for ALS.

Financial Results

- **Cash Position**. Cash, cash equivalents and investments were $219.9 million as of March 31, 2023, compared with $245.2 million as of December 31, 2022. Net cash used in operations was $26.2 million for the quarter ended March 31, 2023, compared with $16.6 million for the quarter ended March 31, 2022.

- **Research and Development (R&D) Expenses**. R&D expenses were $21.3 million for the quarter ended March 31, 2023, compared with $14.5 million for the quarter ended March 31, 2022. The increase was driven primarily by costs related to increased clinical trial activities, headcount and increased preclinical research and development expenses.

- **General and Administrative (G&A) Expenses**. G&A expenses were $8.5 million for the quarter ended March 31, 2023, compared with $8.9 million for the quarter ended March 31, 2022. The decrease was primarily due to a decrease in legal and other general corporate expenses, partially offset by increases in personnel expenses as the Company's headcount expanded to support its continued growth.

- **Net Loss**. Net loss was $27.8 million, or $0.73 per share, for the quarter ended March 31, 2023, compared with a net loss of $21.0 million, or $0.56 per share, for the quarter ended March 31, 2022.

About Athira Pharma, Inc.
Athira Pharma, Inc., headquartered in the Seattle, Washington area, is a late clinical-stage biopharmaceutical company focused on developing small molecules to restore neuronal health and slow neurodegeneration. Athira aims to provide rapid cognitive improvement and alter the course of neurological diseases with its novel mechanism of action. Athira is currently advancing its pipeline therapeutic candidates targeting the HGF/MET neurotrophic system for Alzheimer's and Parkinson's disease, Dementia with Lewy bodies and ALS. For more information, visit www.athira.com. You can also follow Athira on Facebook, LinkedIn, Twitter and @athirapharma on Instagram.

Forward-Looking Statements
This communication contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995. These forward-looking statements are not based on historical fact and include statements regarding: product candidates as a potential treatment for Alzheimer's disease, Parkinson's disease dementia, Dementia with Lewy bodies, neuropsychiatric diseases, and other neurodegenerative diseases, such as amyotrophic lateral sclerosis; Athira's platform technology and potential therapies; future development plans; clinical and regulatory objectives and the timing thereof; expectations regarding the safety, potential efficacy and commercial potential of Athira's product candidates; the anticipated reporting of data; the potential learnings from the LIFT-AD unblinded interim efficacy and futility analysis and their ability to inform and improve future clinical development plans; Athira's plans to further extend the open label extension trial for its LIFT-AD and ACT-AD clinical trials; and Athira's ability to advance its product candidates into later stages of development. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "on track," "would," "expect," "plan," "believe," "intend," "pursue," "continue," "suggest," "potential," and other similar expressions, among others. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the data for our product candidates from our preclinical and clinical trials not supporting the safety, efficacy and tolerability of our product candidates; cessation or delay of Athira's development of product candidates may occur; regulatory authorities could object to protocols, amendments and other submissions; future potential regulatory milestones for product candidates, including those related to current and planned clinical studies, may be insufficient to support regulatory submissions or approval; the impact of the COVID-19 pandemic on Athira's business, research and clinical development plans and timelines, and the regulatory process for Athira product candidates; Athira may not be able to recruit sufficient patients for its clinical trials; the outcome of legal proceedings that have been or may in the future be instituted against us and certain of our directors and officers; clinical trials may not demonstrate safety and efficacy of any of Athira's product candidates; possible negative interactions of Athira's product candidates with other treatments; Athira's assumptions regarding the sufficiency of its cash, cash equivalents and investments to fund its planned operations may be incorrect; adverse conditions in the general domestic and global economic markets; the impact of competition; regulatory agencies may be delayed in reviewing, commenting on or approving any of Athira's clinical development plans as a result of the COVID-19 pandemic, which could further delay development timelines; the impact of expanded product development and clinical activities on operating expenses; the impact of new or changing laws and regulations; as well as the other risks detailed in Athira's filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date hereof and Athira undertakes no obligation to update forward-looking statements. Athira may not actually achieve the plans, intentions, or expectations disclosed in its forward-looking statements, and you should not place undue reliance on the forward-looking statements.

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Athira Pharma, Inc.
Condensed Consolidated Balance Sheets
(Amounts in thousands)

	March 31, 2023	December 31, 2022
	(unaudited)	
Assets		
Cash and cash equivalents	$ 105,182	$ 95,966
Short-term investments	80,799	104,378
Other short-term assets	6,915	7,189

Long-term investments	33,903	44,829
Other long-term assets	5,973	5,791
Total assets	$ 232,772	$ 258,153
Liabilities and stockholders' equity		
Current liabilities	$ 20,150	$ 21,431
Long-term liabilities	1,497	1,585
Total liabilities	21,647	23,016
Stockholders' equity	211,125	235,137
Total liabilities and stockholders' equity	$ 232,772	$ 258,153

Athira Pharma, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(Amounts in thousands, except share and per share amounts)

(Unaudited)

	Three Months Ended March 31,	
	2023	**2022**
Operating expenses:		
Research and development	$ 21,293	$ 14,460
General and administrative	8,477	8,927
Total operating expenses	29,770	23,387
Loss from operations	(29,770)	(23,387)
Grant income	157	2,234
Other income, net	1,793	173
Net loss	$ (27,820)	$ (20,980)
Unrealized gain (loss) on available-for-sale securities	927	(1,068)
Comprehensive loss attributable to common stockholders	$ (26,893)	$ (22,048)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.73)	$ (0.56)
Weighted-average shares used in computing net loss per share attributable to common stockholders, basic and diluted	37,923,402	37,593,328