March 18, 2024

Avenue Therapeutics Reports Full Year 2023 Financial Results and Recent Corporate Highlights

- Enrollment completed in Phase 1b/2a clinical trial of AJ201 for spinal and bulbar muscular atrophy; topline data expected in second quarter of 2024 -

- Presented positive BAER-101 preclinical data at American Epilepsy Society (AES) and American Society for Experimental Neurotherapeutics (ASENT) Annual Meetings and in publication Drug Development Research -

- Final agreement reached with FDA on study design and analysis approach for Phase 3 safety study of IV tramadol –

MIAMI, March 18, 2024 (GLOBE NEWSWIRE) -- Avenue Therapeutics, Inc. (Nasdaq: ATXI) ("Avenue" or the "Company"), a specialty pharmaceutical company focused on the development and commercialization of therapies for the treatment of neurologic diseases, today reported financial results and recent corporate highlights for the year ended December 31, 2023.

"We made considerable progress across our pipeline of differentiated neurologic therapies in 2023," said Alexandra MacLean, M.D., Chief Executive Officer of Avenue. "Avenue demonstrated tremendous execution capabilities with our lead clinical program AJ201, a potential first-in-class treatment for spinal and bulbar muscular atrophy (SBMA), also known as Kennedy's Disease. Within nine months of acquiring the rights to AJ201 in March 2023, we dosed the first patient in the Phase 1b/2a clinical trial, and we completed trial enrollment of 25 SBMA patients across six sites in the U.S. We remain on track to report topline data for AJ201 in the second quarter of 2024, an incredibly exciting milestone for both the Company and patients suffering from SBMA, a debilitating rare neuromuscular disorder with no approved treatments in the U.S. Additionally, we have progressed BAER-101 for the treatment of epilepsy by presenting preclinical data from a translational animal model in multiple peer-reviewed forums. We have also advanced IV tramadol, reaching final agreement with the U.S. Food and Drug Administration (FDA) on the safety study and statistical analysis approach for the final Phase 3 study. Pending additional financing, we look forward to progressing BAER-101 and IV tramadol for patients facing great unmet need. We look forward to another productive year in 2024 as we continue to make significant strides across our clinical pipeline and advance our mission of providing impactful therapies to patients suffering from neurologic diseases."

Recent Corporate Highlights:

AJ201 *(Nrf1 and Nrf2 activator, androgen receptor degradation enhancer for SBMA)*

- In January 2024, Avenue completed enrollment in the Phase 1b/2a clinical trial of AJ201 for the treatment of SBMA. The 12-week, multicenter, randomized, double-blind Phase 1b/2a clinical trial of AJ201 enrolled 25 patients randomly assigned to AJ201 (600 mg/day) or placebo. The primary endpoint of the study is to assess safety and tolerability of AJ201 in subjects with clinically and genetically defined SBMA. Topline data for the Phase 1b/2a study are anticipated in the second quarter of 2024. More information about this study can be found at ClinicalTrials.gov (Identifier: NCT05517603).

BAER-101 *(GABA$_A$ α2/3 positive allosteric modulator)*

- Avenue presented preclinical results for BAER-101, a potentially best-in-class selective GABA$_A$ α2,3 positive allosteric modulator, in three scientific peer-reviewed settings, including the American Epilepsy Society (AES) Annual Meeting in December 2023, the publication *Drug Development Research* in February 2024 and the American Society for Experimental Neurotherapeutics (ASENT) Annual Meeting in March 2024. The *in vivo* data showcase BAER-101's ability to significantly suppress seizures using the SynapCell's Genetic Absence Epilepsy Rat from Strasbourg ("GAERS") model of epilepsy. BAER-101 fully suppressed seizure activity in the GAERS model with a minimal effective dose of 0.3 mg/kg, PO, and the effect was fast in onset and stable throughout the duration of testing. The data also demonstrated BAER-101's ability to selectively target GABA$_A$ α2 and α3 subtypes more than α1 and α5, potentially improving anti-convulsant and anxiolytic activity while minimizing the risk of tolerance and abuse associated with existing treatments in this drug class. Subject to obtaining the necessary financing, which could be provided through a strategic partnership, Avenue plans to initiate a Phase 2a clinical trial of BAER-101 to further study its anti-seizure properties in patients with common or rare epilepsies.

IV Tramadol

- In January 2024, Avenue reached final agreement with the FDA on the safety study protocol and statistical analysis approach for the Phase 3 study for intravenous ("IV") tramadol, which is in development for the treatment of acute post-operative pain in a medically supervised setting. The final non-inferiority study is designed to assess the theoretical risk of opioid-induced respiratory depression related to opioid stacking on IV tramadol compared to IV morphine. The study will randomize approximately 300 post bunionectomy patients to IV tramadol or IV morphine for pain relief administered during a 48-hour post-operative period. Patients will have access to IV hydromorphone, a Schedule II opioid, for rescue of breakthrough pain. Pending additional financing, Avenue aims to initiate the Phase 3 safety study as soon as feasible. The Company believes that the study can be completed and submitted to the FDA within 12 months of the study's initiation.

General Corporate

- In March 2024, Avenue announced that the Nasdaq Hearings Panel granted the

Company's request for an extension to evidence compliance with all applicable criteria for continued listing on The Nasdaq Capital Market, including the $1.00 bid price and $2.5 million stockholders' equity requirements, through May 20, 2024.

2023 Financial Results:

- **Cash Position:** As of December 31, 2023, cash and cash equivalents totaled $1.8 million, compared to $6.7 million at December 31, 2022, a decrease of $4.9 million. In January 2024, the Company completed a warrant inducement transaction resulting in $5.0 million in gross proceeds.
- **R&D Expenses:** Research and development expenses for the full year 2023 were $6.1 million, compared to $2.7 million in 2022. Additionally, there was $4.2 million in expense for the acquisition of the AJ201 license in 2023.
- **G&A Expenses:** General and administrative expenses for the full year 2023 were $4.2 million, compared to $5.3 million in 2022.
- **Net Loss:** Net loss for the full year 2023 was $10.5 million, or $0.98 per share, compared to a net loss of $3.6 million, or $1.63 per share, in 2022.

About Avenue Therapeutics

Avenue Therapeutics, Inc. (Nasdaq: ATXI) is a specialty pharmaceutical company focused on the development and commercialization of therapies for the treatment of neurologic diseases. It is currently developing three assets including AJ201, a first-in-class asset for spinal and bulbar muscular atrophy, BAER-101, an oral small molecule selective $GABA_A$ α2, α3 receptor positive allosteric modulator for CNS diseases, and IV tramadol, which is in Phase 3 clinical development for the management of acute postoperative pain in adults in a medically supervised healthcare setting. Avenue is headquartered in Miami, FL and was founded by Fortress Biotech, Inc. (Nasdaq: FBIO). For more information, visit www.avenuetx.com.

Forward-Looking Statements

This press release contains predictive or "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. All statements other than statements of current or historical fact contained in this press release, including statements that express our intentions, plans, objectives, beliefs, expectations, strategies, predictions or any other statements relating to our future activities or other future events or conditions are forward-looking statements. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "predict," "project," "will," "should," "would" and similar expressions are intended to identify forward-looking statements. These statements are based on current expectations, estimates and projections made by management about our business, our industry and other conditions affecting our financial condition, results of operations or business prospects. These statements are not guarantees of future performance and involve risks, uncertainties and assumptions that are difficult to predict. Therefore, actual outcomes and results may differ materially from what is expressed or forecasted in, or implied by, the forward-looking statements due to numerous risks and uncertainties. Factors that could cause such outcomes and results to differ include, but are not limited to, risks and uncertainties arising from: the fact that we currently have no drug products for sale and that our success is dependent on our product candidates receiving regulatory approval and being successfully commercialized; the possibility that serious adverse or unacceptable side effects are identified during the development of our current or

future product candidates, such that we would need to abandon or limit development of some of our product candidates; our ability to successfully develop, partner, or commercialize any of our current or future product candidates including AJ201, IV tramadol, and BAER-101; the substantial doubt raised about our ability to continue as a going concern, which may hinder our ability to obtain future financing; the significant losses we have incurred since inception and our expectation that we will continue to incur losses for the foreseeable future; our need for substantial additional funding, which may not be available to us on acceptable terms, or at all, which unavailability of could force us to delay, reduce or eliminate our product development programs or commercialization efforts; our reliance on third parties for several aspects of our operations; our reliance on clinical data and results obtained by third parties that could ultimately prove to be inaccurate, or unreliable, or unacceptable to regulatory authorities; the possibility that we may not receive regulatory approval for any or all of our product candidates, or that such approval may be significantly delayed due to scientific or regulatory reasons; the fact that even if one or more of our product candidates receives regulatory approval, they will remain subject to substantial regulatory scrutiny; the effects of current and future laws and regulations relating to fraud and abuse, false claims, transparency, health information privacy and security, and other healthcare laws and regulations; the effects of competition for our product candidates and the potential for new products to emerge that provide different or better therapeutic alternatives for our targeted indications; the possibility that the government or third-party payors fail to provide adequate coverage and payment rates for our product candidates or any future products; our ability to establish sales and marketing capabilities or to enter into agreements with third parties to market and sell our product candidates; our exposure to potential product liability claims; related to the protection of our intellectual property and our potential inability to maintain sufficient patent protection for our technology and products; our ability to maintain compliance with the obligations under our intellectual property licenses and funding arrangements with third parties, without which licenses and arrangements we could lose rights that are important to our business; the fact that Fortress Biotech, Inc. controls a majority of the voting power of our outstanding capital stock and has rights to receive significant share grants annually;; and those risks discussed in our filings which we make with the SEC. Any forward-looking statements speak only as of the date on which they are made, and we undertake no obligation to publicly update or revise any forward-looking statements to reflect events or circumstances that may arise after the date of this press release, except as required by applicable law. Investors should evaluate any statements made by us in light of these important factors.

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AVENUE THERAPEUTICS, INC.
Consolidated Balance Sheets
($ in thousands, except for share and per share amounts)

	December 31, 2023	December 31, 2022

ASSETS		
Current assets:		
Cash and cash equivalents	$ 1,783	$ 6,708
Prepaid expenses and other current assets	67	137
Total assets	$ **1,850**	$ **6,845**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued expenses	$ 287	$ 949
Accounts payable and accrued expenses - related party	323	21
Warrant liability	586	2,609
Total current liabilities	1,196	3,579
Total liabilities	**1,196**	**3,579**
Commitments and Contingencies		
Stockholders' equity		
Preferred stock ($0.0001 par value), 2,000,000 shares authorized		
Class A Preferred stock, 250,000 shares issued and outstanding as of December 31, 2023 and 2022, respectively	—	—
Common stock ($0.0001 par value)		
Common shares, 75,000,000 shares authorized and 25,597,622 shares issued and outstanding as of December 31, 2023; and 20,000,000 shares authorized and 4,773,841 shares issued and outstanding as of December 31, 2022	3	—
Additional paid-in capital	92,507	84,456
Accumulated deficit	(90,928)	(80,551)
Total stockholders' equity attributed to the Company	1,582	3,905
Non-controlling interests	(928)	(639)
Total stockholders' equity	654	3,266
Total liabilities and stockholders' equity	$ **1,850**	$ **6,845**

AVENUE THERAPEUTICS, INC.
Consolidated Statements of Operations
($ in thousands, except for share and per share amounts)

	For the Years Ended	
	December 31, 2023	**December 31, 2022**
Operating expenses		
Research and development	$ 6,131	$ 2,698
Research and development - licenses acquired	4,230	—
General and administrative	4,179	5,345
Loss from operations	(14,540)	(8,043)
Interest income	(126)	(20)
Financing costs – warrant liabilities	332	1,160
Change in fair value of warrant liabilities	(4,258)	(5,580)
Net loss	$ **(10,488)**	$ **(3,603)**
Net loss attributable to non-controlling interests	(111)	(51)
Net loss attributable to common stockholders	$ **(10,377)**	$ **(3,552)**
Net loss per common share attributable to common stockholders, basic and diluted	$ (0.98)	$ (1.63)

Weighted average number of common shares outstanding, basic and diluted	10,591,636	2,185,159

Source: Avenue Therapeutics