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Avenue Therapeutics Announces Publication in Drug Development Research Highlighting First-In-Class Preclinical Data of BAER-101 in a Translational Model of Absence Epilepsy

-In vivo data supports BAER-101's unique ability to significantly suppress seizures using the SynapCell's Genetic Absence Epilepsy Rat from Strasbourg ("GAERS") model of absence epilepsy-

MIAMI, Feb. 22, 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 announced the publication of preclinical *in vivo* data in *Drug Development Research* highlighting BAER-101's full suppression of seizure activity using the Genetic Absence Epilepsy Rats from Strasbourg ("GAERS") model of absence epilepsy. Data published showcase BAER-101's ability to selectively target GABA_A α 2 and α 3 subtypes more than α 1 and α 5, potentially improving anti-convulsant and anxiolytic activity while minimizing the risk of tolerance and abuse associated with existing treatments in this drug class.

The publication describes the extent of anti-seizure activity of BAER-101 in the GAERS model, a widely used and translationally relevant animal model. The study demonstrated full suppression of seizure activity with a minimal effective dose (MED) of 0.3 mg/kg. The effect of BAER-101 was fast in onset and stable throughout the duration of testing. Results from the testing showed that the number of spike-wave discharges were dose-dependently reduced by BAER-101, and no adverse safety events were observed up to a dose 300x the MED.

BAER-101 is the first clinical candidate which is selective for only α 2,3 and not for α 1 or α 5, a pharmacology consistent with anti-seizure activity that avoids the adverse side effects common to the GABA_A positive allosteric modulators (PAM) class. Specifically, the α 1-subtype of GABAAR is associated with dizziness and somnolence in both animal and human studies, and the α 5-subtype of GABAAR is thought to play a key role in synaptic plasticity, cognition, and memory, suggesting that engagement of α 5 risks anti-cognitive effects. The pharmacology of BAER-101 lacks activity at both the α 1- and α 5-subtypes of GABAAR, and these findings indicate that BAER-101's on-target engagement with a selective subset of synaptic GABAARs is sufficient to suppress absence seizures while avoiding adverse side effects common to the GABA_A PAM class.

"Epilepsy remains one of the most prevalent neurological diseases worldwide, with a

population of approximately 65 million patients, but there remains a great unmet need for a safe and effective treatment option that suppresses seizure activity without drug resistance or harmful side effects such as sedation, cognitive impairment, ataxia and addiction,” said Alexandra MacLean, M.D., Chief Executive Officer of Avenue. “The preclinical data published in *Drug Development Research* demonstrate BAER-101’s ability to fully suppress seizures in the GAERS model, a translational animal model for anti-seizure drug development with a documented high predictability of response in humans. Additionally, BAER-101 demonstrated full efficacy with a minimal effective dose of 0.3 mg/kg, indicating that BAER-101 is the most potent compound yet reported in this model, as well as the first to show that a GABA PAM that is selective for the $\alpha 2$ and $\alpha 3$ -subtype GABAARs is active in this model. Building on BAER-101’s proven safety profile in over 700 patients and healthy human volunteers, these first-in-class preclinical findings support BAER-101’s continued development in a Phase 2a trial.”

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.

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 $\alpha 2$, $\alpha 3$ 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: expectations for increases or decreases in expenses; expectations for the clinical and pre-clinical development, manufacturing, regulatory approval, and commercialization of our pharmaceutical product candidate or any other products we may acquire or in-license; our use of clinical research centers and other

contractors; expectations for incurring capital expenditures to expand our research and development and manufacturing capabilities; expectations for generating revenue or becoming profitable on a sustained basis; expectations or ability to enter into marketing and other partnership agreements; expectations or ability to enter into product acquisition and in-licensing transactions; expectations or ability to build our own commercial infrastructure to manufacture, market and sell our product candidates; acceptance of our products by doctors, patients or payors; our ability to compete against other companies and research institutions; our ability to secure adequate protection for our intellectual property; our ability to attract and retain key personnel; availability of reimbursement for our products; estimates of the sufficiency of our existing cash and cash equivalents and investments to finance our operating requirements, including expectations regarding the value and liquidity of our investments; the volatility of our stock price; expected losses; expectations for future capital requirements; 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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