May 16, 2024

Avenue Therapeutics Announces Last Patient Last Visit in Phase 1b/2a Clinical Trial of AJ201 for the Treatment of Spinal and Bulbar Muscular Atrophy (Kennedy's Disease)

Topline results of the Phase 1b/2a clinical trial anticipated mid-year 2024

MIAMI, May 16, 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 completion of the last patient's final visit in the Company's Phase 1b/2a clinical trial of AJ201 for the treatment of spinal and bulbar muscular atrophy ("SBMA"), also known as Kennedy's Disease. Topline data are expected to be reported mid-year 2024.

"We are very excited to announce the last patient visit in the Phase 1b/2a clinical trial of AJ201 in SBMA, the final milestone before the anticipated topline data are reported in the middle of this year," said Alexandra MacLean, M.D., Chief Executive Officer of Avenue. "I want to sincerely thank the trial investigators, the patients, our partner AnnJi Pharmaceutical Co. Ltd., and our internal team for their dedication to completing this study on schedule and for their continued confidence in AJ201. Backed by the drug's promising preclinical efficacy profile and excellent clinical safety data in healthy volunteers, we look forward to further assessing the safety and tolerability of AJ201 in patients with SBMA, as well as AJ201's effect on potential pharmacokinetic and pharmacodynamic biomarkers of SBMA, including degradation of mutant AR proteins in muscles, MRI changes and neuroinflammation. We remain committed to advancing AJ201 for SBMA patients who currently have no effective, approved treatments available, as we work to achieve our mission of delivering impactful therapies to patients suffering from neurologic diseases."

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 (3:1). The primary endpoint of the study is to assess safety and tolerability of AJ201 in subjects with clinically and genetically defined SBMA. Secondary endpoints include pharmacokinetic and pharmacodynamic data measuring change from baseline in mutant AR protein levels in skeletal muscle and changes from baseline in expression of Nrf2-activated genes in skeletal

muscle. Exploratory objectives of the study include changes in the fat and muscle composition as seen on MRI scans. These endpoints are believed to be biomarkers indicating likelihood for longer term clinical improvement. Further details about this study can be found at ClinicalTrials.gov (Identifier: NCT05517603).

In April 2024, Avenue hosted a virtual key opinion leader ("KOL") event highlighting expert perspectives on SBMA. The event featured Christopher Grunseich, M.D., Lasker Clinical Research Scholar and Investigator and Head of the Inherited Neuromuscular Diseases Unit at the National Institute of Neurological Disorders and Stroke, and Tahseen Mozaffar, M.D., Professor of Neurology, Pathology and Laboratory Medicine, Director of the Division of Neuromuscular Diseases and Director of the ALS and Neuromuscular Center at the University of California, Irvine. The two featured speakers discussed the characteristics and treatment landscape of SBMA, as well as the trial design and potential of AJ201 in SBMA. A replay of the event can be accessed here.

About Spinal and Bulbar Muscular Atrophy

Spinal and bulbar muscular atrophy ("SBMA") is a rare, X-linked genetic neuromuscular disease primarily affecting men. The condition is caused by the trinucleotide CAG repeat expansion in the androgen receptor ("AR") which leads to production of a mutant polyglutamine ("polyQ") AR protein that forms aggregates responsible for muscular atrophy focused in the limbs and bulbar region of the body. The weakening of the bulbar muscles affects chewing, speech and swallowing, with patients prone to choking or inhaling foods or liquids, resulting in airway infection. SBMA also affects muscles in the limbs, leading to difficulty walking and injury caused by falling. Although there is a range of cited prevalence rates in scientific literature, a recent study used genetic analysis to estimate disease prevalence of 1:6,887 males. Currently, there are no treatments approved by the U.S. Food and Drug Administration or European Medicines Agency available for patients. For more information about SBMA, also known as Kennedy's Disease, please visit https://kennedysdisease.org/.

About AJ201

AJ201 is a novel, first-in-class asset in development for the treatment of spinal and bulbar muscular atrophy. It was designed to modify SBMA through multiple mechanisms including degradation of the abnormal androgen receptor protein and by stimulating the Nrf1 and Nrf2 pathways, which are involved in protecting cells from oxidative stress which can lead to cell death. A first-in-human Phase 1 study of AJ201 in 72 healthy volunteers revealed an excellent safety and pharmacokinetic profile. It is currently being studied in a Phase 1/2a multicenter, randomized, double-blind clinical trial in six clinical sites across the U.S., which aims to evaluate the safety, PK/PD data and clinical response of AJ201 in patients suffering from SBMA. AJ201 has been granted Orphan Drug Designation by the FDA for multiple polyQ diseases, including SBMA, Huntington's disease and spinocerebellar ataxia. Avenue exclusively licensed AJ201 from AnnJi Pharmaceuticals for use in the United States, Canada, European Union, Great Britain, and Israel.

About Polyglutamine diseases

Polyglutamine diseases are a group of neurodegenerative disorders caused by expanded CAG repeats encoding a long polyQ tract in the affected proteins. To date, a total of nine

polyQ disorders have been described. Mutant protein aggregation in affected tissues is the pathological hallmark of polyQ diseases. Neuroinflammation, oxidative stress and dysregulated protein quality control are thought to be key pathological factors that are either direct results of mutant protein aggregations and/or exacerbate the severity and progression of the diseases. Modulating multiple cellular pathways in enhancing degradation of mutant AR aggregates, inducing antioxidant and heat shock responses, and increasing proteasome expression simultaneously provide the rationale to develop AJ201 for the treatment of SBMA and potentially other polyQ diseases.

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 post-operative 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.

Contact:

Jaclyn Jaffe
Avenue Therapeutics, Inc.
(781) 652-4500
ir@avenuetx.com

Source: Avenue Therapeutics