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Avenue Therapeutics Enters into a Transformational License Agreement with AnnJi Pharmaceutical to Develop and Commercialize AJ201, a First-in-Class Clinical Asset for the Treatment of Spinal and Bulbar Muscular Atrophy

AJ201 is being evaluated in a Phase 1b/2a clinical trial in the U.S. for the rare X-linked genetic neurodegenerative disease also known as Kennedy's Disease which currently has no FDA approved therapy

MIAMI, March 02, 2023 (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 rare and neurologic diseases, today announced that it has entered into an exclusive license agreement with AnnJi Pharmaceutical Co., Ltd. ("AnnJi"), a Taiwanese clinical-stage drug company, for AJ201, a first-in-class clinical asset currently in a Phase 1b/2a study in the U.S. for the treatment of spinal and bulbar muscular atrophy ("SBMA"), also known as Kennedy's Disease.

"The license for AJ201 brings a cutting-edge asset into Avenue's pipeline that is the lead molecule in the clinic to treat Kennedy's Disease, a debilitating rare neuromuscular disorder. With AJ201 leading the way, we are confident in the potential of our diversified portfolio of three assets to deliver value for investors in the near term and patients in the longer term," said Alexandra MacLean, M.D., Chief Executive Officer of Avenue.

SBMA is a rare, inherited, X-linked genetic neuromuscular disease primarily affecting men. The condition is caused by a polyglutamine expansion in the androgen receptor ("AR") which leads to production of an abnormal AR protein that forms aggregates responsible for muscle atrophy focused in the spinal-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 the literature, a recent study used genetic analysis to estimate disease

prevalence of *1:6,887* males[i]. Currently, there is no effective treatment for SBMA.

AJ201 was designed to modify SBMA through multiple mechanisms including degradation of the abnormal AR protein and by stimulating Nrf1 and Nrf2, which are involved in protecting cells from oxidative stress which can lead to cell death. AJ201 completed a Phase 1 clinical trial in 2021, which demonstrated the safety of the molecule. It is currently being studied in a Phase 1b/2a multicenter, randomized, double-blind clinical trial in six clinical sites across the U.S., and screening of patients with SBMA has begun. This study aims to evaluate the safety and clinical response of AJ201 in patients suffering from SBMA. AJ201 has been granted Orphan Drug Designation ("ODD") by the U.S. Food and Drug Administration for the indications of SBMA, Huntington's Disease and Spinocerebellar Ataxia.

Under the terms of the license agreement, AnnJi will receive upfront payments of $3 million and is entitled to receive future development, regulatory and commercialization milestone payments, as well as royalties on net sales of the licensed product. Avenue will also issue 831,618 shares of its common stock to AnnJi in connection with the initial closing of the license transaction and up to an additional 276,652 shares upon achievement of a clinical milestone, aggregating in total to not more than 19.99% of the Company's current total number of outstanding shares of common stock. The agreement includes the U.S., Canada, European Union, Great Britain and Israel as exclusively licensed territories.

Lindsay A. Rosenwald, M.D., Chairman of the Board of Avenue, stated, "We are excited to progress the clinical development of AJ201 to treat SBMA and further expand Avenue as a leading neurological company."

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 and rare 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 α2/3 receptor positive allosteric modulator for CNS diseases, and IV Tramadol, which is in Phase 3 clinical development for the management of moderate-to-moderately-severe 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.

About AnnJi Pharmaceutical
Founded in 2014, AnnJi Pharmaceutical Co., Ltd. ("AnnJi") is an R&D based, clinical-stage new drug company dedicated to the development of first-in-class small molecules focusing on indications with highly unmet needs in the therapeutic areas of neurology, dermatology, and inflammatory disorders, including rare diseases such as idiopathic pulmonary fibrosis and Kennedy's disease, or SBMA (Spinal and bulbar muscular atrophy).

AnnJi's mission is to develop innovative therapeutics to improve the quality of life of patients with neglected diseases. AnnJi's goals are to translate and develop unique and highly differentiated drug therapies and to engage global collaborators and business partners in late-stage product development and commercialization.

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 candidate; 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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[i] M. Zanovello et al., Unexpected frequency of the pathogenic*AR*CAG repeat 2 expansion in the general population. Brain, *in press* (2023).

Source: Avenue Therapeutics