



AnnJi Pharmaceutical Advances AJ201 into Pivotal Phase 3 ROMA-KD Trial for Spinal and Bulbar Muscular Atrophy (SBMA)

Descrizione

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The only known investigational therapy for SBMA in Phase 3 development worldwide

TAIPEI, Oct. 5, 2026 /PRNewswire/ - AnnJi Pharmaceutical Co., Ltd. (AnnJi, TPEX: 7754), a clinical-stage drug development company focused on innovative small molecules, today announced that it is proceeding with the U.S. portion of its pivotal Phase 3 ROMA-KD trial of AJ201 (rosolutamide) in patients with spinal and bulbar muscular atrophy (SBMA, or Kennedy's disease), a progressive, inherited neuromuscular disease with no FDA-approved treatment.

"For people living with SBMA, the disease is felt in everyday moments: a staircase that becomes harder to climb, a meal that becomes harder to swallow, a voice that becomes harder to use," said Dr. Wendy Huang, Chairperson and CEO of AnnJi. "It affects whole families, often across generations, and there is still no FDA-approved treatment. Advancing AJ201 into Phase 3 brings us one step closer to potentially changing that. We are now focused on activating U.S. trial sites, enrolling patients and completing this study as efficiently and responsibly as possible, with patients and families at our side."

AJ201 is an investigational oral small molecule that promotes clearance of the pathogenic mutant androgen receptor (mAR) protein and activates cellular stress-response pathways, including Nrf1, Nrf2 and HSF1. A potential first-in-class treatment for SBMA, AJ201 has received Orphan Drug Designation and Fast Track Designation from the U.S. FDA, as well as Orphan Drug Designation in the European Union.

The Phase 3 program builds on encouraging results from the completed Phase 2 study of AJ201, announced in May 2025, and was informed by subsequent interactions with regulatory authorities.

The ROMA-KD trial is a global, multicenter, randomized, double-blind, placebo-controlled study designed to evaluate the efficacy and safety of AJ201 in ambulatory individuals with symptomatic

SBMA. Approximately 200 patients are expected to enroll globally, with the United States as a key region. The trial is intended to support potential global regulatory submissions for AJ201. AnnJi will coordinate site activation across regions in line with its global development and partnering strategy.

Patient and Care Partner Advisory Council

AnnJi has established its SBMA Patient and Care Partner Advisory Council, first announced in collaboration with the Kennedy's Disease Association (KDA) at the 2026 KDA International Patient and Scientific Conference. The Council brings together people living with SBMA and their care partners, whose lived experience offers insights that clinical data alone cannot capture.

Serving in an advisory capacity, the Council provides a structured forum for ongoing, two-way dialogue between AnnJi and the SBMA community. Members share perspectives that may help inform AnnJi's patient-centered research and clinical development, including considerations for study design and the development of clear, accessible patient-facing materials. The Council operates independently of AnnJi's clinical trials, and participation has no bearing on trial eligibility or enrollment.

Commitment to the SBMA Community

AnnJi remains committed to advancing AJ201 for people living with SBMA and their families, and to working in transparent partnership with patients, care partners, clinicians and advocacy organizations worldwide.

About SBMA and AJ201

SBMA, or Kennedy's disease, is a rare, X-linked inherited neuromuscular disorder caused by a CAG repeat expansion in the androgen receptor (AR) gene. The resulting mutant AR protein contributes to motor neuron and muscle degeneration through mechanisms involving cellular toxicity, oxidative stress and neuroinflammation. SBMA is believed to be substantially underdiagnosed, in part because its symptoms are often mistaken for other neuromuscular conditions, such as amyotrophic lateral sclerosis (ALS), and because the absence of an approved treatment has offered little incentive for genetic testing. There is currently no FDA-approved treatment for SBMA.

AJ201 (rosolutamide; also known as JM17) is an investigational oral small molecule designed to address multiple disease mechanisms implicated in SBMA. AJ201 promotes clearance of the pathogenic mutant AR protein and activates cellular pathways involved in antioxidant defense, protein quality control and stress response, including Nrf1, Nrf2 and HSF1.1 Through these complementary mechanisms, AJ201 is being developed as a potential disease-modifying therapy for SBMA.

1 Bott LC, et al. Hum Mol Genet. 2016.

About AnnJi Pharmaceutical

AnnJi Pharmaceutical Co., Ltd. (TPEx: 7754) is a clinical-stage drug development company focused on innovative new chemical entities (NCEs) for diseases with high unmet medical need in neurology, dermatology and immune-inflammatory conditions. Its pipeline includes AJ201 for Kennedy's disease (SBMA), a rare neuromuscular disease, as well as HDAC6-targeted programs in neuropathy,

including chemotherapy-induced peripheral neuropathy, and fibrotic diseases.

AnnJi is committed to developing differentiated therapies that improve the quality of life of patients with neglected chronic diseases. The company advances high-quality drug candidates through clinical proof of concept and collaborates with global pharmaceutical partners on further development and commercialization, with the goal of bringing its therapies to patients worldwide and driving sustainable growth.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the timing, conduct and potential outcomes of the Phase 3 ROMA-KD trial, site activation and enrollment, the regulatory pathway for AJ201, and AnnJi's development and partnering plans. Words such as "anticipate," "believe," "expect," "intend," "may," "plan," "potential," "will" and similar expressions are intended to identify such statements. These statements are based on management's current expectations and are subject to risks and uncertainties, including clinical, regulatory, manufacturing, partnering, financing and operational risks, that could cause actual results to differ materially. Additional information about these risks is available in AnnJi's disclosures filed with the Taipei Exchange and published on the Market Observation Post System (MOPS). AJ201 is an investigational product and has not been approved by any regulatory authority for any indication. Forward-looking statements speak only as of the date of this press release, and, except as required by applicable law, AnnJi undertakes no obligation to update them.

Media Contacts

Anne Yu: Anne.Yu@ajpharm.com Sylvia Lin: Sylvia.Lin@ajpharm.com

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