



For Immediate Release

Axial Therapeutics Receives FDA Clearance of IND Application and Initiates Phase 2b Trial for AB-2004, Lead Gut-Targeted, Molecular Therapeutic for the Treatment of Irritability in Autism Spectrum Disorder

– Global, placebo-controlled Phase 2b trial now enrolling in the U.S., Australia and New Zealand

– Axial is activating 20 Phase 2b clinical trial sites in the U.S. and seven clinical sites in Australia and New Zealand

– Clinical studies to date confirm AB-2004’s strong safety profile and potential to address large unmet treatment need in irritability associated with autism spectrum disorder (ASD)

Woburn, Mass., October 27, 2021 – Axial Therapeutics, a clinical-stage biopharmaceutical company dedicated to improving the lives of people with neurological disorders and diseases, today announced that the company received clearance from the U.S. Food and Drug Administration (FDA) of its investigational new drug (IND) application for lead candidate, AB-2004, a gut-targeted, molecular therapeutic for the treatment of irritability associated with ASD. In addition to the FDA clearance, clinical trial authorizations have been received for AB-2004 in Australia and New Zealand. By advancing AB-2004 further into the clinic, Axial is aiming to develop new therapeutic options to help manage irritability associated with ASD, and potentially other co-occurring conditions that can prevent children with autism from fully participating in daily life.

“Novel medical interventions for safely managing co-occurring conditions associated with ASD, such as irritability, are desperately needed by children and their families,” said A. Stewart Campbell, Ph.D., chief executive officer of Axial Therapeutics. “With a solid foundation of mechanistic and safety data, AB-2004 has shown promise in reducing irritability in adolescents with ASD. We are now taking the next major step toward realizing the potential of our innovative gut-targeted therapeutic approach by evaluating AB-2004 in a multi-national, randomized, placebo-controlled Phase 2b trial, which we have just initiated.”

AB-2004: Novel Small Molecule Therapy with Defined Mechanism of Action

AB-2004 is a first-in-class, molecular therapeutic that targets the microbiome gut-brain axis and its role in autism. AB-2004 has a unique mechanism of action that selectively sequesters certain bacterially derived metabolites in the gut before they enter the bloodstream and reach the brain. Axial’s gut-targeted approach minimizes the potential for side effects due to a lack of systemic exposure to the drug.

Axial is evaluating AB-2004 in a Phase 2b clinical trial based upon a large body of existing human safety data. In a Phase 1b/2a clinical trial, AB-2004 was safe and well-tolerated, exhibiting no drug-related serious adverse events. AB-2004 was also shown to reduce several key microbial metabolites implicated in autism and showed evidence of improving key co-occurring conditions including irritability and anxiety. Formulated as a powder that can be mixed with food, AB-2004 offers convenient oral dosing for the ASD community.

About the AB-2004 Phase 2b Trial

The Phase 2b trial is a global, randomized, double-blind, placebo-controlled study evaluating the efficacy, safety, and tolerability of AB-2004 in children aged 13 to 17 who have been diagnosed with ASD. The study will enroll an estimated 195 patients who will receive low or high doses of AB-2004 or placebo.

The primary efficacy endpoint of the study is the mean change in irritability using the Aberrant Behavior Checklist (ABC) from baseline to week 8 for AB-2004 high dose. Secondary outcome measures include the mean change in the ABC-I score (Irritability) from baseline to week 8 for AB-2004 low dose; the mean change in the Clinical Global Impression-Severity (CGI-S) from baseline to week 8 for AB-2004 high and low dose; and the number of participants who reported treatment emergent adverse events from baseline to week 8.

“The severity of daily challenges faced by people with autism, combined with the lack of safe and effective treatment options, has resulted in a significant unmet need for innovative medical interventions,” said Raun D. Melmed, M.D., co-founder and medical director of the Southwest Autism Research and Resource Center, who is a principal investigator for the AB-2004 study. “The AB-2004 clinical trial program offers potential hope for children with ASD who too often struggle with anxiety and irritability such that it permeates all aspects of daily life.”

The AB-2004 Phase 2b study will be conducted at approximately 20 clinical trial centers in the United States and approximately seven sites in Australia and New Zealand and is expected to generate topline data in 2023. More information about the U.S. trial is available at [clinicaltrials.gov, identifier NCT04895215](https://clinicaltrials.gov/identifier/NCT04895215).

About Autism Spectrum Disorder (ASD)

According to the Centers for Disease Control and Prevention (CDC), approximately 1 in 54 children has been identified with ASD. Core symptoms of ASD include impairments in social interaction, communication, and the presence of stereotyped repetitive behaviors. Co-occurring conditions that impact quality of life are extensive and diverse, and include irritability, anxiety, ADHD, allergies, autoimmune disorder, neuroinflammation, and epilepsy. Physicians have reported irritability impacts a majority of pediatric ASD patients. The presentation of ASD-associated irritability can vary with severity and age and can be caused by a broad array of different factors, including lack of sleep, the inability to communicate pain, and mental health conditions.

About Axial Therapeutics

Axial Therapeutics is a clinical-stage biopharmaceutical company dedicated to improving the lives of people with neurological disorders and diseases. The company is the scientific leader in the biological role of the microbiome gut-brain axis and its influence on the central nervous system. Harnessing its unique expertise in the microbiome, Axial is developing small molecule drugs with defined mechanisms of action that act on new targets to mitigate the impact of metabolites and bacteria in the gut linked to neurological disease pathology, progression, and symptoms. The company is advancing a pipeline of microbiome-inspired therapeutics for diseases with significant unmet patient need, including autism and Parkinson’s disease, and is also pursuing pre-clinical discovery of gut-targeted therapies in oncology. Axial’s lead product candidate is AB-2004, a molecular therapeutic in Phase 2 clinical trials for the treatment of irritability in children with autism. For more information, visit <https://axialtx.com>.

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